As confidentially submitted to the Securities and Exchange Commission on August 31, 2018.
This draft registration statement has not been publicly filed with the Securities and Exchange Commission, and all information herein remains strictly confidential.

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

INHIBIKASE THERAPEUTICS, INC.

(Exact name of Registrant as specified in its charter)

Delaware	**2836**	**26-3407249**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

3350 Riverwood Parkway SE, Suite 1900
Atlanta, GA 30339
(678) 392-3419
(Address, including zip code, and telephone number, including area code, of Registrant's principal executive offices)

Milton H. Werner, Ph.D.
President and Chief Executive Officer
Inhibikase Therapeutics, Inc.
3350 Riverwood Parkway SE, Suite 1900
Atlanta, GA 30339
(678) 392-3419
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:
Merrill M. Kraines, Esq.
Pepper Hamilton, LLP
The New York Times Building
37th Floor
620 Eighth Avenue
New York, NY 10018-1405
(212) 808-2711

Approximate date of commencement of proposed sale to the public: As soon as practicable after the effective date of this registration statement.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☐

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities to be Registered	**Amount to be Registered[1]**	**Proposed Maximum Offering Price Per Share[2]**	**Proposed Maximum Aggregate Offering Price[1][2]**	**Amount of Registration Fee[3]**
Common Stock, $0.001 par value per share				

(1) Includes [•] shares that the underwriters have an option to purchase.
(2) Estimated solely for the purpose of computing the amount of the registration fee pursuant to Rule 457(a) under the Securities Act of 1933, as amended.
(3) Calculated pursuant to Rule 457(a) based on an estimate of the proposed maximum aggregate offering price of the securities registered hereunder to be sold by the registrant.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended, or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

SUBJECT TO COMPLETION, DATED , 2018

Shares

Common Stock

This is an initial public offering of shares of common stock by Inhibikase Therapeutics, Inc. We are offering [•] shares of our common stock.

Prior to this offering, there has been no public market for our common stock. It is currently estimated that the initial public offering price will be between $[•] and $[•] per share.

We intend to apply to list our shares on The Nasdaq Capital Market under the symbol "IKT."

We are an "emerging growth company" as defined under the federal securities laws and, as such, have elected to comply with certain reduced reporting requirements for this prospectus and may elect to do so in future filings.

Investing in our common stock involves risks. See the section titled "Risk Factors" beginning on page 12 to read about factors you should consider before buying shares of our common stock.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities, or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$	$
Underwriting discounts[(1)]	$	$
Proceeds, before expenses, to us	$	$

(1) See the section titled "Underwriting" for additional information regarding compensation payable to the underwriters.

To the extent that the underwriters sell more than [•] shares of our common stock, the underwriters have the option to purchase up to an additional [•] shares from us at the initial public offering price less the underwriting discount.

The underwriters expect to deliver the shares against payment in New York, New York on or about [•], 2018.

Prospectus dated [•], 2018.

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Prospectus

Through and including [•], 2018 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

We and the underwriters have not authorized anyone to provide you any information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are not making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus is accurate only as of the date on the front cover of this prospectus. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside of the United States: we have not and the underwriters have not done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus and is qualified in its entirety by the more detailed information and financial statements included elsewhere in this prospectus. It does not contain all of the information that may be important to you and your investment decision. You should carefully read this entire prospectus, including the matters set forth under the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes. In this prospectus, unless context requires otherwise, references to "we," "us," "our," or "the Company" refer to Inhibikase Therapeutics, Inc. See the section titled "Glossary" for definitions of key scientific and technical terms used in this prospectus.

Overview

We are a company developing therapeutics for neurodegenerative disease inside and outside of the brain. We anticipate filing two Investigational New Drug Applications, or INDs, for our lead programs with the U.S. Food and Drug Administration, or FDA, in the first quarter of 2019.

Our lead programs utilize small molecule oral protein kinase inhibitors to treat Parkinson's Disease, or PD, and its gastrointestinal complications. We have shown that our lead clinical candidate, IkT-148009, a potent, brain penetrant c-Abl protein kinase inhibitor, halts and/or reverses neurodegeneration in the brain and gastrointestinal tract, or GI tract, in preclinical models that mimic the human disease. We believe our therapeutic approach is disease-modifying.

In our opinion, the multi-decade failures in the treatment of neurodegenerative disease result from a lack of understanding of the biochemistry of the disease processes involved. Historically, symptoms of a neurodegenerative disease, like a "plaque" made up of a misfolded and/or aggregated protein(s), have been the development focus. To our knowledge, a "plaque"-focused strategy has not resulted in approval of a new medication that can alter the disease course for a neurodegenerative disease. We focus instead on the proteins that become dysfunctional in a disease pathway and seek to understand what leads to their dysfunction. In PD and other neurodegenerative diseases, our pathway-focused approach has enabled a deeper understanding of the origins of the disease and an understanding of how individual proteins are linked together to define the disease process. Using this strategy, we have discovered novel therapeutics for the Abelson protein kinase, or c-Abl, which we believe can alter the disease course for PD. Protein kinases are enzymes that modify proteins, including alpha-synuclein. Protein kinase inhibitors are small molecules that are believed to block the actions of protein kinases.

In addition to programs in neurodegeneration, our platform drug discovery and delivery technologies have identified additional opportunities, including potential treatment of bacterial or viral infections using a single agent at fixed dose and a potential near-term commercial opportunity in oncology that we believe will complete clinical development in 2019, subject to future FDA agreements, followed by completion of the FDA requirements for new drug approval in 2020. IkT-001Pro in oncology will seek to validate the pharmacology advantage of our prodrug delivery technology in a cancer patient population that is well understood. Following validation of IkT-001Pro in oncology, we believe the same pharmacology advantages could be applied to IkT-148009, our lead drug for neurodegenerative disease, to enhance clinical development. We believe we are one of the pioneers of the application of protein kinase inhibitors to non-oncology indications, including neurodegeneration and infectious diseases, as well as their more traditional role in the treatment of cancer.

Our Programs

Our portfolio is focused on developing protein kinase inhibitors to treat neurodegeneration in the brain and GI tract that arise from dysfunctional alpha-synuclein. Using IkT-148009, our lead Abelson protein kinase inhibitor, or c-Abl inhibitor, we intend to clinically evaluate the impact of c-Abl inhibition on newly diagnosed PD patients, patients early in the course of their disease, and patients with dysphagia and/or neurogenic constipation. If there are no regulatory objections to our IND submissions of the first quarter of 2019, we intend to initiate clinical development shortly thereafter using a hybrid Phase 1/Phase 2 development approach, subject to agreements with the FDA.

We have also developed an alternate delivery approach for oral kinase inhibitors by converting them into prodrugs. We developed the oncology prodrug, IkT-001Pro, of the anticancer agent Imatinib, to alter the way a protein kinase inhibitor is absorbed in the GI tract and we believe IkT-001Pro will lead to a safer and better tolerated treatment for Imatinib-sensitive cancers. We believe demonstrating the benefits of this technology in a well-known patient population will help validate the utility of our prodrug technology broadly and may also generate revenue to support our pursuits in neurodegenerative disease. IkT-001Pro is a near-term commercial opportunity requiring a single 12 – 24 patient trial, subject to future FDA agreements, that we believe will complete the requirements for submission of a New Drug Application, or NDA, in 2020.

Additional research programs will seek to develop medications for other alpha-synuclein-related diseases, specifically Dementia with Lewy Body, or DLB, and Multiple System Atrophy, or MSA as well as our programs in anti-infectives that target host-factors to block viral or bacterial infections with a single agent at fixed dose. Our first application intends to treat infectious disease by suppressing John Cunningham, or JC, virus infection, the cause of Progressive Multifocal Leukoencephalopathy, or PML.

For Parkinson's Disease, Progressive Multifocal Leukoencephalopathy and stable-phase Chronic Myelogenous Leukemia, multiple FDA pre-IND discussions have outlined a pathway for clinical development.

					Clinical Development			Biomarker		
Drug Target	Drug candidate	Modality	Disease indication	Preclinical Development	Phase 1	Phase 2	Phase 3	Preclinical target engagement	Clinical target engagement	Can be used for patient selection
Neurodegeneration										
c-Abl	IkT-148009	Small molecule	Parkinson's Disease: Treatment Naive		2019			Validated	Validating	Yes
c-Abl	IkT-148009	Small molecule	Parkinson's Disease: Early Stage		2019			Validated	Validating	Yes
c-Abl	IkT-148009	Small molecule	Neurogenic Constipation		2019			Validated	Validating	Yes
c-Abl	IkT-148009	Small molecule	Dysphagea		2019			Validated	Validating	Yes
Oncology										
BCR-Abl	IkT-001Pro	Small molecule	Stable-phase CML (orphan indication)		505(b)(2) single trial to market 2020			Validated	Validating	Yes
Research Phase										
c-Abl	IkT-148x	Small molecule	Dementia with Lewy Body					Validated	Validating	Unknown
c-Abl	IkT-148x	Small molecule	Multiple System Atrphy					Validated	Validating	Unknown
c-Abl	IkT-1427	Small molecule	Progressive multifocal leukoencephalopathy					Validated	Validating	Yes

Dates under 'Clinical Development' represent the anticipated timeframe for initiation of the clinical program. IkT-148x refers to a series of portfolio compounds being evaluated for these indications in preclinical models that are from the same chemical family at IkT-148009. For biomarker status, 'Validated' refers to proof of target engagement in the target tissue. 'Validating' indicates ongoing analysis to prove target engagement using proprietary sources and methods under development. All four IkT-148009 programs use the same Phase 1 program prior to separating into trials in the brain or GI tract.

Our Strategy

- **Identification and characterization of the pathway(s) governing neurodegenerative disease:** We select our therapeutic targets by identification and characterization of disease pathways that we believe drive neurodegenerative disease and elucidate the biochemistry of pathway proteins to enable small molecule targeting to treat PD and related disorders, often involving clinically validated targets.

- **Proprietary method of drug discovery in neurodegeneration:** We use a Re-engineering Approach with Metabolism Preserved method, or RAMP, to imprint the properties we desire from an approved medication onto a new molecular entity for treatment inside and outside of the brain. Using RAMP, we believe we can "pre-determine" the pharmacology and side effect profile of our product candidates using an existing medication as a template.

- **Delivering neurodegenerative treatments as a prodrug to improve pharmacology and safety:** A prodrug is a compound that, after administration, is metabolized by the body into a pharmacologically active drug. Our prodrug technology has been shown in animal models to suppress GI and other adverse events commonly associated with oral protein kinase inhibitors and improve drug absorption from the GI tract. We believe this technology both reduces side effects and enhances drug distribution into the target tissues, which we believe will improve safety and tolerability of our protein kinase inhibitors for neurodegenerative and other diseases.

We believe that the application of these principles will significantly increase the probability of our success and will shorten the time required to bring effective therapeutics to patients with neurodegenerative and other diseases.

Our Indications

Parkinson's Disease and other diseases caused by dysfunctional alpha-synuclein. PD is the second most prevalent neurodegenerative disease of the Central Nervous System, or CNS, with more than 10,000,000 cases worldwide. Historically characterized by the progressive loss of dopamine-secreting neurons near the brain stem, PD's pathology is now recognized to be a more global disease of the brain, as well as affecting nerves in the GI tract and throughout the rest of the body. We believe the development of widespread pathology in PD arises from damage caused by dysfunctional alpha-synuclein. Our lead candidate for PD, IkT-148009, is a potent, selective small molecule inhibitor of the Abelson protein kinase, c-Abl, that is brain penetrant in animal models. c-Abl is crucial for proper neuronal development, but does not have a known function in healthy, adult neurons. Using new animal models, we and our collaborators have shown that PD is a disease of hyperactivation of c-Abl, which acts on dysfunctional alpha-synuclein to drive neurodegeneration inside and outside of the brain. c-Abl acts as a checkpoint, determining whether dysfunctional alpha-synuclein is tagged to be flushed out of a neuron and discarded, or whether dysfunctional alpha-synuclein commits a neuron to degeneration and death. Inhibition of c-Abl by IkT-148009, our lead clinical candidate, completely blocks neurodegeneration in progressive disease models. We anticipate four clinical programs using IkT-148009 will launch in 2019 to treat newly diagnosed and early stage Parkinson's patients and modify the disease course in the brain and GI tract.

Chronic Myelogenous Leukemia (CML). The earliest application of our prodrug technology is to treat stable-phase CML, and we are using this program to validate our prodrug technology in an established patient population prior to its application to neurodegenerative disease. IkT-001Pro is an oncology prodrug of the anti-cancer agent Imatinib that we believe will offer distinct safety advantages over the generic and branded forms of Imatinib to treat CML and related diseases. Up to one-half of Imatinib patients experience on-dosing side effects in the GI tract that diminish adherence to daily therapy to treat their disease. Failure to adhere to therapy significantly reduces likelihood of treatment success. In non-human primates, IkT-001Pro suppresses these GI side effects on dosing, resulting in a 13-fold increase in the No Adverse Event Level, or NOAEL, relative to the NOAEL of Imatinib itself. In both a solid and liquid tumor model in mice, IkT-001Pro is just as active as Imatinib, but at 15% lower dose, because the prodrug more efficiently delivers Imatinib to the target tissue. Since the frequency and severity of side effects from Imatinib vary linearly with oral dose, this data suggests further safety improvements in Imatinib treatment can be realized through dose lowering without sacrificing efficacy. IkT-001Pro is a near-term commercial opportunity that we believe requires only a single dose calibration clinical study, subject to future FDA agreements, prior to seeking approval under the FDA rule 505(b)(2) regulation. We view this product as a potential near-term revenue stream to help support our other programs in neurodegeneration and infectious disease.

We have engaged the FDA in multiple pre-IND discussions for IkT-148009 in PD and the oncology product IkT-001Pro in two FDA divisions: Neurology and Hematology Products. For each division pre-IND discussion, we presented preclinical toxicology data in comparison to Imatinib, because comparative toxicology aids in predicting the safety margin for our lead products in human patients. This comparative toxicology approach is being used to seek early entry into the target patient population, because our compounds closely mirror Imatinib in terms of Absorption, Distribution, Metabolism and Elimination, or ADME, properties. Based on these FDA discussions, substantial truncation of pre-IND requirements and early phase clinical requirements are believed to be achievable, or have been achieved,

shortening the clinical development timeline prior to "Proof-Of-Concept" clinical studies. Using this comparative toxicology approach, we believe we have also lowered the risk-profile for our novel product candidates during clinical development. See "Business — Government Regulation" elsewhere in this prospectus for an overview of the regulatory drug approval process.

We currently have worldwide commercialization rights to all of our development programs and IP protection until 2032 or later.

Our management team is a critical component for the development of our business model and the execution of our strategy. We are led by executives with an average of over 20 years of relevant senior leadership experience, including developing and commercializing branded and generic pharmaceuticals at large multinational pharmaceutical and biotech companies such as AstraZeneca plc and Sanofi S.A. Our team has significant experience in commercialization of pharmaceutical products, translational science, drug evaluation, clinical development, regulatory affairs and business development.

Risks Associated with Our Business

Our business is subject to numerous risks and uncertainties that you should consider before investing in our company. These risks are described more fully in the section titled "Risk Factors" in this prospectus. These risks include, but are not limited to, the following:

- We are a preclinical drug development company with extremely limited resources, a limited operating history and have no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.
- Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in this prospectus.
- Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We have never generated any revenue from product sales, we may never generate any revenue from product sales and we may fail to generate further revenue or be profitable.
- We have incurred significant net losses since our inception and anticipate that we will continue to incur net losses for the foreseeable future.
- If we fail to obtain additional financing, we may be unable to complete the development and, if approved, commercialization of our product candidates.
- Due to the significant resources required for the development of our programs, and depending on our ability to access capital, we must prioritize development of certain product candidates. We may expend our limited resources on programs that do not yield a successful product candidate and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.
- Our business is highly dependent on the success of our initial product candidates targeting neurodegenerative diseases. All of our product candidates will require significant nonclinical and clinical development before we can seek regulatory approval for and launch a product commercially.
- We currently contract with various research institutions to perform the research and development activities needed to develop our products, and if we ever choose to or need to find alternative research institutions, we may not be able to do so at all or, if we are able to do so, it may be costly and may cause significant delays in the development and commercialization of our products.
- Research, development, and commercialization of pharmaceutical products are inherently risky. We are heavily dependent on the successful use of our RAMP drug discovery program and the product candidates that emerge from it and which are undergoing preclinical development. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

- Preclinical development is uncertain. Our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business.
- We have no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.
- If the FDA rejects our INDs, objects to the characteristics of IkT-148009 or places us on clinical hold, we will not be able to commence a Phase 1 clinical trial for IkT-148009 in the U.S., which would likely have a material adverse effect on us.
- We have never dosed any of our product candidates in humans. Our planned clinical trials may reveal significant adverse events, toxicities or other side effects not seen in our preclinical studies and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.
- We face significant competition in an environment of rapid technological and scientific change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer, more advanced or more effective than ours, which may negatively impact our ability to successfully market or commercialize any product candidates we may develop and ultimately harm our financial condition.
- The regulatory approval processes of the FDA, European Medicines Agency, or EMA and comparable foreign regulatory authorities are lengthy, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, we will be unable to generate product revenue and our business will be substantially harmed.
- We expect to depend in whole or in part on collaborations with third parties for the research, development and commercialization of any product candidates we may develop. If any such collaborations are not successful, we may not be able to realize the market potential of those product candidates.
- If we are unable to obtain and maintain patent protection for any product candidates we develop, our competitors could develop and commercialize products or technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop, and our technology may be adversely affected.
- We currently rely on and expect to continue to rely on third parties to conduct our preclinical testing, as well as any future research and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, or testing.
- We contract with third parties for the manufacture of materials for our research programs and preclinical studies and expect to continue to do so for any future clinical trials and for commercialization of any product candidates that we may develop. This reliance on third parties carries and may increase the risk that we will not have sufficient quantities of such materials or product candidates that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.
- We depend on third party suppliers for key raw materials used in the manufacturing processes for our product candidates, and the loss of these third party suppliers or their inability to supply us with adequate raw materials could harm our business.

- Insiders will continue to have substantial influence over us after this offering, which could limit your ability to affect the outcome of key transactions, including a change of control.

Corporate Information

We were incorporated in Delaware in 2010 as a successor to a Georgia limited liability company, formed in September 2008. Our principal executive offices are located at 3350 Riverwood Parkway SE, Suite 1900, Atlanta, Georgia, 30339. We also maintain offices at 485 Massachusetts Avenue, Suite 300, Cambridge, Massachusetts, 02139. Our telephone number is (678) 392-3419. Our website address is www.inhibikase.com. Information contained on our website is not incorporated by reference into this prospectus, and it should not be considered to be part of this prospectus.

We use Inhibikase Therapeutics, the Inhibikase Therapeutics logo, and other marks to represent us in the United States and other countries. Not all of these marks are fully trademarked in the United States and in other countries. This prospectus contains references to our logo and service marks and to those belonging to other entities. Solely for convenience, trademarks and trade names referred to in this prospectus, including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate in any way that we will not assert, to the fullest extent under applicable law, our rights to our logo and trade names or the rights of the applicable licensor. We do not intend our use or display of other entities' names, trade names, trademarks or service marks to imply a relationship with, or endorsement or sponsorship of us by, any other entity.

Implications of Being an Emerging Growth Company and a Smaller Reporting Company

We qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act. As an "emerging growth company," we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include, but are not limited to:

- requiring only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced "Management's discussion and analysis of financial condition and results of operations" and "Selected financial data" disclosure in our Securities Act of 1933, as amended, or the Securities Act, filings;
- reduced disclosure about our executive compensation arrangements;
- no non-binding advisory votes on executive compensation or golden parachute arrangements; and
- exemption from compliance with the auditor attestation requirement in the assessment of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes Oxley Act of 2002, or SOX.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an "emerging growth company." We will continue to remain an "emerging growth company" until the earliest of the following: (i) the last day of the fiscal year following the fifth anniversary of the date of the completion of this offering; (ii) the last day of the fiscal year in which our total annual gross revenue is equal to or more than $1.07 billion; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or the SEC.

We are also a "smaller reporting company" as defined in the Securities Exchange Act of 1934, or the Exchange Act and have elected to take advantage of certain of the scaled disclosures available to smaller reporting companies. To the extent that we continue to qualify as a "smaller reporting company" as such term is defined in Rule 12b-2 under the Exchange Act, after we cease to qualify as an emerging growth company, certain of the exemptions available to us as an "emerging growth company" may continue to be available to us as a "smaller reporting company," including (i) exemption from compliance with the auditor attestation requirements pursuant to SOX; (ii) reduced disclosure about our executive compensation arrangements; (iii) the requirement to provide only two years of audited financial statements, instead of three years; and (iv) not being required to provide certain quantitative and qualitative disclosures about

market risk. We will continue to be a "smaller reporting company" until we have more than $250 million in public float (based on our common stock) measured as of the last business day of our most recently completed second fiscal quarter or, in the event we have no public float (based on our common stock), annual revenues of more than $100 million during the most recently completed fiscal year.

We may choose to take advantage of some, but not all, of these exemptions. We have taken advantage of reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. In addition, the JOBS Act provides that an emerging growth company may take advantage of an extended transition period for complying with new or revised accounting standards, delaying the adoption of these accounting standards until they would apply to private companies. We have elected to avail ourselves of the extended transition period for complying with new or revised financial accounting standards. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging growth companies which may make comparison of our financials to those of other public companies more difficult.

THE OFFERING

Common stock offered by us	[•] shares
Common stock to be outstanding after this offering	[•] (or [•] shares if the underwriters exercise their option to purchase additional shares in full)
Underwriters' option to purchase additional shares of common stock from us	[•] shares
Use of proceeds	We estimate that the net proceeds from our issuance and sale of [•] shares of our common stock in this offering will be approximately $[•] million, assuming an initial public offering price of $[•] per share, which is the midpoint of the estimated initial public offering price range set forth on the cover page of this prospectus, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that our net proceeds will be approximately $[•] million. We currently anticipate using the net proceeds from this offering, together with our existing resources, as follows: (1) to fund the remaining costs of IkT-148009 IND applications to the FDA for PD and related GI complications; (2) to fund Phase 1 trials in healthy volunteers for IkT-148009 and a Phase 1b study in treatment-naive PD patients, as well as to fund a Phase 2 clinical trial in this patient population; (3) to fund the costs to advance IkT-148009 in early-stage PD patients; (4) to optimize additional novel c-Abl inhibitors in our compound portfolio for DLB and MSA; (5) to validate target engagement markers in the central and peripheral nervous systems for all these medications; and (6) the remainder, if any, to fund general research and development activities, working capital and other general corporate activities including outstanding costs for intellectual property prosecution. See the section titled "Use of Proceeds" for additional information.
Proposed NASDAQ Capital Market trading symbol	"IKT"

You should carefully read the "Risk Factors" section of this prospectus for a discussion of factors that you should consider before deciding to invest in our common stock.

The number of shares of our common stock to be outstanding after this offering is based on the 8,919,665 shares of our common stock outstanding as of March 31, 2018, and excludes the following:

- 3,204,166 shares of common stock issuable upon exercise of options to purchase shares of common stock outstanding as of March 31, 2018, with a weighted-average exercise price of $0.85 per share;
- 102,108 shares of common stock issuable upon exercise of warrants to purchase shares of common stock outstanding as of March 31, 2018, with a weighted-average exercise price of $1.08 per share;

- 145,834 shares of common stock reserved for future issuance as of March 31, 2018, under our 2011 Equity Incentive Plan, or 2011 Plan; and
- 8,770,834 shares of common stock reserved for future issuance under our 2018 Equity Incentive Plan, or 2018 Plan, which will become effective in connection with this offering.

Unless otherwise indicated, this prospectus reflects and assumes the following:

- no exercise of outstanding options or warrants;
- no exercise by the underwriters of their option to purchase up to an additional [•] shares of our common stock from us;
- no conversion of any amounts outstanding under any convertible notes subsequent to March 31, 2018; and
- the filing and effectiveness of our amended and restated certificate of incorporation and the effectiveness of our amended and restated bylaws, which will occur immediately prior to the closing of this offering.

SUMMARY FINANCIAL DATA

The following tables summarize our financial data for the periods and as of the dates indicated. We have derived the statements of operations data for the years ended December 31, 2017 and 2016 from our audited financial statements and related notes included elsewhere in this prospectus. The statements of operations data for the three months ended March 31, 2018 and 2017 and the balance sheet data as of March 31, 2018 have been derived from our unaudited condensed financial statements and related notes included elsewhere in this prospectus and have been prepared in accordance with generally accepted accounting principles in the United States of America on the same basis as the annual audited financial statements and, in the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, necessary for the fair presentation of the financial information in those statements. Our historical results are not necessarily indicative of results that may be expected in the future, and results for the three months ended March 31, 2018 are not necessarily indicative of the results to be expected for the full year ending December 31, 2018. You should read the following summary financial data together with our financial statements and the related notes appearing elsewhere in this prospectus and the information in the sections titled "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

	Three Months Ended March 31,		Year Ended December 31,	
	2018	2017	2017	2016
	(unaudited)			
Statements of Operations Data:				
Total revenue	$ 691,197	$ 247,378	$ 2,060,937	$ 967,386
Operating expenses:				
Research and development	(585,291)	(256,890)	(1,755,692)	(846,386)
Selling, general and administrative	(218,975)	(169,783)	(710,375)	(734,288)
Loss from operations	(113,069)	(179,295)	(405,130)	(613,288)
Other Income (Expense):				
Interest expense, net	(11,915)	(6,354)	(30,945)	(15,449)
Net loss	$ (124,984)	$ (185,649)	$ (436,075)	$ (628,737)
Net loss per share of common stock, basic and diluted[(1)]	$ (0.01)	$ (0.02)	$ (0.05)	$ (0.07)
Weighted average number of shares outstanding, basic and diluted[(1)]	8,919,665	8,919,665	8,919,665	8,919,665
Pro forma net loss per share, basic and diluted (unaudited)[(1)]	[•]		[•]	
Pro forma weighted-average common shares outstanding, basic and diluted (unaudited)[(1)]	[•]		[•]	

(1) See Note 8 to our audited financial statements appearing at the end of this prospectus for details on the calculation of basic and diluted net loss per share attributable to common stockholders for the years ended December 31, 2017 and 2016 and Note 6 to our unaudited condensed financial statements for details on the calculation of basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2018 and 2017.

	As of March 31, 2018		
	Actual	Pro Forma[1]	Pro Forma As Adjusted[2]
		(unaudited)	
Balance Sheet Data:			
Cash[3]	$ 65,179	$ [•]	$ [•]
Working capital[4]	(1,486,897)	[•]	[•]
Total assets	300,529	[•]	[•]
Total liabilities	1,699,948	[•]	[•]
Accumulated deficit	(4,433,278)	[•]	[•]
Total stockholders' equity (deficit)	(1,399,419)	[•]	[•]

(1) The pro forma balance sheet data in the table above reflects the conversion of an outstanding convertible note in an aggregate principal amount of $62,500 and accrued interest of $435 as of August 17, 2018 into an aggregate of 18,774 shares of our common stock upon the closing of this offering, based on the assumed initial public offering price of $[•] and the midpoint of the price range set forth on the cover page of this prospectus.

(2) The pro forma as adjusted balance sheet data in the table above reflects the pro forma adjustments described in footnote (1) above plus the sale and issuance by us of shares of our common stock in this offering, based upon the assumed initial public offering price of $[•], the midpoint of the price range set forth on the cover page of this prospectus, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

(3) As of March 31, 2018, we had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. This amount represents additional resources to pay for certain prescribed expenses incurred after March 31, 2018 pursuant to our various notices of award from the National Institute of Health.

(4) We define working capital as current assets less current liabilities. See our condensed financial statements for further details regarding our current assets and current liabilities.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our financial statements and the related notes and the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" in this prospectus, before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risks Related to Our Business, Financial Condition and Capital Requirements

We are a preclinical drug development company with extremely limited resources, a limited operating history and have no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.

We are a preclinical drug development company that commenced operations in September 2008. We have limited facilities to conduct fundamental research and we have performed our research and development activities by collaboration with contract service providers, and contract manufacturers and by designing and developing research programs in collaboration with university-based experts who work with us to evaluate mechanism(s) of disease for which we have designed and developed product candidates. Our direct research capabilities are very limited. As of the date of this offering, we have not maintained a principal laboratory or primary research facility for the development of our product candidates. In addition, we have no products approved for commercial sale and therefore all of our revenue has been obtained solely through grants from private foundations, and state and federal grants from institutions such as the National Institutes of Health, and by conducting research services for the Department of Defense.

Drug development is a highly uncertain undertaking and involves a substantial degree of risk. Prior to and at the time of this offering, we have not initiated or completed clinical trials for any of our product candidates, obtained marketing approval for any product candidates, manufactured a commercial scale product, or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Given the highly uncertain nature of drug development, we may never initiate or complete clinical trials for any of our product candidates, obtain marketing approval for any product candidates, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization.

Our limited operating history as a company makes any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage pharmaceutical companies in rapidly evolving fields, and we have not yet demonstrated an ability to successfully overcome such risks and difficulties. If we do not address these risks and difficulties successfully, our business, operating results and financial condition will suffer.

Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in this prospectus.

The report from our independent registered public accounting firm for the year ended December 31, 2017 includes an explanatory paragraph stating that our recurring losses from operations, working capital deficit and accumulated deficit raise substantial doubt about our ability to continue as a going concern. We may seek to raise additional working capital through public equity, private equity or debt financings. Our failure to raise additional working capital, or do so on commercially favorable terms, would materially and adversely affect our business, prospects, financial condition and results of operations, and we may be unable to continue as a going concern. Future reports from our independent registered public accounting firm may also contain statements expressing substantial doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms, if at all.

Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We have never generated any revenue from product sales, we may never generate any revenue from product sales and we may fail to generate further revenue or be profitable.

We have no products approved for commercial sale and have not generated any revenue from product sales. We anticipate generating additional revenue from private foundations and state and federal grants and contracts prior to generating revenue from product sales, but such grants and contracts are not guaranteed and will not make us profitable. Our ability to successfully commercialize our existing product candidates depends on our ability to successfully obtain regulatory approvals, among other factors. Thus, we may not generate meaningful revenue until after we have successfully begun and completed clinical development and received regulatory approval for the commercial sale of a product candidate. We may never begin clinical development or receive regulatory approval for the commercial sale of a product candidate and thus may never generate further revenue.

Our ability to generate revenue and achieve profitability depends significantly on many factors, including:

- successfully competing for grant revenue from private foundations and state and federal agencies;
- successfully completing research and preclinical and clinical development of our product candidates;
- obtaining regulatory approvals and marketing authorizations for product candidates once we have successfully begun and completed clinical development and clinical trials;
- identifying, assessing, acquiring and/or developing new product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- launching and successfully commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure;
- obtaining and maintaining an adequate price for our product candidates, both in the United States and in foreign countries where our products are commercialized;
- obtaining adequate reimbursement for our product candidates from payors;
- obtaining market acceptance of our product candidates as viable treatment options;
- addressing any competing technological and market developments;
- maintaining, protecting, expanding and enforcing our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of our expenses, or when, if ever, we will be able to generate any meaningful revenue or achieve or maintain profitability. In addition, our expenses could increase beyond our current expectations if we are required by the FDA or foreign regulatory agencies to perform studies in addition to those that we currently anticipate, or if there are any delays in any of our or our future collaborators' preclinical or clinical trials or the development of any of our product candidates. Even if one or more of our product candidates is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate and ongoing compliance efforts.

Even if we are able to generate revenue from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations. Revenue from the sale of any product candidate for which regulatory approval is obtained will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to get reimbursement at any price and whether we own the commercial rights for that territory. If the number of addressable patients is not as significant as we anticipate, the indication approved by regulatory

authorities is narrower than we expect, or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations, and cause a decline in the value of our common stock, all or any of which may adversely affect our viability.

We have incurred significant net losses since our inception and anticipate that we will continue to incur net losses for the foreseeable future.

We have incurred net losses since our inception, including net losses of $628,737 and $436,075 for the years ended December 31, 2016 and 2017, respectively, and $124,984 for the three months ended March 31, 2018. As of March 31, 2018, we had an accumulated deficit of $4,433,278.

We have invested significant financial resources in research and development activities, including for our preclinical product candidates and our RAMP drug discovery program and prodrug technologies. We do not expect to generate revenue from product sales for several years, if at all. The amount of our future net losses will depend, in part, on the level of our future expenditures and our ability to generate revenue. Moreover, our net losses may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We expect to continue to incur significant expenses and increasingly higher operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue our research and discovery activities;
- continue the development of our RAMP drug discovery platform and prodrug technologies;
- progress our current and any future product candidates through preclinical and clinical development;
- initiate and conduct additional preclinical, clinical or other studies for our product candidates;
- work with our contract manufacturers to scale up the manufacturing processes for our product candidates or, in the future, establish and operate a manufacturing facility;
- change or add additional contract manufacturers or suppliers;
- seek regulatory approvals and marketing authorizations for our product candidates;
- establish sales, marketing and distribution infrastructure to commercialize any products for which we obtain approval;
- acquire or in-license product candidates, intellectual property and technologies;
- make milestone, royalty or other payments due under any license or collaboration agreements;
- obtain, maintain, protect and enforce our intellectual property portfolio, including intellectual property obtained through license agreements;
- attract, hire and retain qualified personnel;
- provide additional internal infrastructure to support our continued research and development operations and any planned commercialization efforts in the future;
- experience any delays or encounter other issues related to our operations;
- meet the requirements and demands of being a public company; and
- defend against any product liability claims or other lawsuits related to our products.

Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' deficit and working capital. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

If we fail to obtain additional financing, we may be unable to complete the development and, if approved, commercialization of our product candidates.

Our operations have required substantial amounts of cash since inception. To date, we have financed our operations primarily through revenue generated by private, state and federal grants and contracts. We anticipate submitting INDs to the FDA for PD and GI complications of PD patients in the first quarter of 2019. Developing our product candidates is expensive, and we expect to continue to spend substantial amounts as we fund our early-stage research projects, continue preclinical development of our early-stage programs and, in particular, advance our lead program candidates through preclinical development and clinical trials. Even if we are successful in developing our product candidates, obtaining regulatory approvals and launching and commercializing any product candidate will require substantial additional funding beyond the net proceeds of this offering.

As of March 31, 2018, we had $65,179 in cash. In addition, as of March 31, 2018, we had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. This amount represents additional resources to pay for certain prescribed expenses incurred after March 31, 2018 pursuant to our various notices of award from the National Institute of Health. We estimate that our net proceeds from this offering will be approximately $[•] million, assuming an initial public offering price of $[•] per share, which is the midpoint of the estimated initial public offering price range set forth on the cover page of this prospectus, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. Our estimate as to how long we expect our existing cash to be adequate to fund our operations is based on assumptions that may prove inaccurate, and we could use our available capital resources sooner than we currently expect. In addition, changing circumstances may cause us to increase our spending significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control or if we choose to expand more rapidly than we presently anticipate.

We will require additional capital for the further development and, if approved, commercialization of our product candidates. Additional capital may not be available when we need it, on terms acceptable to us, or at all. We have no committed source of additional capital. If adequate capital is not available to us on a timely basis, we may be required to significantly delay, scale back or discontinue our research and development programs or the commercialization of any product candidates, if approved, or be unable to continue or expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Furthermore, debt financing, if available, may require payment of interest and potentially involve restrictive covenants that could impose limitations on our flexibility to operate. Any difficulty or failure to successfully obtain additional funding may jeopardize our ability to continue the business and our operations.

Due to the significant resources required for the development of our programs, and depending on our ability to access capital, we must prioritize development of certain product candidates. We may expend our limited resources on programs that do not yield a successful product candidate and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We have a portfolio that applies our RAMP drug discovery platform and prodrug technology across three therapeutic areas: neurodegeneration in the brain and GI complications of PD patients, oncology and bacterial and viral disease in the brain. We seek to maintain a process of prioritization and resource allocation to maintain an optimal balance between aggressively advancing lead programs and ensuring replenishment of our portfolio.

Due to the significant resources required for the development of our programs, we must focus our programs on specific diseases and disease pathways and decide which product candidates to pursue and advance and the amount of resources to allocate to each. Our decisions concerning the allocation of

research, development, collaboration, management and financial resources toward particular product candidates or therapeutic areas may not lead to the development of any viable commercial product and may divert resources away from better opportunities. Similarly, our potential decisions to delay, terminate or collaborate with third parties in respect of certain programs may subsequently also prove to be suboptimal and could cause us to miss valuable opportunities. We may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases and disease pathways that may later prove to have greater commercial potential than those we choose to pursue, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain sole development and commercialization rights. If we make incorrect determinations regarding the viability or market potential of any or all of our programs or product candidates or misread trends in the pharmaceutical industry, in particular, for neurodegenerative diseases, our business, prospects, financial condition and results of operations could be materially adversely affected.

Our business is highly dependent on the success of our initial product candidates targeting neurodegenerative diseases. All of our product candidates will require significant nonclinical and clinical development before we can seek regulatory approval for and launch a product commercially.

Our business and future success depends on our ability to obtain regulatory approval of, and then successfully launch and commercialize, our initial product candidates targeting neurodegenerative diseases, including IkT-148009, IkT-1427 and IkT-001Pro. Our product candidates, including IkT-148009, may experience preliminary complications surrounding trial execution, such as complexities surrounding the submission and regulatory acceptance of our IND, trial design and establishing trial protocols, patient recruitment and enrollment, quality and supply of clinical doses and safety issues.

All of our product candidates are in the early stages of development and will require additional nonclinical and clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. In addition, because IkT-148009 is our lead product candidate that is not a prodrug, if IkT-148009 encounters safety, efficacy, supply or manufacturing problems, developmental delays, regulatory or commercialization issues or other problems, our development plans and business would be significantly harmed.

Risks Related to the Discovery, Development and Commercialization of Our Product Candidates

We currently contract with various research institutions to perform the research and development activities needed to develop our products, and if we ever choose to or need to find alternative research institutions, we may not be able to do so at all or, if we are able to do so, it may be costly and may cause significant delays in the development and commercialization of our products.

We do not currently own, lease or operate a principal laboratory, research and development or manufacturing facility of our own. Currently, we contract with various research institutions to perform research and development for our products, including: Johns Hopkins University, University of Massachusetts Medical School — Worcester Campus, Louisiana State University at Shreveport, and the Parkinson's Institute. Establishing our own facilities would result in significant additional expenses and may result in potential delays in testing and production. Building and operating our own production facilities would require substantial additional funds and other resources, of which there can be no assurance that we will be able to obtain. In addition, there cannot be any assurance that we would be able to enter into any arrangement with third parties to manufacture our product, if any, on acceptable terms or at all. The commercial success of products outside the United States will also be dependent on the successful completion of arrangements with future partners, licensees or distributors in each territory. There can be no assurance that we will be successful in continuing to contract with research institutions to perform research and development for our products, that we would be able to establish our own facilities should we choose to or find it necessary to do so, that we would be successful in establishing additional collaborative arrangements or that, if established, such future partners will be successful in commercializing our products.

Research, development, and commercialization of pharmaceutical products are inherently risky. We are heavily dependent on the successful use of our RAMP drug discovery program and the product candidates that emerge from it and which are undergoing preclinical development. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

We are at an early stage of development of the product candidates currently in our programs and are further developing our RAMP drug discovery program and prodrug technologies to provide future additional product candidates. To date, we have invested substantially all of our efforts and financial resources to identify, acquire intellectual property for, and develop our programs, including conducting preclinical studies in our lead programs, and providing general and administrative support for these operations. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize our product candidates, and we may fail to do so for many reasons, including the following:

- our product candidates may not successfully complete preclinical studies or begin or complete clinical trials;
- our product candidates may fail to be delivered across the Blood Brain Barrier, or BBB, and therefore may not be clinically viable for CNS diseases such as PD;
- a product candidate may on further study be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- our competitors may develop therapeutics that render our product candidates obsolete or less attractive;
- our competitors may develop alternative technologies to deliver therapeutics across the BBB that outperform our product candidates;
- the product candidates that we develop may not be sufficiently covered by intellectual property for which we hold exclusive rights;
- the product candidates that we develop may be covered by third parties' patents or other intellectual property or exclusive rights;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable or commercially attractive;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all;
- if a product candidate obtains regulatory approval, we may be unable to establish sales and marketing capabilities, or successfully market such approved product candidate, to gain market acceptance; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or governmental third party payors.

We may not be successful in our efforts to further develop current or future product candidates. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. Each of our product candidates is in the early stages of development, has not undergone clinical trials, and will require significant clinical development, management of preclinical, clinical, and manufacturing activities, regulatory approval, adequate manufacturing supply, a commercial organization, and significant marketing efforts before we generate any revenue from product sales, if at all.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations.

Preclinical development is uncertain. Our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business.

All of our product candidates are still in the preclinical stage, and their risk of failure is high. Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our planned INDs in the United States, or similar applications in other jurisdictions. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA or other regulatory authorities will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our programs or be deemed safe for clinical testing in humans. As a result, we cannot be sure that we will be able to submit INDs or similar applications for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA or other regulatory authorities allowing clinical trials to begin. If our preclinical programs experienced delays, if we were not able to submit INDs, if the FDA rejects our INDs for filing or if we experience similar setbacks from regulatory authorities in other jurisdictions, our business, prospects, financial condition and results of operations could be significantly harmed.

We have no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

Our operations to date have been limited to research, financing and staffing our company, developing our technology and developing our lead product candidate, IkT-148009, and other product candidates. Our company has never completed a clinical development program for a new interventional drug, and has not commercialized product candidates. We have not yet initiated clinical development for any of our product candidates, nor have we completed all preclinical testing necessary to advance to the clinical development phase. Our product development strategy has included attempts to create molecules through RAMP that have predictable human safety margins for the target patient population, but we have never proved that our product candidates have this safety margin in clinical studies. None of our product candidates have advanced into clinical development, late-stage development or a pivotal clinical trial and it may be years before any such trial is initiated, if at all. We cannot be certain that planned clinical trials will begin or be completed on time, if at all, that our planned development programs would be acceptable to the FDA or other regulatory authorities, or that, if approval is obtained, such product candidates can be successfully commercialized. Clinical trials and commercializing our product candidates will require significant additional financial and management resources, and reliance on third party clinical investigators, contract research organizations or CROs, consultants or collaborators. Relying on third party clinical investigators, CROs or collaborators may result in delays that are outside of our control. If our clinical development program, clinical trials or commercialization of our product candidates were to fail, it would have a material adverse effect on our business, prospects, financial condition and results of operations.

If the FDA rejects our IND, objects to the characteristics of IkT-148009 or places us on clinical hold, we will not be able to commence a Phase 1 clinical trial for IkT-148009 in the U.S., which would likely have a material adverse effect on us.

Using our lead c-Abl inhibitor, IkT-148009, we intend to evaluate both newly diagnosed PD patients, patients early in the course of their disease and patients with GI complications that arise from PD. During our pre-IND discussions with the FDA, the FDA has provided us with guidance regarding the completion of preclinical development. We may be delayed in submitting our IND to the FDA, either due to guidance provided by the FDA or due to our delay in preparing the materials necessary to submit our IND. No assurance can be given whether the FDA will further object to elements of the IND or reject the IND once we submit it. If we are successful in preparing the IND submission and if our IND does not raise objections from the FDA, we plan to commence a Phase I clinical trial for our lead product candidate, IkT-148009 while continuing additional preclinical development. The FDA may not approve our request to begin the clinical trial or may place the clinical trial program on hold after it has begun. We cannot provide assurance as to the timing of our IND submission to the FDA or whether the FDA will reject it. In the event the FDA objects to elements of our IND or rejects our IND, we will not be able to commence our clinical trial involving IkT-148009 which would have a material adverse effect on our business, prospects, financial condition and results of operations.

We have never dosed any of our product candidates in humans. Our planned clinical trials may reveal significant adverse events, toxicities or other side effects not seen in our preclinical studies and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.

In order to obtain marketing approval for any of our product candidates, we must demonstrate the safety and efficacy of the product candidate for the relevant clinical indication or indications through clinical trials as well as additional supporting data. If our product candidates are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to interrupt, delay or abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective.

We have not yet initiated any clinical trials for our product candidates or dosed any of our product candidates, including IkT-148009, in humans. We have conducted various preclinical studies of our product candidates but have not yet completed preclinical development. We cannot guarantee that any positive results in preclinical studies will successfully translate to human patients. Additionally, we cannot guarantee that additional preclinical studies will show positive results. It is not uncommon to observe results in human clinical trials that are unexpected based on preclinical testing, and many product candidates fail in clinical trials despite promising preclinical results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for their products. Subjects in our planned clinical trials may suffer significant adverse events or other side effects not observed in our preclinical studies. The observed potency and kinetics of our product candidates in preclinical studies may not be observed in human clinical trials. We have tested the dosing frequency and route of administration of our product candidates in preclinical studies, which will inform our dosing strategy for future clinical trials. However such dose and route of administration may not result in sufficient exposure or pharmacological effect in humans, and may lead to unforeseen toxicity not previously observed in preclinical testing. Further, if our planned clinical trials of our product candidates fail to demonstrate efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

If significant adverse events or other side effects are observed in any of our future clinical trials, we may have difficulty recruiting patients to the related clinical trial, patients may drop out of the trial, or we may be required to abandon the trial or our development efforts of that product candidate altogether. We, the FDA, or other applicable regulatory authorities, or an institutional review board may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in the pharmaceutical industry that initially showed therapeutic promise in early-stage studies have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the drug from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance of the approved product due to its tolerability versus other therapies. Any of these developments could materially harm our business, financial condition and prospects.

Further, if any of our product candidates obtains marketing approval, toxicities associated with our product candidates may also develop after such approval and lead to a requirement to conduct additional clinical safety trials, additional warnings being added to the labeling, significant restrictions on the use of the product or the withdrawal of the product from the market. We cannot predict whether our product candidates will cause toxicities in humans that would preclude or lead to the revocation of regulatory approval.

Positive results from early preclinical studies of our product candidates are not necessarily predictive of the results of later preclinical studies and any future clinical trials of our product candidates. If we cannot show positive results or replicate any positive results from our earlier preclinical studies of our product candidates in our later preclinical studies and future clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.

Any positive results from preclinical studies of our product candidates may not necessarily be predictive of the results from later preclinical studies and clinical trials. Similarly, even if we are able to

complete our planned preclinical studies or any future clinical trials of our product candidates according to our current development timeline, the positive results from such preclinical studies and clinical trials of our product candidates may not be replicated in subsequent preclinical studies or clinical trial results.

Many companies in the pharmaceutical industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, preclinical findings made while clinical trials were underway, or safety or efficacy observations made in preclinical studies and clinical trials, including previously unreported adverse events. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain regulatory approval.

We may not be successful in our efforts to continue to create a pipeline of product candidates or to develop commercially successful products. If we fail to successfully identify and develop additional product candidates, our commercial opportunity may be limited.

One of our strategies is to identify and pursue clinical development of additional product candidates. We currently have eight programs, all of which are in the research, discovery or preclinical stages of development. Identifying, developing, obtaining regulatory approval and commercializing additional product candidates will require substantial additional funding beyond the net proceeds of this offering and is prone to the risks of failure inherent in drug development. We may not be able to successfully identify or acquire additional product candidates, advance any of these additional product candidates through the development process, successfully commercialize any such additional product candidates, if approved. If we are unable to successfully identify, acquire, develop and commercialize additional product candidates, our commercial opportunity may be limited.

If any of our product candidates successfully completes its planned clinical trials, we plan to seek regulatory approval to market such product candidates in the United States, the European Union, or EU, and in additional foreign countries where we believe there is a viable commercial opportunity. We have never commenced, compiled or submitted an application seeking regulatory approval to market any product candidate. We may never receive regulatory approval to market any product candidates even if such product candidates successfully complete clinical trials, which would adversely affect our viability. To obtain regulatory approval in countries outside the United States, we must comply with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, chemistry, manufacturing and controls, clinical trials, commercial sales, pricing, and distribution of our product candidates. We may also rely on collaborators or partners to conduct the required activities to support an application for regulatory approval, and to seek approval, for one or more of our product candidates. We cannot be sure that collaborators or partners will conduct these activities or do so within the timeframe we desire. Even if we (or our collaborators or partners) are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdictions. If we are unable to obtain approval for our product candidates in multiple jurisdictions, our revenue and results of operations could be negatively affected.

Even if we receive regulatory approval to market any of our product candidates, whether for the treatment of neurodegenerative diseases or other diseases, we cannot assure you that any such product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives.

Investment in pharmaceutical product development involves significant risk that any product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval, and become commercially viable. We cannot provide any assurance that we will be able to successfully advance any of our product candidates through the development process or, if approved, successfully commercialize any of our product candidates.

We have concentrated much of our research and development efforts on the treatment of neurodegenerative diseases, a field that has seen limited success in drug development.

We have focused much of our research and development efforts on addressing neurodegenerative diseases. Collectively, efforts by pharmaceutical companies in the field of neurodegenerative diseases have

seen limited successes in drug development. There are currently no marketed disease-modifying therapeutic options available for patients with PD and other neurodegenerative diseases; disease-modifying therapies are therapies that would slow, stop or reverse neurodegenerative disease. While we believe our approach to therapy could be disease-modifying, no markers to quantify disease progression have been identified. Our future success may be dependent on demonstrating disease-modification for neurodegenerative diseases using our product candidates. Developing and, if approved, commercializing our product candidates for treatment of neurodegenerative diseases subjects us to a number of challenges, including engineering product candidates to cross the BBB to enable optimal concentration of the therapeutic in the brain and obtaining regulatory approval from the FDA and other regulatory authorities who have only a limited set of precedents to rely on.

Our approach to the treatment of neurodegenerative diseases aims to identify and select targets with a biochemical link to neurodegenerative diseases, identify and develop biomarkers for the intended targets, which are biological molecules found in blood, other bodily fluids or tissues that are signs of a normal or abnormal process or of a condition or disease, to select the right patient population and demonstrate target engagement, pathway engagement and impact on disease progression of our molecules, identify and develop molecules that engage the intended target, and engineer our molecules to cross the BBB and act directly in the brain. This strategy may not prove to be successful. We cannot be sure that our approach will yield satisfactory therapeutic products that are safe and effective, scalable, profitable or able to obtain regulatory approval.

Moreover, public perception of drug safety issues, including adoption of new therapeutics or novel approaches to treatment, may adversely influence the willingness of subjects to participate in clinical trials, or if approved, of physicians to subscribe to novel treatments.

If we receive authorization to conduct our planned clinical trials, we may encounter substantial delays in our planned clinical trials, or may not be able to conduct or complete our clinical trials on the timelines we expect, if at all.

If we complete our preclinical trials and receive authorization to conduct our planned clinical trials, such testing is expensive, time consuming, and subject to uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. We cannot be sure that submission of an IND, or, in the case of the EMA, a clinical trial application, or CTA, will result in the FDA or EMA allowing clinical trials to begin in a timely manner, if at all. Moreover, even if these trials begin, issues may arise that could suspend or terminate such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our future clinical trials may not be successful. Events that may prevent successful or timely initiation or completion of clinical trials include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation or continuation of clinical trials;
- delays in confirming target engagement, patient selection or other relevant biomarkers to be utilized in preclinical and clinical product candidate development;
- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- delays in identifying, recruiting and training suitable clinical investigators;
- delays in obtaining required IRB approval at each clinical trial site;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including, but not limited to, after review of an IND or amendment, CTA or amendment, or equivalent application or amendment; as a result of a new safety finding that presents unreasonable risk to clinical trial participants; a negative finding from an inspection of

our clinical trial operations or study sites; developments in trials conducted by competitors that raise FDA or EMA concerns about risk to patients broadly; or if the FDA or EMA finds that the investigational protocol or plan is clearly deficient to meet its stated objectives;

- delays in identifying, recruiting and enrolling suitable patients to participate in our clinical trials, and delays caused by patients withdrawing from clinical trials or failing to return for post-treatment follow-up;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties, or us to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's or any other regulatory authority's current good clinical practices, or cGCPs, requirements, or applicable EMA or other regulatory guidelines in other countries;
- occurrence of adverse events associated with a product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- the cost of clinical trials of our product candidates being greater than we anticipate;
- clinical trials of our product candidates producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon product development programs; and
- delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing.

Any inability to successfully initiate or complete future clinical trials could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may be required to or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA, EMA or any other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

We may in the future advance product candidates into clinical trials and terminate such trials prior to their completion, which could adversely affect our business.

Delays in the completion of any planned clinical trial of our product candidates will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of planned clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

We may encounter difficulties enrolling patients in our planned clinical trials, and our clinical development activities could thereby be delayed or otherwise adversely affected.

If we receive authorization to conduct any planned clinical trials, the timely completion of such planned clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in such trials until their conclusion. We may experience difficulties in patient enrollment in our planned clinical trials for a variety of reasons, including:

- the size and nature of the patient population;
- the patient eligibility criteria defined in the protocol, and/or certain criteria related to stage of disease progression, which may limit the patient populations eligible for our clinical trials;
- the size of the study population required for analysis of a trial's primary endpoints;
- the proximity of patients to a trial site;
- the design of a trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- competing clinical trials for similar therapies or targeting patient populations meeting our patient eligibility criteria;
- clinicians' and patients' perceptions as to the potential advantages and side effects of the product candidate being studied in relation to other available therapies and product candidates;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will not complete such trials, for any reason.

Our planned clinical trials may fail to demonstrate substantial evidence of the safety and efficacy of our product candidates, which would prevent, delay or limit the scope of regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that our product candidates are both safe and effective for use in each target indication. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies of our product candidates may not be predictive of the results of early-stage or later-stage clinical trials, and results of early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. The results of our planned clinical trials in one set of patients or disease indications may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. This is particularly true in neurodegenerative diseases, where failure rates historically have been higher than in many other disease areas. Most product candidates that begin clinical trials are never approved by regulatory authorities for commercialization.

We cannot be certain that our planned future clinical trials will be successful. Additionally, any safety concerns observed in any one of our planned clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could have a material adverse effect on our business, prospects, financial condition and results of operations.

Even if our planned clinical trials were to be successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Even if regulatory approval is secured for any of our product candidates, the terms of such approval may limit the scope and use of our product candidate, which may also limit its commercial potential.

Clinical development is a lengthy and expensive process with an uncertain outcome, and failure can occur at any stage of clinical development. If we are unable to design, conduct and complete our planned clinical trials successfully, our product candidates will not be able to receive regulatory approval.

In order to obtain FDA approval for any of our product candidates, we must submit to the FDA an NDA with substantial evidence that demonstrates that the product candidate is both safe and effective in humans for its intended use. This demonstration will require significant research, preclinical studies and clinical trials. All of our product candidates are in preclinical development. We have not undertaken clinical trials for any of our products.

Clinical trials are time-consuming, expensive and difficult to design and implement, in part because they are subject to rigorous requirements and the outcomes are inherently uncertain. Clinical testing may take many years to complete, and failure can occur at any time during the clinical trial process, even with active ingredients that have previously been approved by the FDA as safe and effective. If we receive authorization to conduct our planned clinical trials, we could encounter problems that could halt our planned clinical trials or require us to repeat such clinical trials. If patients participating in our planned clinical trials suffer drug-related adverse reactions during the course of such clinical trials, or if we or the FDA believe that patients are being exposed to unacceptable health risks, such clinical trials may have to be suspended or terminated. Suspension, termination or the need to repeat a clinical trial can occur at any stage.

The clinical trial success of each of our product candidates depends on reaching statistically significant changes in patients' symptoms based on clinician-rated scales. There is a lack of consensus regarding standardized processes for assessing clinical outcomes based on clinician-rated scales. Accordingly, the scores from our clinical trials may not be reliable, useful or acceptable to the FDA or other regulatory agencies.

Changes in standards related to clinical trial design could have a material adverse effect on our ability to design and conduct clinical trials as planned. For example, we expect to conduct clinical trials comparing our product candidates to both placebo and other approved drugs, but regulatory authorities may not allow us to compare our product candidates to a placebo in a particular clinical indication where approved products are available. In that case, both the cost and the amount of time required to conduct such a planned clinical trial could increase. The FDA may disagree with our trial design and our interpretation of data from our planned clinical trials, or may change the requirements for approval even after it has reviewed and commented on the design for our planned clinical trials. The FDA may also approve a product candidate for fewer or more limited indications than we request, or may grant approval contingent on the performance of costly post-approval clinical trials. In addition, the FDA may not approve the labeling claims or removal of certain warnings that we believe are necessary or desirable for the successful commercialization of our product candidates. Approval may be contingent on a Risk Evaluation and Mitigation Strategy, or REMS, which could have a material adverse effect on the labeling, distribution or promotion of a drug product.

Any of these delays or additional requirements could cause our product candidates to not be approved, or if approved, significantly impact the timing and commercialization of our product candidates and significantly increase our overall costs of drug development.

We face significant competition in an environment of rapid technological and scientific change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer, more advanced or more effective than ours, which may negatively impact our ability to successfully market or commercialize any product candidates we may develop and ultimately harm our financial condition.

The development and commercialization of new drug products is highly competitive. Moreover, the neurodegenerative field is characterized by strong and increasing competition, and a strong emphasis on intellectual property. Our competitors may be able to develop other compounds, drugs, cellular or gene therapies that are able to achieve similar or better results. We may face competition with respect to any product candidates that we seek to develop or commercialize in the future from major pharmaceutical companies and specialty pharmaceutical companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical companies that are currently pursuing the development of products for the treatment of the neurodegenerative disease indications for which we have research programs, including PD. Companies developing therapeutics in the neurodegenerative disease area include large companies with significant financial resources, such as Biogen, Inc., Neuropore Therapies, Inc., Celgene Corporation, Roche Holdings AG, Prothena Corporation plc, Sanofi S.A., Takeda Pharmaceutical Co. Ltd. and UCB, S.A. In addition to competition from other companies targeting neurodegenerative indications, any products we may develop may also face competition from other types of therapies, such as gene-editing therapies.

Many of our current or potential competitors, either alone or with their strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunities could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Furthermore, currently approved products could be discovered to have application for treatment of the same disease indications as our product candidates, which could give such products significant regulatory and market timing advantages over any of our product candidates. Our competitors also may obtain FDA, EMA or other regulatory approval for their products more rapidly than we may obtain approval for ours and may obtain orphan product exclusivity from the FDA for indications our product candidates are targeting, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, we could face litigation or other proceedings with respect to the scope, ownership, validity and/or enforceability of our patents relating to our competitors' products and our competitors may allege that our products infringe, misappropriate or otherwise violate their intellectual property. See "— Risks Related to Our Intellectual Property." The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize.

The manufacture of our product candidates is complex and difficulties may be encountered in production. If such difficulties are encountered or failure to meet regulatory standards occurs, our ability to provide supply of our product candidates for clinical trials or our products for patients, if approved, could be delayed or stopped, or we may be unable to maintain a commercially viable cost structure.

The processes involved in manufacturing our drug product candidates are complex, expensive, highly-regulated and subject to multiple risks. Even minor deviations from normal manufacturing processes

could result in reduced production yields, product defects and other supply disruptions. Further, as product candidates are developed through preclinical studies to potential future clinical trials towards approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives, and any of these changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials. We expect to rely on third-party manufacturers for the manufacturing of our products.

In order to conduct planned or future clinical trials of our product candidates, or supply commercial products, if approved, we will need to have them manufactured in small and large quantities. Our manufacturing partners may be unable to successfully increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities. If our manufacturing partners are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing and potential clinical trials of that product candidate may be delayed or become infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. The same risks would apply to our internal manufacturing facilities, should we in the future decide to build internal manufacturing capacity. In addition, building internal manufacturing capacity would carry significant risks in terms of being able to plan, design and execute on a complex project to build manufacturing facilities in a timely and cost-efficient manner.

In addition, the manufacturing process for any products that we may develop is subject to FDA, EMA and foreign regulatory authority approval processes and continuous oversight, and we will need to contract with manufacturers who can meet all applicable FDA, EMA and foreign regulatory authority requirements, including complying with current good manufacturing processes, or on an ongoing basis. If we or our third party manufacturers are unable to reliably produce products to specifications acceptable to the FDA, EMA or other regulatory authorities, we may not obtain or maintain the approvals we need to commercialize such products. Even if we obtain regulatory approval for any of our product candidates, there is no assurance that either we or our CMOs will be able to manufacture the approved product to specifications acceptable to the FDA, EMA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product, or to meet potential future demand. Any of these challenges could delay completion of clinical trials, require bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidate, impair commercialization efforts, increase our cost of goods, and have an adverse effect on our business, prospects, financial condition, results of operations and growth prospects.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any product candidates we may develop, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have a sales or marketing infrastructure, nor have we sold, marketed, or distributed pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a sales, marketing, and commercial support infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

Factors that may inhibit our efforts to commercialize any approved product on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, customer service, medical affairs, and other support personnel;

- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future approved products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our product revenue or the profitability of product revenue may be lower than if we were to market and sell any products we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates if approved.

Even if any product candidates we develop receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors, and others in the medical community necessary for commercial success.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third party payors, and others in the medical community. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors, and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in pivotal clinical trials and published in peer-reviewed journals;
- the potential and perceived advantages compared to alternative treatments;
- the ability to offer our products for sale at competitive prices;
- the ability to offer appropriate patient access programs, such as co-pay assistance;
- the extent to which physicians recommend our products to their patients;
- convenience and ease of dosing and administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by FDA, EMA or other comparable foreign regulatory agencies;
- product labeling or product insert requirements of the FDA, EMA or other comparable foreign regulatory authorities, including any limitations, contraindications or warnings contained in a product's approved labeling;
- restrictions on how the product is distributed;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the effectiveness of marketing and distribution efforts by us and other licenses and distributors;

- sufficient governmental third party coverage or reimbursement; and
- the prevalence and severity of any side effects.

If any product candidates we develop do not achieve an adequate level of acceptance by physicians, healthcare payors, patients and the medical community, we will not be able to generate significant revenue, and we may not become or remain profitable. The failure of any of our product candidates to find market acceptance would harm our business prospects.

Even if we are able to commercialize any product candidates, such products may become subject to unfavorable pricing regulations, third party reimbursement practices, or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, and reimbursement for new drugs vary widely from country to country. In the United States, continual legislative changes may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to successfully commercialize any products that we may develop also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Government authorities currently impose mandatory discounts for certain patient groups, such as Medicare, Medicaid and Veterans Affairs, or VA, hospitals, and may seek to increase such discounts at any time. Future regulation may negatively impact the price of our products, if they are approved for commercial sale. Increasingly, third party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, of the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. In order to get reimbursement, physicians may need to show that patients have superior treatment outcomes with our products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval. In the United States, no uniform policy of coverage and reimbursement for products exists among third party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the medicine is approved by the FDA, EMA or other comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels

already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize product candidates, and our overall financial condition.

If any of our product candidates obtain regulatory approval, additional competitors could enter the market with generic versions of such drugs, which may result in a material decline in sales of affected products.

Under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, a pharmaceutical manufacturer may file an abbreviated new drug application, or ANDA, seeking approval of a generic copy of an approved, small molecule innovator product. Under the Hatch-Waxman Act, a manufacturer may also submit an NDA under section 505(b)(2) that references the FDA's prior approval of the small molecule innovator product. A 505(b)(2) NDA product may be for a new or improved version of the original innovator product. The Hatch-Waxman Act also provides for certain periods of regulatory exclusivity, which preclude FDA approval (or in some circumstances, FDA filing and reviewing) of an ANDA or 505(b)(2) NDA. These include, subject to certain exceptions, the period during which an FDA-approved drug is subject to orphan drug exclusivity. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the "Orange Book." If there are patents listed in the Orange Book, a generic or 505(b)(2) applicant that seeks to market its product before expiration of the patents must include in the ANDA a "Paragraph IV certification," challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. Notice of the certification must be given to the innovator, too, and if within 45 days of receiving notice the innovator sues to protect its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

Accordingly, if any of our product candidates are approved, competitors could file ANDAs for generic versions of our drug products or 505(b)(2) NDAs that reference our drug products, respectively. If there are patents listed for our drug products in the Orange Book, those ANDAs and 505(b)(2) NDAs would be required to include a certification as to each listed patent indicating whether the ANDA applicant does or does not intend to challenge the patent. We cannot predict which, if any, patents in our current portfolio or patents we may obtain in the future will be eligible for listing in the Orange Book, how any generic competitor would address such patents, whether we would sue on any such patents, or the outcome of any such suit.

We may not be successful in securing or maintaining proprietary patent protection for products and technologies we develop or license. Moreover, if any of our owned or in-licensed patents that are listed in the Orange Book are successfully challenged by way of a Paragraph IV certification and subsequent litigation, the affected product could immediately face generic competition and its sales would likely decline rapidly and materially. Should sales decline, we may have to write off a portion or all of the intangible assets associated with the affected product and our results of operations and cash flows could be materially and adversely affected. See "— Risks Related to Our Intellectual Property."

Conducting any future clinical trials of our product candidates and any future commercial sales of a product candidate may expose us to expensive product liability claims, and we may not be able to maintain product liability insurance on reasonable terms or at all and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of the preclinical and future clinical testing of our product candidates and will face an even greater risk when and if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during preclinical or clinical testing, manufacturing, marketing or sale. Any such

product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit testing and commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased or interrupted demand for our products;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to continue our clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate; and
- a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. Our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

The regulatory approval processes of the FDA, EMA and comparable foreign regulatory authorities are lengthy, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, we will be unable to generate product revenue and our business will be substantially harmed.

The time required to obtain approval by the FDA, EMA and comparable foreign regulatory authorities is unpredictable, typically takes many years following the commencement of clinical trials, and depends upon numerous factors, including the type, complexity and novelty of the product candidates involved. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. We have not submitted for, or obtained regulatory approval for any product candidate, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for our product candidates could fail to receive regulatory approval for many reasons, including, but not limited to the following:

- the FDA, EMA or comparable foreign regulatory authorities may disagree with the design, implementation or results of our preclinical or clinical trials;
- the FDA, EMA or comparable foreign regulatory authorities may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use;
- the population studied in the clinical program may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;
- we may be unable to demonstrate to the FDA or comparable foreign regulatory authorities that a product candidate's risk-benefit ratio when compared to the standard of care is acceptable;
- the data collected from preclinical or clinical trials of our product candidates may not be sufficient to support the submission of an NDA, or other submission or to obtain regulatory approval in the United States or elsewhere;
- we may be unable to demonstrate to the FDA, EMA or comparable foreign regulatory authorities that a product candidate's risk-benefit ratio for its proposed indication is acceptable;
- the FDA, EMA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third party manufacturers with which we contract for preclinical, clinical and commercial supplies; and
- the approval policies or regulations of the FDA, EMA or comparable foreign regulatory authorities may significantly change in a manner rendering our preclinical or clinical data insufficient for approval.

This lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failing to obtain regulatory approval to market any of our product candidates, which would significantly harm our business, results of operations, and prospects.

Our product candidates may cause undesirable side effects or have other properties that could halt their clinical development, prevent their regulatory approval, limit their commercial potential, or result in significant negative consequences.

Adverse events or other undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA, EMA or other comparable foreign regulatory authorities. Drug-related side effects could affect patient recruitment, the ability of enrolled patients to complete the study, and/or result in potential product liability claims. We are required to maintain product liability insurance pursuant to our business practice. We may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations, prospects, and business. In addition, regardless of merit or eventual outcome, product liability claims may result in impairment of our business reputation, withdrawal of clinical trial participants, costs due to related litigation, distraction of management's attention from our primary business, initiation of investigations by regulators, substantial monetary awards to patients or other claimants, the inability to commercialize our product candidates, and decreased demand for our product candidates, if approved for commercial sale.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects or adverse events caused by such products, a number of potentially significant negative consequences could result, including, but not limited to:

- regulatory authorities may withdraw approvals of such product or impose restrictions on distribution;
- regulatory authorities may require additional warnings or contraindications on the label that could diminish the usage or otherwise limit the commercial success of the product;

- we may be required to change the way the product is administered or conduct additional clinical trials or post-approval studies;
- we may be forced to suspend marketing of the product;
- we may be required to create a REMS plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, prospects, results of operations, and prospects.

We may conduct in the future clinical trials for our product candidates outside the United States, and the FDA, EMA and applicable foreign regulatory authorities may not accept data from such trials.

We may in the future choose to conduct one or more clinical trials outside the United States, including in Europe. The acceptance of study data from clinical trials conducted outside the United States or another jurisdiction by the FDA, EMA or applicable foreign regulatory authority may be subject to certain conditions. In cases where data from foreign clinical trials are intended to serve as the basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practice; and (ii) the trials were performed by clinical investigators of recognized competence and pursuant to cGCP regulations. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical significance, must be met. Many foreign regulatory bodies have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA, EMA or any applicable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA, EMA or any applicable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval or clearance for commercialization in the applicable jurisdiction, and could significantly harm our business, prospects, financial condition, and results of operations.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, but a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA or EMA grants marketing approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate for those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we or any partner we work with fail to comply with the regulatory requirements in international markets or fail to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to extensive regulatory scrutiny.

If any of our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive requirements imposed by the FDA, EMA and comparable foreign regulatory authorities, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA, biologics license application to the FDA, or BLA or marketing authorization application, or MAA. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we receive for our product candidates will be subject to limitations on the approved indicated uses for which the product may be marketed and promoted or to the conditions of approval (including the requirement to implement a REMS), or contain requirements for potentially costly post-marketing testing. We will be required to report certain adverse reactions and production problems, if any, to the FDA, EMA and comparable foreign regulatory authorities. Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have approval. The holder of an approved NDA, BLA, or MAA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical trials to verify the safety and efficacy of our products in general or in specific patient subsets. If original marketing approval was obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical trial to confirm clinical benefit for our products. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters that would result in adverse publicity;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approvals;
- suspend any of our ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- refuse to allow us to enter into government contracts;

- seize or detain products, refuse to permit the import or export of products; or
- require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected.

In addition, the FDA's regulations, policies or guidance may change and new or additional statutes or government regulations in the United States and other jurisdictions may be enacted that could further restrict or regulate post-approval activities. We cannot predict the likelihood, nature or extent of adverse government regulation that may arise from pending or future legislation or administrative action, either in the United States or abroad. If we are not able to achieve and maintain regulatory compliance, we may not be permitted to market our products and/or product candidates, which would adversely affect our ability to generate revenue and achieve or maintain profitability.

We plan to continue to seek orphan drug designation for at least one product candidate, but we may be unable to ultimately obtain such designation or to maintain the benefits associated with orphan drug status, including market exclusivity, which may cause our revenue, if any, to be reduced.

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States when there is no reasonable expectation that the cost of developing and making available the drug or biologic in the United States will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting an NDA or BLA. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. We plan to continue to seek orphan drug designation for at least one product candidate, IkT-001Pro, following FDA request for additional information. We may be unable to ultimately obtain such a designation.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other NDA or BLA applications to market the same drug or biologic for the same indication for seven years, except in limited circumstances such as a showing of clinical superiority to the product with orphan exclusivity or if FDA finds that the holder of the orphan exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan product to meet the needs of patients with the disease or condition for which the drug was designated. As a result, even if one of our product candidates receives orphan exclusivity, the FDA can still approve other drugs that have a different active ingredient for use in treating the same indication or disease. Furthermore, the FDA can waive orphan exclusivity if we are unable to manufacture sufficient supply of our product.

Although we intend to seek a breakthrough therapy designation for IkT-148009 and may seek a breakthrough therapy designation for other product candidates in the future, we might not receive such designation, and even if we do, such designation may not lead to a faster development of any product candidate or approval process for any product candidate.

We intend to seek a breakthrough therapy designation for IkT-148009 in one or more indications or for other product candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs and biologics that have been designated as breakthrough therapies, interaction and

communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA may also be eligible for priority review if supported by clinical data at the time the NDA is submitted to the FDA.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive breakthrough therapy designation, the receipt of such designation for a product candidate may not result in a faster development of any product candidate or approval process for product candidate. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Healthcare legislative measures aimed at reducing healthcare costs may have a material adverse effect on our business and results of operations.

Third party payors, whether domestic or foreign, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Affordable Care Act, or ACA, was enacted, which, among other things addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, and provided incentives to programs that increase the federal government's comparative effectiveness research. The repeal of or changes in some or all of the ACA, and complying with any new legislation or reversing changes implemented under the ACA could be time-intensive and expensive, resulting in a material adverse effect on our business. Until the ACA is fully implemented or there is more certainty concerning the future of the ACA, it will be difficult to predict its full impact and influence on our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in 2013, and will remain in effect through 2025 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012 further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for our product candidates, if we obtain regulatory approval;
- our ability to receive or set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, lower reimbursement, and new payment methodologies. This could lower the price that we receive for any approved product. Any denial in coverage or reduction in reimbursement from Medicare or other government-funded programs may result in a similar denial or reduction in payments from private payors, which may prevent us from being able to generate sufficient revenue, attain profitability or commercialize our product candidates, if approved.

Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and negligent conduct that fails to:

- comply with the laws of the FDA, EMA and other comparable foreign regulatory authorities;
- provide true, complete and accurate information to the FDA, EMA and other comparable foreign regulatory authorities;
- comply with manufacturing standards we have established;
- comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws; or
- report financial information or data accurately or to disclose unauthorized activities to us.

If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. In particular, research, sales, marketing, education and other business arrangements in the healthcare industry are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, educating, marketing and promotion, sales and commission, certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter misconduct by employees and third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

If we fail to comply with healthcare laws, we could face substantial penalties and our business, operations and financial conditions could be adversely affected.

Healthcare providers, physicians and payors play a primary role in the recommendation and prescription of any product candidates for which we may obtain marketing approval. Our future arrangements with payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any product candidates for which we may obtain marketing approval. Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal, state and foreign healthcare laws and regulations may affect our ability to operate and expose us to areas of risk, including:

- federal civil and criminal false claims laws and civil monetary penalty laws, including the False Claims Act, which impose criminal and civil penalties, including through civil "qui tam" or

"whistleblower" actions, against individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other third party payors that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government. A person or entity does not need to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;
- the federal Physician Payments Sunshine Act, created under the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the U.S. Department of Health and Human Services under the Open Payments Program, information related to payments or other transfers of value made to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members, as well as other state and foreign laws regulating marketing activities;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including, but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third party payer, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could, despite our efforts to comply, be subject to challenge under one or more of such laws. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition

of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We and any contract manufacturers and suppliers we currently or may in the future engage are subject to numerous federal, state, and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment, and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air, and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research, product development and manufacturing efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty, and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our preclinical trials, future clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, prospects, financial condition, results of operations, and prospects.

Our business activities may be subject to the Foreign Corrupt Practices Act, or FCPA, and similar anti-bribery and anti-corruption laws.

Our business activities may be subject to the FCPA and similar anti-bribery or anti-corruption laws, regulations or rules of other countries in which we operate, including the U.K. Bribery Act. The FCPA generally prohibits offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a non-U.S. government official in order to influence official action, or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-U.S. governments. Additionally, in many other countries, the health care providers who prescribe pharmaceuticals are employed by their government, and the purchasers of pharmaceuticals are government entities; therefore, our dealings with these prescribers and purchasers are subject to regulation under the FCPA. Recently, the SEC and Department of Justice have increased their FCPA enforcement activities with respect to pharmaceutical companies. There is no certainty that all of our employees, agents, contractors, or collaborators, or those of our affiliates, will comply with all applicable laws and regulations, particularly given the high level of complexity of these laws. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers, or our employees, the closing down of our facilities, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries and could materially damage our reputation, our

brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results, and financial condition.

Risks Related to Our Reliance on Third Parties

We currently rely on and expect to continue to rely on third parties to conduct our preclinical testing, as well as any future research and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, or testing.

We currently rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct our research and preclinical testing and will rely on such third parties to conduct any future clinical trials. Any of these third parties may terminate their engagements with us or be unable to fulfill their contractual obligations. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that any future clinical trials would be conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with cGCPs for conducting, recording, and reporting the results of any future clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. We also are required to register any future clinical trials and post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions.

Our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical programs and any future clinical trials. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our preclinical or future clinical protocols, regulatory requirements or for other reasons, our preclinical and any future clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed.

Switching or adding additional CROs involves additional cost and requires management time and focus. Identifying, qualifying and managing performance of third party service providers can be difficult, time-consuming and cause delays in our development programs. In addition, there is a natural transition period when a new CRO commences work and the new CRO may not provide the same type or level of services as the original provider. If any of our relationships with our CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines.

Additionally, if these third parties do not successfully carry out their contractual duties, meet expected deadlines, or conduct our preclinical or any future clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines.

We also expect to rely on other third parties to store and distribute drug supplies for any future clinical trials. Any performance failure on the part of our distributors could delay future clinical development or marketing approval of any product candidates we may develop or commercialization of our medicines, producing additional losses and depriving us of potential product revenue.

We expect to depend in whole or in part on collaborations with third parties for the research, development and commercialization of any product candidates we may develop. If any such collaborations are not successful, we may not be able to realize the market potential of those product candidates.

We expect to work with third party collaborators in whole or in part for the development and commercialization of any product candidates we may develop. Our collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and academic institutions and commercial research organizations. If we enter into any such arrangements with any third parties, we will likely have shared or limited control over the amount and timing of resources that our collaborators dedicate to the development or potential commercialization of any product candidates we may seek to develop with them. Our ability to generate revenue from these arrangements with commercial entities will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Such collaborations pose the following risks to us:

- collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not properly obtain, maintain, enforce, or defend intellectual property or proprietary rights relating to our product candidates or research programs or may use our proprietary information in such a way as to expose us to potential litigation or other intellectual property related proceedings, including proceedings challenging the scope, ownership, validity and enforceability of our intellectual property;
- we may need the cooperation of our collaborators to enforce or defend any intellectual property we contribute to or that arises out of our collaborations, which may not be provided to us;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our product candidates or research programs or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborators may decide to not pursue development and commercialization of any product candidates we develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates or research programs if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators with marketing and distribution rights to one or more product candidates may not commit sufficient resources to the marketing and distribution of such product candidates;
- we may lose certain valuable rights under circumstances identified in our collaborations;
- collaborators may undergo a change of control and the new owners may decide to take the collaboration in a direction which is not in our best interest;
- collaborators may become bankrupt, which may significantly delay our research or development programs, or may cause us to lose access to valuable technology, know-how or intellectual property of the collaborator relating to our products, product candidates or research programs;
- key personnel at our collaborators may leave, which could negatively impact our ability to productively work with our collaborators;

- collaborations may require us to incur short- and long-term expenditures or issue securities that dilute our stockholders or disrupt our management and business;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our development or commercialization program under such collaboration could be delayed, diminished, or terminated.

We may face significant competition in seeking appropriate collaborations. Recent business combinations among biotechnology and pharmaceutical companies have resulted in a reduction of potential collaborators. In addition, the negotiation process is time-consuming and complex, and we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue.

If we enter into collaborations to develop and potentially commercialize any product candidates, we may not be able to realize the benefit of such transactions if we or our collaborator elect not to exercise the rights granted under the agreement or if we or our collaborator are unable to successfully integrate a product candidate into existing operations. In addition, if our agreement with any of our collaborators terminates, our access to technology and intellectual property licensed to us by that collaborator may be restricted or terminate entirely, which may delay our continued development of our product candidates utilizing the collaborator's technology or intellectual property or require us to stop development of those product candidates completely. We may also find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. Many of the risks relating to product development, regulatory approval, and commercialization described in this "Risk Factors" section also apply to the activities of our collaborators and any negative impact on our collaborators may adversely affect us.

We contract with third parties for the manufacture of materials for our research programs and preclinical studies and expect to continue to do so for any future clinical trials and for commercialization of any product candidates that we may develop. This reliance on third parties carries and may increase the risk that we will not have sufficient quantities of such materials or product candidates that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.

We do not currently have any manufacturing facilities. We currently rely on third party manufacturers for the manufacture of our materials for preclinical studies and expect to continue to do so, including for any future clinical trials, unless we choose to establish our own manufacturing facilities for preclinical studies, any future clinical trials and for commercial supply of any product candidates that we may develop.

We may be unable to establish any further agreements with third party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third party manufacturers, reliance on third party manufacturers entails additional risks, including:

- the possible breach of the manufacturing agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;

- reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting; and
- the inability to produce required volume in a timely manner and to quality standards.

Third party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our third party manufacturers may have little or no experience manufacturing materials that we require for our preclinical studies and future clinical trials. Our failure, or the failure of our third party manufacturers, to comply with applicable regulations could result in clinical holds on our trials, sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates, operating restrictions, and criminal prosecutions, any of which could significantly and adversely affect our business, prospects, financial condition, results of operations, and prospects.

Any product candidates that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay any future clinical development or marketing approval. We do not currently have arrangements in place for redundant supply for any of our product candidates. If any one of our current contract manufacturers cannot perform as agreed, we may be required to replace that manufacturer and may incur added costs and delays in identifying and qualifying any such replacement. Furthermore, securing and reserving production capacity with contract manufacturers may result in significant costs.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop may adversely affect our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

Manufacturing issues may arise that could increase product and regulatory approval costs or delay commercialization.

As we scale up manufacturing of our product candidates and conduct required stability testing, we may encounter product, packaging, equipment and process-related issues that may require refinement or resolution in order to proceed with any future clinical trials and obtain regulatory approval for commercial marketing. In the future, we may identify impurities, which could result in increased scrutiny by regulatory authorities, delays in our clinical programs and regulatory approval, increases in our operating expenses, or failure to obtain or maintain approval for our product candidates.

We depend on third party suppliers for key raw materials used in the manufacturing processes for our product candidates, and the loss of these third party suppliers or their inability to supply us with adequate raw materials could harm our business.

We rely on third party suppliers for the raw materials required for the production of our product candidates. Our dependence on these third party suppliers and the challenges we may face in obtaining adequate supplies of raw materials involve several risks, including limited control over pricing, availability, quality and delivery schedules. As a small company, our negotiation leverage is limited and we are likely to get lower priority than our competitors who are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements. Any supply interruption in limited or sole sourced raw materials could materially harm the ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for future clinical trials and regulatory approvals, which would have a material adverse effect on our business.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for any product candidates we develop, our competitors could develop and commercialize products or technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop, and our technology may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries for compositions of matter for each of our product candidates and any other technologies we may develop. We seek to protect our proprietary position by prosecuting intellectual property and filing patent applications in the United States and abroad relating to our product candidates, as well as other technologies that are important to our business. Given that the development of our technology and product candidates is at an early stage, our intellectual property portfolio with respect to certain aspects of our technology and product candidates is also at an early stage. We have filed patent applications on these aspects of our technology and core product candidates; however, there can be no assurance that any such patent applications will issue as granted patents. Furthermore, in some cases, we have only filed provisional patent applications on certain aspects of our technology and product candidates and each of these provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of the applicable provisional patent application. Any failure to file a non-provisional patent application within this timeline could cause us to lose the ability to obtain patent protection for the inventions disclosed in the associated provisional patent applications. Furthermore, in some cases, we may not be able to obtain issued claims covering compositions relating to our product candidates, as well as other technologies that are important to our business, and instead may need to rely on filing patent applications with claims covering a method of use and/or method of manufacture for protection of such product candidates and other technologies. There can be no assurance that any such patent applications will issue as granted patents, and even if they do issue, such patent claims may be insufficient to prevent third parties, such as our competitors, from utilizing our technology. Any failure to obtain or maintain patent protection with respect to our product candidates could have a material adverse effect on our business, prospects, financial condition, results of operations, and prospects.

If any of our owned or in-licensed patent applications do not issue as patents in any jurisdiction, we may not be able to compete effectively.

Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents. With respect to both in-licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors or other third parties.

The patent prosecution process is expensive, time-consuming, and complex, and we may not be able to file, prosecute, maintain, enforce, or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output in time to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. In addition, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our inventions and the prior art allow our inventions to be patentable over the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in any of our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical technology and product candidates would be adversely affected.

The patent position of pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are highly uncertain. Our owned or in-licensed pending and future patent applications may not result in patents being issued which protect our product candidates or other technologies or which effectively prevent others from commercializing competitive technologies and product candidates.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we own or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether our product candidates or other technologies will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, prospects, financial condition, results of operations and prospects.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. We or our licensors may be subject to a third party pre-issuance submission of prior art to the United States Patent and Trademark Office, or USPTO, or become involved in opposition, derivation, revocation, reexamination, post-grant and *inter partes* review, or interference proceedings or other similar proceedings challenging our owned or licensed patent rights. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, our owned or in-licensed patent rights, allow third parties to commercialize our product candidates or other technologies and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third party patent rights. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our or our licensor's priority of invention or other features of patentability with respect to our owned or in-licensed patents and patent applications. Such challenges may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our product candidates and other technologies. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us.

In addition, given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Some of our owned and in-licensed patents and patent applications are, and may in the future be, co-owned with third parties. For example, we co-own certain patents and patent applications relating to our prodrug technology to be applied to protein kinase inhibitors for oncology and non-oncology indications that was jointly developed with Sphaera Pharma Pte. Ltd., or Sphaera. Our exclusive rights to certain of these patents and patent applications are dependent, in part, on operating agreements between the joint owners of such patents and patent applications. If our licensors or co-owners fail to sustain the grant of exclusive licenses to us or we are otherwise unable to maintain such exclusive rights, our licensors or co-owners may be able to license these rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of our licensors and co-owners in order to enforce such patents against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on our competitive position, business, prospects, financial conditions, results of operations, and prospects.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We have entered into license agreements with third parties and may need to obtain additional licenses from others to advance our research or allow commercialization of product candidates. It is possible that we may be unable to obtain additional licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, prospects, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third party patents do not exist which might be enforced against our current technology, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

In addition, each of our license agreements, and we expect our future agreements, will impose various development, diligence, commercialization, and other obligations on us. Certain of our license agreements also require us to meet development timelines, or to exercise commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. In spite of our efforts, our licensors might conclude that we have materially breached our obligations under such license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of certain of our product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, prospects, financial conditions, results of operations, and prospects.

Moreover, disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, prospects, financial condition and results of operations. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, prospects, financial conditions and results of operations.

We may not be able to protect our intellectual property and proprietary rights throughout the world.

Filing, prosecuting, and defending patents on our product candidates and other technologies in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but enforcement is not as strong as that in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and this may have material adverse effects on our business, prospects, financial condition and results of operations.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees, and various other government fees on patents and applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned or licensed patents and applications. In certain circumstances, we rely on our licensing partners to pay these fees due to U.S. and non-U.S. patent agencies. The USPTO and various non-U.S. government agencies require compliance with several procedural, documentary, fee payment, and other similar provisions during the patent application process. We are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In some cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in a partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market with similar or identical products or technology, which could have a material adverse effect on our business, prospects, financial condition and results of operations.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to

March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, enacted in September 2011, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before us, could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or other technologies or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, prospects, financial condition and results of operations.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals is particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Issued patents covering our product candidates and other technologies could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

If we or one of our licensors initiated legal proceedings against a third party to enforce a patent covering our product candidates or other technologies, the defendant could counterclaim that such patent is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may raise claims challenging the validity or enforceability of our owned or in-licensed patents before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, inter partes review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in the revocation of, cancellation of, or amendment to our patents in such a way that they no longer cover product candidates or other technologies. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we or our licensing partners and the patent examiner were unaware during prosecution. If a third party were to

prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates or other technologies. Such a loss of patent protection would have a material adverse impact on our business, prospects, financial condition and results of operations.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our owned or in-licensed U.S. patents may be eligible for limited patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent term extension of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. Similar extensions as compensation for patent term lost during regulatory review processes are also available in certain foreign countries and territories, such as in Europe under a Supplementary Patent Certificate. However, we may not be granted an extension in the United States and/or foreign countries and territories because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is shorter than what we request, our competitors may obtain approval of competing products following our patent expiration, and could have a material adverse effect on our business, prospects, financial condition and results of operations.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets, or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates or other technologies. Litigation may be necessary to defend against these and other claims challenging inventorship or our licensors' ownership of our owned or in-licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates and other technologies. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, prospects, financial condition and results of operations.

Some intellectual property may have been discovered through government funded programs and thus may be subject to federal regulations such as "march in" rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Our in-licensed patent rights from Emory University and Duke University were each funded in part by the U.S. government and are therefore subject to certain federal regulations. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. The U.S. government's rights may also permit it to disclose the funded inventions and technology to third parties and to exercise march in rights to use or allow third parties to use the technology we have licensed that was developed using U.S. government funding. The U.S. government may exercise its march in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, or because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to

manufacture products embodying such inventions in the United States in certain circumstances and if this requirement is not waived. Any exercise by the U.S. government of such rights or by any third party of its reserved rights could have a material adverse effect on our business, prospects, financial condition, and results of operations.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our product candidates and other technologies, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology, and other proprietary information and to maintain our competitive position. Trade secrets and know-how can be difficult to protect. We expect our trade secrets and know-how to over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology, and the movement of personnel from academic to industry scientific positions. In addition, because we may collaborate with various collaborators on the development and commercialization of one or more of our product candidates and because we may rely on third parties to manufacture our product candidates, we may be required, at times, to share trade secrets with them prior to disclosing proprietary information. We seek to protect these trade secrets and other proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants as well as train our employees not to bring or use proprietary information or technology from former employers to us or in their work, and remind former employees when they leave their employment of their confidentiality obligations. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. Despite our efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. Given that our proprietary position is based, in part, on our know-how and trade secrets, if any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be materially and adversely harmed, and may have an adverse effect on our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. For example, any academic institution that we may collaborate with may be granted rights to publish data arising out of such collaboration, provided that we are notified in advance and given the opportunity to delay publication for a limited time period in order for us to secure patent protection of intellectual property rights arising from the collaboration, in addition to the opportunity to remove confidential or trade secret information from any such publication. Our existing collaborative research and development programs may require us to share trade secrets under the terms of our research and development collaborations or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third party collaborators. A competitor’s discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

We may be subject to claims that our employees, consultants, or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants, and advisors are currently or were previously employed at universities or other pharmaceutical companies, which may include competitors and potential competitors. Although we try to ensure that our employees, consultants, and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, prospects, financial condition and results of operations.

Third party claims of intellectual property infringement, misappropriation or other violation against us, our licensors or our collaborators may prevent or delay the development and commercialization of our product candidates and other technologies.

The field of discovering treatments for our target indications is highly competitive and dynamic. Due to the research and development that is taking place in this field, the intellectual property landscape is in flux, and it may remain uncertain in the future. As such, there may be significant intellectual property related litigation and proceedings relating to our owned and in-licensed, and other third party, intellectual property and proprietary rights in the future.

Our commercial success depends in part on our, our licensors' and our collaborators' ability to avoid infringing, misappropriating and otherwise violating the patents and other intellectual property rights of third parties. There is a substantial amount of complex litigation involving patents and other intellectual property rights in the pharmaceutical industry, as well as administrative proceedings for challenging patents, including interference, derivation and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. As discussed above, recently, due to changes in U.S. law referred to as patent reform, new procedures including inter partes review and post-grant review have been implemented. As stated above, this reform adds uncertainty to the possibility of challenge to our patents in the future.

Numerous U.S. and foreign issued patents and pending patent applications owned by third parties exist relating to the fields in which we are developing our product candidates. As the pharmaceutical industry expands and more patents are issued, the risk increases that our product candidates and other technologies may give rise to claims of infringement of the patent rights of others. We cannot assure you that our product candidates and other technologies that we have developed, are developing or may develop in the future will not infringe existing or future patents owned by third parties. We may not be aware of patents that have already been issued and that a third party, for example, a competitor in the fields in which we are developing our product candidates, and other technologies might assert are infringed by our current or future product candidates or other technologies, including claims to compositions, formulations, methods of manufacture or methods of use or treatment that cover product candidates or other technologies. It is also possible that patents owned by third parties of which we are aware, but which we do not believe are relevant to our product candidates or other technologies, could be found to be infringed by our product candidates or other technologies. In addition, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates or other technologies may infringe.

Third parties may have patents or obtain patents in the future and claim that the manufacture, use or sale of our product candidates or other technologies infringes upon these patents. In the event that any third party claims that we infringe their patents or that we are otherwise employing their proprietary technology without authorization and initiates litigation against us, even if we believe such claims are without merit, a court of competent jurisdiction could hold that such patents are valid, enforceable and infringed by our product candidates or other technologies. In this case, the holders of such patents may be able to block our ability to commercialize the applicable product candidate or technology unless we obtain a license under the applicable patents, or until such patents expire or are finally determined to be held invalid or unenforceable. Such a license may not be available on commercially reasonable terms or at all. Even if we are able to obtain a license, the license would likely obligate us to pay license fees or royalties or both, and the rights granted to us might be nonexclusive, which could result in our competitors gaining access to the same intellectual property. If we are unable to obtain a necessary license to a third party patent on commercially reasonable terms, we may be unable to commercialize our product candidates or other technologies, or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

Defense of infringement claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of management and other employee resources from our business, and may impact our reputation. In the event of a successful claim of infringement against us, we may be enjoined from further developing or commercializing our infringing product candidates or other technologies. In addition, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties and/or redesign our infringing product candidates or technologies, which may be impossible or require substantial time and monetary expenditure. In that event, we would be unable to further develop and commercialize our product candidates or other technologies, which could harm our business significantly.

Engaging in litigation to defend against third parties alleging that we have infringed, misappropriated or otherwise violated their patents or other intellectual property rights is very expensive, particularly for a company of our size, and time-consuming. Some of our competitors may be able to sustain the costs of litigation or administrative proceedings more effectively than we can because of greater financial resources. Patent litigation and other proceedings may also absorb significant management time. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings against us could impair our ability to compete in the marketplace. The occurrence of any of the foregoing could have a material adverse effect on our business, prospects, financial condition or results of operations.

We may become involved in lawsuits to protect or enforce our patents and other intellectual property rights, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensing partners, or we may be required to defend against claims of infringement. In addition, our patents or the patents of our licensing partners also may become involved in inventorship, priority or validity disputes. To counter or defend against such claims can be expensive and time consuming. In an infringement proceeding, a court may decide that a patent owned or in-licensed by us is invalid or unenforceable, the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §271(e)(1), or may refuse to stop the other party from using the technology at issue on the grounds that our owned and in-licensed patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our owned or in-licensed patents at risk of being invalidated or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for

development activities or any future sales, marketing, or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We have not yet registered our trademarks or trade names in any of our geographic markets, and failure to secure those registrations could adversely affect our business. Our unregistered trademarks and trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, prospects, financial condition and results of operations.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make products that are similar to our product candidates or utilize similar technology but that are not covered by the claims of the patents that we license or may own;
- we, or our current or future licensors or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our current or future pending owned or licensed patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors or other third parties;
- our competitors or other third parties might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, prospects, financial condition and results of operations.

Risks Related to Our Operations

We will need to grow the size and capabilities of our organization, and we may experience difficulties in managing this growth.

As of August 17, 2018, we had two full-time employees, and five contractors to oversee critical activities and perform services on our behalf. Due to our limited employee headcount and dependence on contractors, we have operated with our employees and contractors conducting most of their activities outside of our offices.

As our development plans and strategies develop, and as we transition into operating as a public company, we must add a significant number of additional managerial, operational, financial, and other personnel, as well as expand our facilities. Future growth will impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, retaining, and motivating additional employees and consultants;
- identifying and leasing suitable corporate, development and/or research facilities;
- managing our internal development efforts effectively, including the clinical and FDA review process for our current and future product candidates, while complying with our contractual obligations to contractors and other third parties;
- expanding our operational, financial and management controls, reporting systems, and procedures; and
- managing increasing operational and managerial complexity.

Our future financial performance and our ability to continue to develop and, if approved, commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth. Our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to manage these growth activities. Our ability to successfully manage our expected growth is uncertain given the fact that only one of our executive officers has been a full-time employee since our incorporation in June 2010. This lack of full-time experience working together as a company may adversely impact our senior management team's ability to effectively manage our business and growth.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services. These independent organizations, advisors and consultants may be employed by entities other than us, and may have commitments that limit their time, resources and availability to perform services for us. There can be no assurance that the services of these independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements if necessary. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed, or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, if at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our set of service providers, we may not be able to successfully implement the tasks necessary to further develop our product candidates and, accordingly, may not achieve our research, development, and commercialization goals.

Management has determined that we lack sufficient staff for adequate segregation of accounting functions and this may result in undetected errors within the financial statements.

Our management has identified a material weakness with respect to our process of internal control over financial reporting. Specifically, management determined that we lacked sufficient staff to adequately segregate accounting functions within its critical financial reporting applications, the related modules and

financial reporting processes. The lack of adequate segregation of accounting functions could result in undetected errors within the financial statements arising from accounting errors that may not be detected in the ordinary course of business.

We are highly dependent on our key personnel, and if we are not successful in attracting, motivating and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive pharmaceutical industries depends upon our ability to attract, motivate and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, particularly on our Chief Executive Officer, Dr. Werner, and our scientific and medical personnel, including our board of directors and scientific advisory board, many of whom have significant experience in drug development and marketing, and who could prove hard to replace. The loss of the services provided by any of our executive officers, key employees and consultants, or other scientific and medical advisors, and our inability to find suitable replacements, could result in delays in the development of our product candidates and harm our business.

We conduct our operations in Atlanta, Georgia and Boston, Massachusetts, both regions that are headquarters to many other pharmaceutical companies and many academic and research institutions. Competition for skilled personnel is intense and the turnover rate can be high, which may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. Our consultants and advisors may be engaged or employed by entities other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. We expect that we may need to recruit talent from outside of our regions, and doing so may be costly and difficult.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided and will continue to provide restricted stock and/or stock option grants that vest over time. The value to employees of these equity grants that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. Although we have employment agreements with our key employees, these employment agreements, other than for Dr. Werner, provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain “key man” insurance policies on the lives of all of these individuals or the lives of any of our other employees. If we are unable to attract and incentivize quality personnel on acceptable terms, or at all, it may cause our business and operating results to suffer.

If we engage in acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities, and subject us to other risks.

We may engage in various acquisitions and strategic partnerships in the future, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the potential issuance of our equity securities which would result in dilution to our stockholders;
- assimilation of operations, intellectual property, products and product candidates of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management’s attention from our existing product programs and initiatives in pursuing such an acquisition or strategic partnership;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and

- our inability to generate revenue from acquired intellectual property, technology and/or products sufficient to meet our objectives or even to offset the associated transaction and maintenance costs.

In addition, if we undertake such a transaction, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our computer systems, or those used by our third party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our future CROs and other contractors and consultants may be vulnerable to damage from computer viruses and unauthorized access. Although to our knowledge we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on our third party research institution collaborators for research and development of our product candidates and other third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations, and those of our third party research institution collaborators, CROs, CMOs, suppliers, and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics, and other natural or man-made disasters or business interruptions, for which we may not be insured. In addition, we rely on our third party research institution collaborators for conducting research and development of our product candidates, and they may be affected by government shutdowns or withdrawn funding. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third party manufacturers to produce and process our product candidates. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. Damage or extended periods of interruption to our facilities due to fire, natural disaster, power loss, communications failure, unauthorized entry or other events could cause us to cease or delay development of some or all of our product candidates.

Our business is subject to economic, political, regulatory and other risks associated with conducting business internationally.

Our business is subject to risks associated with conducting business internationally because some of our suppliers and collaborative relationships are located outside the United States. Accordingly, our future results could be harmed by a variety of factors, including:

- economic weakness, including inflation, or political instability in particular non-U.S. economies and markets;
- differing and changing regulatory requirements in non-U.S. countries;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;

- difficulties in compliance with non-U.S. laws and regulations;
- changes in non-U.S. regulations and customs, tariffs and trade barriers;
- changes in non-U.S. currency exchange rates and currency controls;
- changes in a specific country's or region's political or economic environment;
- trade protection measures, import or export licensing requirements or other restrictive actions by U.S. or non-U.S. governments;
- negative consequences from changes in tax laws;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- difficulties associated with staffing and managing international operations, including differing labor relations;
- potential liability under the FCPA or comparable foreign laws; and
- business interruptions resulting from geo-political actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

These and other risks associated with conducting business internationally may materially adversely affect our ability to attain profitable operations.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

As of August 17, 2018, we had federal net operating loss carryforwards of approximately $1,572,000, which will begin to expire in varying amounts beginning in 2030. Under Sections 382 and 383 of the United States Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, if a corporation undergoes an "ownership change" (generally defined as a greater than 50-percentage-point cumulative change (by value) in the equity ownership of certain stockholders over a rolling three-year period), the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change taxable income or taxes may be limited. We may experience ownership changes in connection with this offering and in the future as a result of subsequent shifts in our stock ownership, some of which are outside our control. Our net operating loss carryforwards may also be subject to limitation under state laws. Further, our ability to utilize net operating loss carryforwards of companies that we may acquire in the future may also be subject to limitations. There is also a risk that due to tax law changes, such as suspensions on the use of net operating loss carryforwards, or other unforeseen reasons, our ability to use our pre-change net operating loss carryforwards and other pre-change tax attributes to offset post-change taxable income or taxes may be subject to limitation or expire.

For losses arising after December 31, 2017, the use of such losses as net operating loss carryforwards are limited to a deduction of 80% of taxable income for the corresponding taxable year, and may not be carried back to previous taxable years. Further, such net operating loss carryforwards are not limited to a 20-year carryforward.

Recent U.S. tax legislation may adversely affect our future cash flows.

The Tax Cuts and Jobs Act, or TCJA, which was enacted into law on December 22, 2017, significantly changed the U.S. federal income taxation of U.S. corporations, including by reducing the U.S. corporate income tax rate, limiting interest deductions, permitting immediate expensing of certain capital expenditures, revising the rules governing net operating losses and repealing the deduction of certain performance-based compensation paid to an expanded group of executive officers. Many of these changes are effective immediately, without any transition periods or grandfathering for existing transactions. The legislation is unclear in many respects and could be subject to potential amendments and technical corrections, as well as interpretations and implementing regulations by the Treasury and Internal Revenue

Service, or IRS, any of which could lessen or increase certain adverse impacts of the legislation. In addition, it is unclear how these U.S. federal income tax changes will affect state and local taxation, since taxing authorities often use federal taxable income as a starting point for computing state and local tax liabilities.

While certain changes made by the TCJA may adversely affect our company, we believe that other changes, such as the reduction in the U.S. corporate income tax rate, will be beneficial. We continue to work with our tax advisors and auditors to determine the full impact that the TCJA will have on us.

We could be subject to additional income tax liabilities.

We compute our income tax provision based on enacted federal and state tax rates. As tax rates vary among jurisdictions, a change in earnings attributable to the various jurisdictions in which we operate could result in an unfavorable change in our overall tax provision. Additionally, changes in the enacted tax rates, adverse outcomes in tax audits, or any change in the pronouncements relating to accounting for income taxes could have a material adverse effect on our financial condition and results of operations.

Risks Related to This Offering and Ownership of Our Common Stock

We do not know whether a market will develop for our common stock or what the market price of our common stock will be, and, as a result, it may be difficult for you to sell your shares of our common stock.

Before this offering, there was no public trading market for our common stock. If a market for our common stock does not develop or is not sustained, it may be difficult for you to sell your shares of common stock at an attractive price or at all. We cannot predict the prices at which our common stock will trade. It is possible that in one or more future periods our results of operations and progression of our product candidates may not meet the expectations of public market analysts and investors, and, as a result of these and other factors, the price of our common stock may fall.

The market price of our common stock may be volatile, which could result in substantial losses for investors purchasing shares in this offering.

The initial public offering price for our common stock will be determined through negotiations with the underwriters. This initial public offering price may differ from the market price of our common stock after the offering. As a result, you may not be able to sell your common stock at or above the initial public offering price. Some of the factors that may cause the market price of our common stock to fluctuate include:

- results of preclinical studies, clinical trials, or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- delays in filing our INDs, or objections by the FDA as to the content of our INDs;
- failure or discontinuation of any of our product development and research programs;
- any delay of the FDA in approving, or failure to approve, the design of our planned clinical trials for our current product candidates or for any future product candidates that we may develop;
- the results of our efforts to develop additional product candidates or products;
- commencement or termination of collaborations for our product development and research programs;
- the success of existing or new competitive products or technologies;
- the level of expenses related to any of our research programs, clinical development programs, or product candidates that we may develop;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents, or other proprietary rights;

- actual or anticipated changes in estimates as to financial results, development timelines, or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders, or other stockholders;
- expiration of lock-up agreements;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical sector;
- general economic, industry, and market conditions; and
- the other factors described in this "Risk Factors" section.

In recent years, the stock market in general, and the market for pharmaceutical companies in particular, has experienced significant price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. These fluctuations may be even more pronounced in the trading market for our stock shortly following this offering. Following periods of such volatility in the market price of a company's securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. Securities litigation could result in substantial costs and divert management's attention and resources from our business.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We do not currently have and may never obtain research coverage by industry or financial analysts. If no or few analysts commence coverage of us, the trading price of our stock could decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, upon the expiration of the lock-up agreements, the early release of these agreements, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. After this offering, we will have [•] shares of common stock outstanding based on 8,919,665 shares of our common stock outstanding as of March 31, 2018. Of these shares, the [•] shares we are selling in this offering may be resold in the public market immediately. The remaining [•] shares, or [•]% of our outstanding shares after this offering, are currently prohibited or otherwise restricted under securities laws or lock-up agreements entered into by our stockholders with the underwriters. However, subject to applicable securities law restrictions and excluding shares of restricted stock that will remain unvested, these shares will be able to be sold in the public market beginning 180 days after the date of this prospectus. The representatives of the underwriters may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements at any time and for any reason. Shares held by our directors, executive officers and other affiliates will continue to be subject to certain limitations of Rule 144 under the Securities Act of 1933, as amended, or the Securities Act. Shares

issued upon the exercise of stock options outstanding under our equity incentive plans or pursuant to future awards granted under those plans will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable lock-up agreements, and Rule 144 under the Securities Act. See the section titled "Shares Eligible for Future Sale" for additional information.

Moreover, after this offering, Dr. Werner, the holder of an aggregate of 6,000,000 shares of our common stock will have rights, subject to conditions, to require us to file registration statements covering his shares or to include his shares in registration statements that we may file for ourselves or other stockholders. We also plan to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements described in the section titled "Underwriting" in this prospectus. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

You will incur immediate and substantial dilution as a result of this offering.

If you purchase common stock in this offering, you will incur immediate and substantial dilution of $[•] per share, representing the difference between the assumed initial public offering price of $[•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, and our pro forma net tangible book value per share after giving effect to this offering. As of March 31, 2018, there were 3,204,166 shares subject to outstanding options with a weighted-average exercise price of $0.85 per share. To the extent that these outstanding options are ultimately exercised or the underwriters exercise their option to purchase additional shares, you will incur further dilution. See the section titled "Dilution" for a further description of the dilution you will experience immediately after this offering.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. We, and indirectly, our stockholders, will bear the cost of issuing and servicing such securities. Because our decision to issue debt or equity securities in any future offering will depend on market conditions and other factors beyond our control, we cannot predict or estimate the amount, timing or nature of any future offerings. To the extent that we raise additional capital through the sale of equity or debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Additionally, any future collaborations we enter into with third parties may provide capital in the near term but limit our potential cash flow and revenue in the future. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.

Insiders will continue to have substantial influence over us after this offering, which could limit your ability to affect the outcome of key transactions, including a change of control.

After this offering, our directors, executive officers, holders of more than 5% of our outstanding stock and their respective affiliates will beneficially own shares representing approximately [•]% of our outstanding common stock. As a result, these stockholders, if they act together, will be able to influence our management and affairs and all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions. Dr. Werner alone will continue to beneficially own shares representing approximately [•]% of our outstanding common stock. This concentration of ownership may have the effect of delaying or preventing a change in control of our company and might affect the market price of our common stock.

We are an "emerging growth company" and a "smaller reporting company" and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include, but are not limited to: (i) exemption from compliance with the auditor attestation requirements pursuant to SOX; (ii) exemption from compliance with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements; (iii) reduced disclosure about our executive compensation arrangements; and (iv) exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We will continue to remain an emerging growth company until the earliest of the following: (i) the last day of the fiscal year following the fifth anniversary of the date of the completion of this offering; (ii) the last day of the fiscal year in which our total annual gross revenue is equal to or more than $1.07 billion; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

In addition, we are currently a "smaller reporting company," meaning that we are not an investment company, an asset-backed issuer, or a majority-owned subsidiary of a parent company that is not a "smaller reporting company," and had a public float of less than $75 million and annual revenues of less than $50 million during the most recently completed fiscal year. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including, but not limited to (i) exemption from compliance with the auditor attestation requirements pursuant to SOX; (ii) reduced disclosure about our executive compensation arrangements; (iii) the requirement to provide only two years of audited financial statements, instead of three years; and (iv) not being required to provide certain quantitative and qualitative disclosures about market risk.

As a result, the information we provide stockholders will be different than the information that is available with respect to other public companies. In this prospectus, we have not included all of the executive compensation related information that would be required if we were not an emerging growth company, nor have we included all of the quantitative and qualitative disclosures about market risk that would be required if we were not a smaller reporting company. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock, and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have opted to take advantage of this extended transition period for the adoption of certain accounting standards.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. SOX, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of NASDAQ, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance, and other personnel in connection with our becoming, and our efforts to comply with the requirements of being, a

public company, and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that the rules and regulations applicable to us as a public company may make it more difficult and more expensive for us to obtain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors. We are currently evaluating these rules and regulations and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of SOX, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC after we become a public company. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 of SOX within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented, and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404 of SOX. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

We cannot specify with certainty the particular uses of the net proceeds we will receive from this offering. Our management will have broad discretion in the application of the net proceeds, including for any of the purposes described in the section titled “Use of Proceeds” in this prospectus. Our management may spend a portion or all of the net proceeds from this offering in ways that our stockholders may not desire or that may not yield a favorable return. The failure by our management to apply these funds effectively could have a material adverse effect on our business, prospects, financial condition and results of operations. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

We do not expect to pay any dividends for the foreseeable future. Investors in this offering may never obtain a return on their investment.

You should not rely on an investment in our common stock to provide dividend income. We do not anticipate that we will pay any dividends to holders of our common stock in the foreseeable future. Instead, we plan to retain any earnings to maintain and expand our existing operations. In addition, any future credit facility we enter into, or debt instrument that we issue, may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Accordingly, investors must rely on sales of their common stock after price appreciation, which may never occur, as the only way to realize any return on their investment. As a result, investors seeking cash dividends should not purchase our common stock.

Delaware law and provisions in our amended and restated certificate of incorporation and bylaws that will become effective upon the closing of this offering might discourage, delay, or prevent a change in control of our company or changes in our management and, therefore, depress the trading price of our common stock.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering may discourage, delay, or prevent a merger,

acquisition, or other change in control that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares of our common stock. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our charter documents will:

- establish that our board of directors is divided into three classes, Class I, Class II and Class III, with each class serving staggered three year terms;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- provide that our directors may only be removed for cause;
- eliminate cumulative voting;
- authorize our board of directors to issues shares of preferred stock and determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval;
- provide our board of directors with the exclusive right to elect a director to fill a vacancy or newly created directorship;
- permit stockholders to only take actions at a duly called annual or special meeting and not by written consent;
- prohibit stockholders from calling a special meeting of stockholders;
- require that stockholders give advance notice to nominate directors or submit proposals for consideration at stockholder meetings;
- authorize our board of directors, by a majority vote, to amend the bylaws; and
- require the affirmative vote of at least 66⅔% or more of the outstanding shares of common stock to amend many of the provisions described above.

In addition, Section 203 of the General Corporation Law of the State of Delaware, or DGCL, prohibits a publicly-held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three years has owned, 15% of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner.

Any provision of our amended and restated certificate of incorporation, amended and restated bylaws, or Delaware law that has the effect of delaying or preventing a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our capital stock and could also affect the price that some investors are willing to pay for our common stock.

Our amended and restated certificate of incorporation that will become effective upon the completion of this offering provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our amended and restated certificate of incorporation that will become effective upon the completion of this offering provides that the Court of Chancery of the State of Delaware is the exclusive forum for:

- any action asserting a claim of breach of fiduciary duty;
- any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

In addition, our amended and restated certificate of incorporation provides that the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements. The forward-looking statements are contained principally in the sections entitled "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and "Business." These forward-looking statements involve a number of risks and uncertainties. We caution readers that any forward-looking statement is not a guarantee of future performance and that actual results could differ materially from those contained in the forward-looking statement. These statements are based on current expectations of future events. Such statements include, but are not limited to, statements about future financial and operating results, plans, objectives, expectations and intentions, costs and expenses, interest rates, outcome of contingencies, financial condition, results of operations, liquidity, cost savings, objectives of management, business strategies, success of competing drugs, financing, potential growth and market opportunities, product candidates, clinical trial timing and plans, clinical and regulatory pathways for our development programs, the achievement of clinical and commercial milestones, the advancement of our technologies and our proprietary, co-developed and partnered products and product candidates, and other statements that are not historical facts.

In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "would," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions. Forward-looking statements contained in this prospectus include, but are not limited to, statements about:

- the success, cost and timing of our development activities, preclinical studies and potential clinical trials;
- the extent to which any limitations that we are subject to may affect the success of our product candidates;
- the timing or likelihood of regulatory filings and approvals;
- our ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations and/or warnings in the label of any approved product candidate;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- the terms and conditions of licenses granted to us and our ability to license additional intellectual property relating to our product candidates and other technologies;
- our ability to obtain funding for our operations, including funding necessary to develop and commercialize our product candidates;
- our ability to successfully commercialize our product candidates;
- our plans and ability to establish sales, marketing and distribution infrastructure to commercialize any product candidates for which we obtain approval;
- future agreements with third parties in connection with the commercialization of our product candidates;
- the size and growth potential of the markets for our product candidates, if approved for commercial use, and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates;
- potential claims relating to our intellectual property and third party intellectual property;
- our ability to contract with third party suppliers and manufacturers and their ability to perform adequately;
- the pricing and reimbursement of our product candidates, if approved;
- the success of competing products or prodrug technologies that are or may become available;
- our ability to attract and retain key managerial, scientific and medical personnel;

- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- our financial performance;
- our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act; and
- our anticipated use of the proceeds from this offering.

We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate, and financial trends that we believe may affect our business, prospects, financial condition and results of operations, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this prospectus and are subject to a number of risks, uncertainties and assumptions described in the section titled "Risk Factors" and elsewhere in this prospectus. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and you are cautioned not to unduly rely upon these statements.

All subsequent written or oral forward-looking statements attributable to us or any person acting on our behalf are expressly qualified in their entirety by the cautionary statements contained or referred to in this section. We do not undertake any obligation to release publicly any revisions to these forward-looking statements to reflect events or circumstances after the date of this prospectus or to reflect the occurrence of unanticipated events, except as may be required under applicable law. If we do update one or more forward-looking statements, no inference should be drawn that we will make additional updates with respect to those or other forward-looking statements.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements.

MARKET, INDUSTRY AND OTHER DATA

This prospectus contains estimates, projections and other information concerning our industry, our business and the markets for our product candidates. We obtained the industry, market and similar data set forth in this prospectus from our own internal estimates and research and from academic and industry research, publications, surveys and studies conducted by third parties, including governmental agencies. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. While we believe that the data we use from third parties is reliable, we have not separately verified these data. Although we are responsible for all of the disclosure contained in this prospectus and we believe the information from third party sources included in this prospectus is reliable, such information is inherently imprecise. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section titled "Risk Factors." These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us. Further, while we believe our internal research is reliable, such research has not been verified by any third party. You are cautioned not to give undue weight to any such information, projections and estimates.

In some cases, we do not expressly refer to the sources from which data is derived. In that regard, when we refer to one or more sources of this type of data in any paragraph, you should assume that other data of this type appearing in the same paragraph is derived from the same sources, unless otherwise expressly stated or the context otherwise requires.

The sources of industry and market data contained in this prospectus include those listed below:

1. S. Brahmachari, et al., "Activation of tyrosine kinase c-Abl contributes to α-synuclein-induced neurodegeneration." *J. Clin. Invest*, 126: 2970-88 (2016).

2. X. Mao, et al., "Pathological α-synuclein transmission initiated by binding lymphocyte-activation gene 3." *Science,* 353 (2016).

3. The Michael J. Fox Foundation website (www.michaeljfox.org).

4. The Cure Parkinson's Trust website (www.cureparkinsons.org.uk).

5. Parkinson's Disease Foundation (www.pdf.org), Decisions Resources 2016 Parkinson's Report.

6. Jones J.D., et al., "Health comorbidities and cognition in 1948 patients with idiopathic Parkinson's disease." *Parkinsonism and Related Disorders*, 18:1073-1078 (2012).

7. Wright Willis, et al., "Geographic and ethnic variation in Parkinson disease: a population-based study of US Medicare beneficiaries." *Neuroepidemiology*, 34:143-151 (2012).

8. de Rijk, et al., "Prevalence of parkinsonism and Parkinson's disease in Europe: the EUROPARKINSON Collaborative Study. European Community Concerted Action on the Epidemiology of Parkinson's disease." *J Neurol Neurosurg Psychiatry*, 62:10-5 (1997).

9. Ying Zhao, et al., "Progression of Parkinson's Disease as Evaluated by Hoehn and Yahr Stage Transition Times." *Movement Disorders* 25, (6):710-716 (2010).

USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of [•] shares of our common stock in this offering will be approximately $[•] million, assuming an initial public offering price of [•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that our net proceeds will be approximately $[•] million.

A $1.00 increase (decrease) in the assumed initial public offering price of [•] per share would increase (decrease) the aggregate net proceeds to us from this offering by approximately $[•] million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1.0 million shares in the number of shares offered by us would increase (decrease) the net proceeds to us from this offering by approximately $[•] million, assuming that the assumed initial public offering price remains the same, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We do not expect that a change in the initial public offering price or the number of shares by these amounts would have a material effect on our uses of the proceeds from this offering, although it may accelerate the time at which we will need to seek additional capital.

The principal purposes of this offering are to obtain additional capital to support our operations, establish a public market for our common stock and facilitate our future access to the public capital markets. We currently anticipate that we will use the net proceeds from this offering, together with our existing resources, as follows:

- approximately $2 million to fund the remaining costs of IkT-148009 IND applications to the FDA for PD and related GI complications;
- approximately $18 million to fund Phase 1 trials in healthy volunteers for IkT-148009 and a Phase 1b study in treatment-naive PD patients, as well as fund a Phase 2 clinical trial in this patient population;
- approximately $12 million to fund the costs to advance IkT-148009 in a Phase 2 clinical trial in early-stage PD patients;
- approximately $2 million to optimize additional novel c-Abl inhibitors in our compound portfolio for DLB and MSA;
- approximately $1 million to validate target engagement markers in the central and peripheral nervous systems for all these medications; and
- the remainder, if any, to fund general research and development activities, working capital and other general corporate activities to include outstanding costs for intellectual property prosecution.

We believe opportunities may exist from time to time to expand our current business through license or acquisitions of, or investments in, complementary businesses, products or technologies. While we currently have no agreements or commitments to complete any such transaction at this time, we may use a portion of the net proceeds for these purposes.

The net proceeds from this offering, together with our cash, will not be sufficient for us to fund any of our product candidates through regulatory approval, and we will need to raise additional capital to complete the development and commercialization of our product candidates. We expect to finance our incremental cash needs through a combination of equity offerings, debt financings, working capital lines of credit, grant funding and potential licenses and collaboration agreements. The expected use of net proceeds from this offering represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials, as well as any collaborations that we may enter

into with third parties for our programs, and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds. We cannot specify with certainty all of the particular uses for the net proceeds to be received upon the closing of this offering.

Based on our current operational plans and assumptions, we expect that the net proceeds from this offering together with our existing cash will be sufficient to fund our operating expenses and capital expenditure requirements at least through the first quarter of 2020. We have based this estimate on assumptions that may prove to be incorrect, and we could use our available capital resources sooner that we currently expect. Pending use of the proceeds as described above, we intend to invest the proceeds in a variety of capital preservation investments, including interest-bearing, investment-grade instruments and U.S. government securities.

DIVIDEND POLICY

We have not declared or paid any cash dividends on our capital stock since our inception. We intend to retain future earnings, if any, to finance the operation and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future. Payment of future cash dividends, if any, will be at the discretion of our board of directors after taking into account various factors, including our financial condition, operating results, current and anticipated cash needs, the requirements and contractual restrictions of then-existing debt instruments and other factors that our board of directors deems relevant.

CAPITALIZATION

The following table sets forth our cash and capitalization as of March 31, 2018, as follows:

- on an actual basis;
- on a pro forma basis to reflect (i) the conversion of an outstanding convertible note in an aggregate principal amount of $62,500 and accrued interest of $435 as of August 17, 2018 into an aggregate of 18,774 shares of our common stock upon the closing of this offering, and (ii) the filing and effectiveness of our amended and restated certificate of incorporation, which will occur immediately prior to the closing of this offering, as if such conversion had occurred on March 31, 2018; and
- on a pro forma as adjusted basis to further reflect our issuance and sale of [•] shares of common stock in this offering at the assumed initial public offering price of $[•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this information in conjunction with our financial statements and the related notes and the sections titled "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" that are included elsewhere in this prospectus.

	As of March 31, 2018		
	Actual	Pro Forma	Pro Forma As Adjusted[(1)]
		(unaudited)	
Cash	$ 65,179	$ [•]	$ [•]
Notes payable	$ 444,523	$ [•]	$ [•]
Stockholder's equity (deficit):			
Preferred stock, par value $0.001 per share; no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized, no shares issued and outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, par value $0.001 per share; 30,000,000 shares authorized; 8,919,665 shares issued and outstanding, actual; 100,000,000 shares authorized, [•] shares issued and outstanding, pro forma; 100,000,000 shares authorized, [•] shares issued and outstanding, pro forma as adjusted	8,920	[•]	[•]
Additional paid-in capital	3,024,939	[•]	[•]
Accumulated deficit	(4,433,278)	[•]	[•]
Total stockholders' deficit	(1,399,419)	[•]	[•]
Total capitalization	$ (954,896)	$ [•]	$ [•]

Each $1.00 increase (decrease) in the assumed initial public offering price of [•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, would increase (decrease) our pro forma as adjusted cash, additional paid-in capital, total stockholders' equity (deficit) and total capitalization by approximately $[•] million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. Each increase (decrease) of 1.0 million shares in the number of shares of common stock offered by us would increase (decrease) our pro forma as adjusted cash, additional paid-in capital, total stockholders' equity (deficit) and total capitalization by approximately $[•] million, assuming the assumed initial public offering price of $[•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. The pro forma as adjusted information discussed above is illustrative only and will be adjusted based on the actual public offering price and other terms of this offering determined at pricing.

The number of shares of common stock that will be outstanding after this offering is based on 8,919,665 shares of common stock outstanding as of March 31, 2018, and excludes the following:

- 3,204,166 shares of common stock issuable upon exercise of options to purchase shares of common stock outstanding as of March 31, 2018, with a weighted-average exercise price of $0.85 per share;
- 102,108 shares of common stock issuable upon exercise of warrants to purchase shares of common stock outstanding as of March 31, 2018, with a weighted-average exercise price of $1.08 per share;
- 145,834 shares of common stock reserved for future issuance as of March 31, 2018, under our 2011 Plan; and
- 8,770,834 shares of common stock reserved for future issuance under our 2018 Plan, which will become effective in connection with this offering.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book value (deficit) as of March 31, 2018 was ($1,399,419), or $(0.16) per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities. Historical net tangible book value per share represents historical net tangible book value (deficit) divided by the number of shares of our common stock outstanding as of March 31, 2018.

Our pro forma net tangible book value as of March 31, 2018 was $[•] million, or $[•] per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the conversion of an outstanding convertible note in an aggregate principal amount of $62,500 and accrued interest of $435 as of August 17, 2018 into an aggregate of [•] shares of our common stock upon the closing of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of March 31, 2018, after giving effect to the conversion of an outstanding convertible note in an aggregate principal amount of $62,500 and accrued interest of $435 as of August 17, 2018 into an aggregate of 18,774 shares of our common stock upon the closing of this offering.

After giving further effect to our issuance and sale of [•] shares of common stock in this offering at an assumed initial public offering price of $[•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2018 would have been approximately $[•] million, or approximately $[•] per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $[•] to our existing stockholders and an immediate dilution in pro forma as adjusted net tangible book value per share of approximately $[•] to new investors purchasing common stock in this offering. Dilution per share to new investors purchasing common stock in this offering is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors.

The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share		$ [•]
Historical net tangible book value (deficit) per share as of March 31, 2018	$(0.16)	
Pro forma increase in net tangible book value (deficit) per share as of March 31, 2018	$ [•]	
Pro forma net tangible book value per share as of March 31, 2018	$ [•]	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing shares in this offering	$ [•]	
Pro forma as adjusted net tangible book value per share after this offering		$ [•]
Dilution per share to new investors purchasing shares in this offering		$ [•]

Each $1.00 increase (decrease) in the assumed initial public offering price of [•] per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted net tangible book value per share after this offering by $[•] per share and the dilution to new investors purchasing common stock in this offering by $[•] per share, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1.0 million shares in the number of shares offered by us would increase the pro forma as adjusted net tangible book value per share after this offering by $[•] and decrease the dilution per share to new investors participating in this offering by $[•], assuming no change in the assumed initial public offering price and after deducting underwriting discounts and commissions and estimated offering expenses payable

by us. A decrease of 1.0 million shares in the number of shares offered by us would decrease the pro forma as adjusted net tangible book value per share after this offering by $[•] and increase the dilution per share to new investors participating in this offering by $[•], assuming no change in the assumed initial public offering price and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their option to purchase [•] additional shares of common stock in this offering in full at the assumed initial public offering price of $[•] per share, which is the midpoint of the estimated offering price range set forth on the cover of this prospectus, and assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, the pro forma as adjusted net tangible book value per share after this offering would be $[•] per share, and the dilution in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering would be $[•] per share.

The number of shares of common stock that will be outstanding after this offering is based on 8,919,665 shares of common stock outstanding as of March 31, 2018, and excludes the following:

- 3,204,166 shares of common stock issuable upon exercise of options to purchase shares of our common stock outstanding as of March 31, 2018, with a weighted-average exercise price of $0.85 per share;
- 102,108 shares of common stock issuable upon exercise of warrants to purchase shares of common stock outstanding as of March 31, 2018, with a weighted-average exercise price of $1.08 per share;
- 145,834 shares of common stock reserved for future issuance as of March 31, 2018, under our 2011 Plan; and
- 8,770,834 shares of common stock reserved for future issuance under our 2018 Plan, which will become effective in connection with this offering, and any additional shares that become available.

The foregoing discussion does not reflect the potential purchase of any shares in this offering.

To the extent that any outstanding options are exercised or new options are issued under the equity benefit plans, or we issue additional shares of common stock or convertible securities in the future, there will be further dilution to investors participating in this offering.

SELECTED FINANCIAL DATA

The following tables summarize our selected financial data for the periods and as of the dates indicated. We have derived our selected statements of operations data for the years ended December 31, 2017 and 2016, and the balance sheets data as of December 31, 2017 and 2016, from our audited financial statements and related notes included elsewhere in this prospectus. We have derived the selected statements of operations data for the three months ended March 31, 2018 and 2017, and the balance sheet data as of March 31, 2018, from our unaudited condensed financial statements and related notes included elsewhere in this prospectus. The unaudited condensed financial statements have been prepared on the same basis as the audited financial statements and reflect, in the opinion of management, all adjustments, which include only normal, recurring adjustments that are necessary to present fairly the unaudited condensed financial statements. Our historical results are not necessarily indicative of the results that may be expected in the future, and the results for the three months ended March 31, 2018 and 2017, are not necessarily indicative of results to be expected for the full year or any other period. You should read the financial and other data below in conjunction with the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus.

	Three Months Ended March 31,		Year Ended December 31,	
	2018	2017	2017	2016
	(unaudited)			
Statements of Operations Data:				
Total revenue	$ 691,197	$ 247,378	$ 2,060,937	$ 967,386
Operating expenses:				
Research and development	(585,291)	(256,890)	(1,755,692)	(846,386)
Selling, general and administrative	(218,975)	(169,783)	(710,375)	(734,288)
Loss from operations	(113,069)	(179,295)	(405,130)	(613,288)
Other Income (Expense):				
Interest expense, net	(11,915)	(6,354)	(30,945)	(15,449)
Net loss	$ (124,984)	$ (185,649)	$ (436,075)	$ (628,737)
Net loss per share of common stock, basic and diluted[1]	$ (0.01)	$ (0.02)	$ (0.05)	$ (0.07)
Weighted average number of shares outstanding, basic and diluted[1]	8,919,665	8,919,665	8,919,665	8,919,665
Pro forma net loss per share, basic and diluted (unaudited)[1]	[•]		[•]	
Pro forma weighted-average common shares outstanding, basic and diluted (unaudited)[1]	[•]		[•]	

(1) See Note 8 to our audited financial statements appearing at the end of this prospectus for details on the calculation of basic and diluted net loss per share attributable to common stockholders for the years ended December 31, 2017 and 2016 and Note 6 to our unaudited condensed financial statements for details on the calculation of basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2018 and 2017.

	As of December 31,		As of March 31, 2018
	2017	2016	(unaudited)
Balance Sheet Data:			
Cash[(1)]	$ 16,665	$ 12,036	$ 65,179
Working capital[(2)]	(1,528,105)	(1,434,473)	(1,486,897)
Total assets	285,167	162,927	300,529
Total liabilities	1,726,175	1,514,637	1,699,948
Accumulated deficit	(4,308,294)	(3,872,219)	(4,433,278)
Total stockholders' deficit	(1,441,008)	(1,351,710)	(1,399,419)

(1) As of March 31, 2018, we had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. This amount represents additional resources to pay for certain prescribed expenses incurred after March 31, 2018 pursuant to our various notices of awards from the National Institute of Health.

(2) We define working capital as current assets less current liabilities. See our financial statements for further details regarding our current assets and current liabilities.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the section titled "Selected Financial Data" and our financial statements and the related notes to those statements included elsewhere in this prospectus. This discussion and analysis and other parts of this prospectus contain forward-looking statements based upon current beliefs, plans and expectations related to future events and our future financial performance that involve risks, uncertainties and assumptions, such as statements regarding our intentions, plans, objectives, expectations, forecasts and projections. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under the section titled "Risk Factors" and elsewhere in this prospectus. You should carefully read the "Risk Factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section titled "Special Note Regarding Forward-Looking Statements."

Overview

We are a company developing therapeutics for neurodegenerative disease inside and outside of the brain. We anticipate filing two INDs for our lead programs with the FDA in the first quarter of 2019.

Our lead programs utilize small molecule oral protein kinase inhibitors to treat PD, and its gastrointestinal complications. We have shown that our lead clinical candidate, IkT-148009, a potent, brain penetrant c-Abl protein kinase inhibitor, halts and/or reverses neurodegeneration in the brain and GI tract in preclinical models that mimic the human disease. We believe our therapeutic approach is disease-modifying.

In our opinion, the multi-decade failures in the treatment of neurodegenerative disease result from a lack of understanding of the biochemistry of the disease processes involved. Historically, symptoms of a neurodegenerative disease, like a "plaque" made up of a misfolded and/or aggregated protein(s), have been the development focus. To our knowledge, a "plaque"-focused strategy has not resulted in approval of a new medication that can alter the disease course for a neurodegenerative disease. We focus instead on the proteins that become dysfunctional in a disease pathway and seek to understand what leads to their dysfunction. In PD and other neurodegenerative diseases, our pathway-focused approach has enabled a deeper understanding of the origins of the disease and an understanding of how individual proteins are linked together to define the disease process. Using this strategy, we have discovered novel therapeutics for c-Abl, which we believe can alter the disease course for PD. Protein kinases are enzymes that modify proteins, including alpha-synuclein. Protein kinase inhibitors are small molecules that are believed to block the actions of protein kinases.

In addition to programs in neurodegeneration, our platform drug discovery and delivery technologies have identified additional opportunities, including potential treatment of bacterial or viral infections using a single agent at fixed dose and a potential near-term commercial opportunity in oncology that we believe will complete clinical development in 2019, subject to future FDA agreements, followed by completion of the FDA requirements for new drug approval in 2020. IkT-001Pro in oncology will seek to validate the pharmacology advantage of our prodrug delivery technology in a cancer patient population that is well understood. Following validation of IkT-001Pro in oncology, we believe the same pharmacology advantages could be applied to IkT-148009, our lead drug for neurodegenerative disease, to enhance clinical development. We believe we are one of the pioneers of the application of protein kinase inhibitors to non-oncology indications, including neurodegeneration and infectious diseases, as well as their more traditional role in the treatment of cancer.

Our Programs

Our portfolio is focused on developing protein kinase inhibitors to treat neurodegeneration in the brain and GI tract that arise from dysfunctional alpha-synuclein. Using IkT-148009, our lead c-Abl inhibitor, we intend to clinically evaluate the impact of c-Abl inhibition on newly diagnosed PD patients, patients early in

the course of their disease, and patients with dysphagia and/or neurogenic constipation. If there are no regulatory objections to our IND submissions of the first quarter of 2019, we intend to initiate clinical development shortly thereafter using a hybrid Phase 1/Phase 2 development approach, subject to agreements with the FDA.

We have also developed an alternate delivery approach for oral kinase inhibitors by converting them into prodrugs. We developed the oncological prodrug, IkT-001Pro, of the anticancer agent Imatinib, to alter the way a protein kinase inhibitor is absorbed in the GI tract and we believe IkT-001Pro will lead to a safer and better tolerated treatment for Imatinib-sensitive cancers. We believe demonstrating the benefits of this technology in a well-known patient population will help validate the utility of our prodrug technology broadly and may also generate revenue to support our pursuits in neurodegenerative disease. IkT-001Pro is a near-term commercial opportunity requiring a single 12 – 24 patient trial, subject to future FDA agreements, that we believe will complete the requirements for submission of an NDA in 2020.

Additional research programs will seek to develop medications for other alpha-synuclein-related diseases, specifically DLB, and MSA as well as our programs in anti-infectives that target host-factors to block viral or bacterial infections with a single agent at fixed dose. Our first application intends to treat infectious disease is to suppress JC virus infection, the cause of PML.

Our Strategy

- **Identification and characterization of the pathway(s) governing neurodegenerative disease:** We select our therapeutic targets by identification and characterization of disease pathways that we believe drive neurodegenerative disease and elucidate the biochemistry of pathway proteins to enable small molecule targeting to treat PD and related disorders, often involving clinically validated targets.
- **Proprietary method of drug discovery in neurodegeneration:** We use our RAMP method to imprint the properties we desire from an approved medication onto a new molecular entity for treatment inside and outside of the brain. Using RAMP, we believe we can “pre-determine” the pharmacology and side effect profile of our product candidates using an existing medication as a template.
- **Delivering neurodegenerative treatments as a prodrug to improve pharmacology and safety:** A prodrug is a compound that, after administration, is metabolized by the body into a pharmacologically active drug. Our prodrug technology has been shown in animal models to suppress GI and other adverse events commonly associated with oral protein kinase inhibitors and improve drug absorption from the GI tract. We believe this technology both reduces side effects and enhances drug distribution into the target tissues, which we believe will improve safety and tolerability of our protein kinase inhibitors for neurodegenerative and other diseases.

We believe that the application of these principles will significantly increase the probability of our success and will shorten the time required to bring effective therapeutics to patients with neurodegenerative and other diseases.

Components of Operating Results

Operating Expenses

Research and Development

Research and development activities account for a significant portion of our operating expenses. We record research and development expenses as incurred. Research and development expenses incurred by us for the discovery and development of our product candidates and prodrug technologies include:

- external research and development expenses, including: expenses incurred under arrangements with third parties, such as CROs, preclinical testing organizations, CMOs, academic and non-profit institutions and consultants;
- fees related to our license and collaboration agreements;

- personnel related expenses, including salaries, benefits and non-cash stock-based compensation expense; and
- other expenses, which include direct and allocated expenses for laboratory, facilities and other costs.

A portion of our research and development expenses are direct external expenses, which we track on a program-specific basis from inception of the program.

Program expenses include expenses associated with our most advanced product candidates and the discovery and development of compounds that are potential future candidates. We also track external expenses associated with our third party research and development efforts. All external costs are tracked by therapeutic indication. We do not track personnel or other operating expenses incurred for our research and development programs on a program-specific basis. These expenses primarily relate to salaries and benefits and stock-based compensation and office consumables.

At this time, we can only estimate the nature, timing and costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from sales or licensing of our product candidates. This is due to the numerous risks and uncertainties associated with drug development, including the uncertainty of:

- our ability to add and retain key research and development personnel and other key employees;
- our ability to successfully file IND and NDA applications with the FDA;
- our ability to establish an appropriate safety profile with IND-enabling toxicology studies;
- our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize, our product candidates;
- our successful enrollment in and completion of future clinical trials;
- the costs associated with the development of any additional product candidates we identify in-house or acquire through collaborations;
- our ability to discover, develop and utilize biomarkers to demonstrate target engagement, pathway engagement and the impact on disease progression of our molecules;
- our ability to establish agreements with third party manufacturers for clinical supply for any future clinical trials and commercial manufacturing, if our product candidates are approved;
- the terms and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder;
- our ability to obtain and maintain patent, trade secret and other intellectual property protection and regulatory exclusivity for our product candidates if and when approved;
- our receipt of marketing approvals from applicable regulatory authorities;
- our ability to commercialize products, if and when approved, whether alone or in collaboration with others; and
- the continued acceptable safety profiles of the product candidates following approval.

A change in any of these variables with respect to the development of any of our product candidates would significantly change the costs, timing and viability associated with the development of that product candidate. We expect our research and development expenses to increase at least over the next several years as we continue to implement our business strategy, advance our current programs, expand our research and development efforts, seek regulatory approvals for any product candidates that successfully complete clinical trials, access and develop additional product candidates and incur expenses associated with hiring additional personnel to support our research and development efforts. In addition, product candidates in later stages of clinical development generally incur higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

Selling, General and Administrative

Selling, general and administrative expenses include personnel related expenses, such as salaries, benefits, travel and non-cash stock-based compensation expense, expenses for outside professional services and allocated expenses. Outside professional services consist of legal, accounting and audit services and other consulting fees. Allocated expenses consist of rent expenses related to our offices in Boston, Massachusetts and Atlanta, Georgia not otherwise included in research and development expenses.

We expect to incur additional expenses as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the SEC and those of any national securities exchange on which our securities are traded, additional insurance expenses, investor relations activities and other administrative and professional services. We also expect to increase our administrative headcount when operating as a public company and as we advance our product candidates through clinical development, which will also likely require us to increase our selling, general and administrative expenses.

Interest Expense, Net

Interest expense, net, consists primarily of interest income and investment income earned on our cash and our interest expenses related to outstanding debt instruments to McDaniel & Associates, PC and Flagship Consulting, Inc., and debt instruments to Dr. Mueller, Mr. Fante and the Georgia Research Alliance. Each of the debt instruments to Dr. Mueller, Mr. Fante and the Georgia Research Alliance has subsequently been converted into shares of our common stock. The debt instrument to Flagship Consulting, Inc. is subject to a conversion right for conversion of the unpaid principal and accrued interest into shares of our common stock.

Results of Operations

Comparison of the Three Months Ended March 31, 2018 and 2017

The following table sets forth the significant components of our results of operations:

	Three Months Ended March 31,		Change	
	2018	2017	($)	(%)
	(unaudited)			
Grant revenue	$ 691,197	$ 246,312	$444,885	180.6
Consulting revenue	—	1,066	(1,066)	(100.0)
Research and development	(585,291)	(256,890)	328,401	127.8
Selling, general and administrative	(218,975)	(169,783)	49,192	29.0
Loss from operations	(113,069)	(179,295)	(66,226)	(36.9)
Interest expense, net	(11,915)	(6,354)	5,561	87.5
Net loss	$(124,984)	$(185,649)	$ (60,665)	(32.7)

Grant Revenue

Grant revenue for the three months ended March 31, 2018 increased by $444,885 or 180.6% to $691,197 from $246,312 in the comparable period in 2017. The increase was driven primarily by the addition of revenue from three new grants awarded during 2017 partially offset by decreased revenue from winding down of grant awards in existence during 2016.

Research and Development

Research and development expenses for the three months ended March 31, 2108 increased by $328,401 or 127.8% to $585,291 from $256,890 in the comparable period in 2017. The increase was driven by approximately $37,000 increased non-cash charges for equity compensation related to expensing of equity awards and $318,000 of increased research and development activities related to increased grant revenue partially offset by decreases in independent research and development activities not associated with grant revenue of approximately $27,000.

Selling, General and Administrative

Selling, general and administrative expenses for the three months ended March 31, 2018 increased by $49,192 or 29.0% to $218,975 from $169,783 in the comparable period in 2017. The increase was driven by approximately $54,000 in increased legal and professional fees, approximately $8,000 in increased non-cash charges for equity compensation related to expensing of equity awards, partially offset by decreases of approximately $10,000 in advertising and promotion costs and a net decrease in all other general and administrative costs of approximately $3,000.

Interest Expense, Net

Interest expense, net for the three months ended March 31, 2018 increased by $5,561 or 87.5% to $11,915 from $6,354 during the comparable period in 2017. The increase was due to increases in outstanding note payable balances related to new notes issued during second and third quarters of 2017 plus increased interest expense related to higher average balances on the Company's credit cards used for operating expenses partially offset by lower principal balances in other older term loans.

Comparison of the Years Ended December 31, 2017 and 2016

The following table sets forth the significant components of our results of operations:

	Year Ended December 31,		Change	
	2017	2016	($)	(%)
Grant revenue	$ 2,059,871	$ 967,386	$1,092,485	112.9
Consulting revenue	1,066	—	1,066	100.0
Research and development	(1,755,692)	(846,386)	909,306	107.4
Selling, general and administrative	(710,375)	(734,288)	(23,913)	(3.3)
Loss from operations	(405,130)	(613,288)	(208,158)	(33.9)
Interest expense, net	(30,945)	(15,449)	15,496	100.3
Net loss	$ (436,075)	$(628,737)	$ (192,662)	(30.6)

Grant Revenue

Grant revenue for the year ended December 31, 2017 increased by $1,092,485 or 112.9% to $2,059,871 from $967,386 in the prior year. The increase was driven primarily by the addition of approximately $1,208,000 in new revenue from grants awarded during 2017 partially offset by decreased revenue of approximately $116,000 from grant awards in existence during 2016.

Research and Development

Research and development expenses increased by $909,306 or 107.4% to $1,755,692 from $846,386 in the prior year. The increase was driven by approximately $878,000 from increased research and development activities related to increased grant revenue plus an approximately $79,000 increase in non-cash charges for equity compensation related to expensing of equity awards, partially offset by decreases in independent research and development activities not associated with grant revenue of approximately $48,000.

Selling, General and Administrative

Selling, general and administrative expenses decreased by $23,913 or 3.3% to $710,375 from $734,288 in the prior year. The decrease was driven by approximately $42,000 in decreased non-cash charges for equity compensation related to expensing of equity awards, decreases of approximately $30,000 in legal and professional fees partially offset by an increase of approximately $61,000 in advertising and promotion costs and a net decrease in all other general and administrative costs of approximately $13,000.

Interest Expense, Net

Interest expense, net increased by $15,496 or 100.3% to $30,945 from $15,449 in the prior year. The increase was due to increases in average outstanding notes payable balances related to new notes issued during the second and third quarter of 2017 plus increased interest expense related to higher average balances on the Company's credit cards used for operating expenses partially offset by lower principal balances in other older term loans.

Liquidity and Capital Resources

Sources of Liquidity

From our inception through March 31, 2018, we have funded our operations primarily through private, state and federal contracts and grants. From our inception through March 31, 2018, we generated aggregate cash proceeds of approximately $15,466,000 from private, state and federal contracts and grants. As of March 31, 2018, we had cash in the amount of $65,179. In addition, as of March 31, 2018, we had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. This amount represents additional resources to pay for certain prescribed expenses incurred after March 31, 2018 pursuant to our various notices of award from the National Institute of Health. We expect the trend of financing our operations through grants to continue.

Future Funding Requirements

To date, we have not generated any revenue from the sale of commercial products. We do not expect to generate any significant revenue from product sales unless and until we obtain regulatory approval of and successfully commercialize any of our product candidates and we do not know when, or if, this will occur. We expect to continue to incur significant losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any future approved products. We are subject to all of the risks typically related to the development of new product candidates, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. Moreover, following the completion of this offering, we expect to incur additional costs associated with operating as a public company. We anticipate that we will need substantial additional funding in connection with our continuing operations.

Until we can generate a sufficient amount of revenue from the commercialization of our product candidates, if ever, we expect to finance our incremental cash needs through a combination of equity offerings, debt financings, working capital lines of credit, grant funding and potential licenses and collaboration agreements. Additional working capital may not be available on commercially reasonable terms, if at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, reduce or discontinue the development or commercialization of one or more of our product candidates. If we raise additional funds through the issuance of additional debt or equity securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and the existence of securities with rights that may be senior to those of our common stock. If we incur indebtedness, we could become subject to covenants that would restrict our operations and potentially impair our competitiveness, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Additionally, any future collaborations we enter into with third parties may provide capital in the near term but limit our potential cash flow and revenue in the future. Any of the foregoing could have a material adverse effect on our business, financial condition and results of operations.

Since our inception, we have incurred significant losses and negative cash flows from operations. We have an accumulated deficit of $4,433,278 through March 31, 2018. We expect to incur substantial additional losses in the future as we conduct and expand our research and development activities.

We may seek to fund our operations through public equity or private equity or debt financings, as well as other sources. However, we may be unable to raise additional working capital, or if we are able to raise additional working capital we may be unable to do so on commercially favorable terms. Our failure to raise

capital or enter into such other arrangements if and when needed would have a negative impact on our business, results of operations and financial condition and our ability to continue to develop our product candidates.

As of March 31, 2018, we had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. However, as certain elements of our operating plan are outside of our control, including the receipt of anticipated grants and funding from this offering, they cannot be considered probable. If we do not receive additional working capital from future anticipated grants and future anticipated capital raises, our existing resources are projected to be sufficient to fund our operations through May 31, 2019.

These conditions raise substantial doubt regarding our ability to continue as a going concern for a period of one year after the date the financial statements included in this prospectus are issued. Our management's plans to alleviate the conditions that raise substantial doubt include delaying certain research projects and capital expenditures and eliminating certain future operating expenses in order to fund operations at reduced levels for us to continue as a going concern for a period of 12 months from the date the financial statements are issued. Our management has concluded that the success of its plan to obtain sufficient funding from one or more of these sources or to adequately reduce expenditures, while reasonably possible, is less than probable. Accordingly, we have concluded that substantial doubt exists about our ability to continue as a going concern for a period of at least 12 months from the date of issuance of the financial statements included in this prospectus.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainties described above.

The expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. However, we have based these estimates on assumptions that may prove to be wrong, and we could deplete our working capital sooner than planned.

The timing and amount of our operating expenditures will depend largely on:

- the timing and progress of preclinical and future clinical development activities;
- the number and scope of preclinical and future clinical programs we decide to pursue;
- the progress of the development efforts of third parties with whom we have entered into license and collaboration agreements;
- our ability to maintain our current research and development programs and to establish new research and development, license or collaboration arrangements;
- our ability and success in securing manufacturing relationships with third parties or, in the future, in establishing and operating a manufacturing facility;
- the costs involved in prosecuting, defending and enforcing patent claims and other intellectual property claims;
- the cost and timing of regulatory approvals;
- our efforts to enhance operational, financial and information management systems and hire additional personnel, including personnel to support development of our product candidates; and
- the costs and ongoing investments to in-license and/or acquire additional technologies.

A change in the outcome of any of these or other variables with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. Furthermore, our operating plans may change in the future, and we may need additional funds to meet operational needs and capital requirements associated with such operating plans.

Cash Flows

The following table sets forth a summary of the primary sources and uses of cash for each of the periods presented below:

	Year Ended December 31,		Three Months Ended March 31,	
	2017	2016	2018	2017
Cash provided by (used in) operating activities	$ (126,391)	$ (29,573)	$ 63,953	$ 94,098
Cash provided by (used in) financing activities	131,020	(23,769)	(15,439)	(10,045)
Net increase (decrease) in cash	$ 4,629	$ (53,342)	$ 48,514	$ 84,053

Net Cash Flows Used in or Provided by Operating Activities

Net cash flows used in operating activities for the year ended December 31, 2017 totaled $126,391, and consisted primarily of a net loss of $436,075 adjusted for non-cash stock compensation of $300,659, non-cash warrant expense of $46,118 and a net change in operating assets and liabilities of $37,093.

Net cash flows used in operating activities for the year ended December 31, 2016 totaled $29,573, and consisted primarily of a net loss of $628,737 adjusted for non-cash stock compensation of $310,088 and a net change in operating assets and liabilities of $290,214.

Net cash flows provided by operating activities for the three months ended March 31, 2018 totaled $63,953, and consisted primarily of a net loss of $124,984 adjusted for non-cash stock compensation of $166,574 and a net change in operating assets and liabilities of $22,363.

Net cash flows provided by operating activities for the three months ended March 31, 2017 totaled $94,098 and consisted primarily of a net loss of $185,649 adjusted for non-cash stock compensation of $75,185, non-cash warrant expense of $46,118 and a net change in operating assets and liabilities of $158,444.

Cash Used in or Provided by Financing Activities

Net cash flows provided by financing activities for the year ended December 31, 2017 totaled $131,020, which included proceeds from issuance of notes payable in the amount of $150,000 partially offset by repayments of notes payable of $18,980.

Net cash flows used in financing activities for the year ended December 31, 2016 totaled $23,769, which consisted of repayments of notes payable.

Net cash flows used in financing activities for the three months ended March 31, 2018 totaled $15,439, which included repayments of notes payable.

Net cash flows used in financing activities for the three months ended March 31, 2017 totaled $10,045, which included increases of notes payable and increase in amounts due from shareholder of $3,955 and $14,000, respectively.

Since our inception through March 31, 2018, we have raised an aggregate of approximately $15.5 million in net proceeds, through awarded grants or contracts.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements.

Contractual Obligations and Commitments

In June 2018, the Company entered into a one-year, non-cancelable operating lease for space in Boston, Massachusetts. The total lease obligation is $54,000, payable in 12 equal monthly installments commencing August 1, 2018.

Critical Accounting Policies and Significant Judgments and Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. While our significant accounting policies are described in more detail in the notes to our financial statements included elsewhere in this prospectus, we believe that the following accounting policies are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Research and Development Expenses

We record research and development expenses to operations as incurred. Research and development expenses represent costs incurred by us for the discovery and development of our product candidates and the development of our RAMP drug discovery program and prodrug technologies and include: employee-related expenses, including salaries, benefits, travel and non-cash stock-based compensation expense; external research and development expenses incurred under arrangements with third parties, such as CROs, preclinical testing organizations, CMOs, academic and non-profit institutions and consultants; costs to acquire technologies to be used in research and development that have not reached technological feasibility and have no alternative future use; license fees; and other expenses, which include direct and allocated expenses for laboratory, facilities and other costs.

As part of the process of preparing financial statements, we are required to estimate and accrue expenses. A portion of our research and development expenses are external costs, which we track on a program-specific basis. We record the estimated expenses of research and development activities conducted by third party service providers as they are incurred and provided within research and development expense in the statements of operations. These services include the conduct of preclinical studies and consulting services. These costs are a significant component of our research and development expenses.

Costs for research and development activities are recognized based on costs incurred. We make significant judgments and estimates in determining the accrued balance in each reporting period. As actual costs become known, we adjust our accrued estimates. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed may vary from our estimates and could result in us reporting amounts that are too high or too low in any particular period. Our accrued expenses are dependent, in part, upon the receipt of timely and accurate reporting from external clinical research organizations and other third party service providers. Due to the nature of estimates, we cannot assure you that we will not make changes to our estimates in the future as we become aware of additional information about the status or conduct of our clinical trials and other research activities.

Stock-Based Compensation

We have granted stock-based awards, consisting of non-qualified stock options, to our employees, certain non-employee consultants and members of our board of directors, both past and present. We measure stock-based compensation expense for stock options granted to our employees and directors on the date of grant and recognize the corresponding compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. We account for stock-based compensation arrangements with non-employee consultants using a fair value approach. The estimated fair value of unvested options granted to non-employee consultants is remeasured at each reporting date through the date of final vesting. As a result, the noncash charge to operations for nonemployee options with vesting conditions is affected in each reporting period by changes in the estimated fair value of our common stock. We adjust for actual forfeitures as they occur.

We estimate the fair value of stock options granted to our employees and directors on the grant date, and the resulting stock-based compensation expense, using the reduced Net Product Value, or rNPV, option-pricing model as performed by an independent third party consultant. For options or warrants granted to non-employee consultants, the fair value of these options is also remeasured using the rNPV option-pricing model reflecting the same assumptions as applied to employee options in each of the reported periods, other than the expected life, which is assumed to be the remaining contractual life of the option.

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by an independent third party consultant using an rNPV process. These factors include, but are not limited to: our most recently available valuations of our common stock by an unrelated third party; our results of operations, financial position and capital resources; current business conditions and projections; the lack of marketability of our common stock; the hiring of key personnel and the experience of management; the risk inherent in the development of our products; our stage of development and material risks related to its business; the fact that the option grants involve illiquid securities in a private company; and the likelihood of achieving a liquidity event, such as an initial public offering or sale, in light of prevailing market conditions.

All of our common stock valuations prior to our initial public offering have been prepared by an independent third party consultant using the rNPV method.

Following the closing of this offering, our board of directors, advised by an independent third party consultant, will determine the fair market value of our stock-based awards based on the closing price of our common stock as reported on the date of grant on the primary stock exchange on which our common stock is traded.

The intrinsic value of all outstanding options as of [•] was approximately $[•], based on the assumed initial public offering price of $[•] per share, which is the midpoint of the estimated initial public offering price range set forth on the cover page of this prospectus, of which approximately $[•] is related to vested options and approximately $[•] million is related to unvested options.

JOBS Act

The JOBS Act permits an emerging growth company such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to avail ourselves of the extended transition period for complying with new or revised financial accounting standards.

We will remain an emerging growth company until the earliest of (i) the last day of our first fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the date on which we are deemed to be a "large accelerated filer" under the rules of the SEC with at least $700.0 million of outstanding equity securities held by non-affiliates; (iii) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the previous three years; or (iv) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering.

Quantitative and Qualitative Disclosures About Market Risk

As a smaller reporting company, we are not required to provide disclosure regarding quantitative and qualitative market risk.

Recent Accounting Pronouncements

The JOBS Act permits an emerging growth company such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

Accounting Standards Adopted

In March 2016, the FASB released ASU No. 2016-09,*Improvements to Employee Share-Based Payment Accounting* ("ASU 2016-09"), which is intended to simplify income tax accounting for excess tax benefits, accounting for forfeitures, and employer statutory withholding. Under the current guidance, excess tax benefits that result from an award vesting or settling are recognized in additional paid-in capital in the period that they reduce cash taxes payable. This requires the provision to be computed on a with and without option basis and may result in net operating loss and credit carryforwards on the balance sheet being less than what is available on the tax return. Under the new guidance, the income tax effects of awards will be recognized as a component of income tax expense when the awards vest or are settled (regardless if cash taxes are reduced). For interim reporting purposes, companies will account for excess tax benefits and tax deficiencies as discrete items in the period during which they occurred. The guidance is effective for public entities for fiscal years beginning after December 15, 2016 and interim periods within those years, and after December 31, 2017 and interim periods beginning after December 31, 2018 for all other entities. Early adoption is permitted; however, all of the guidance included in the update must be applied when adopted. We must use a modified retrospective transition method for adopting and record the cumulative effect of all unrecognized benefits and any change in valuation allowances at the end of the prior tax period as an adjustment to retained earnings. Our adoption of this standard did not have a material effect on our financial statements.

In March 2016, the FASB issued ASU No. 2016-06,*Derivatives and Hedging (Topic 815): Contingent Put and Call Options in Debt Instruments* ("ASU 2016-06"), which applies to all issuers of or investors in debt instruments with embedded call or put options. ASU 2016-06 clarifies the requirements for assessing whether contingent call or put options that can accelerate the payment of principal on debt instruments are clearly and closely related to their debt hosts. Entities performing the assessment under the guidance of ASU 2016-06 are required to assess the embedded call or put options solely in accordance with the four-step decision process. In addition, ASU 2016-06 clarifies what steps are required when assessing whether the economic characteristics and risks of call or put options are clearly and closely related to the economic characteristics and risks of their debt hosts. ASU 2016-06 is effective for public entity financial statements issued for fiscal years beginning after December 15, 2016 and interim periods within those fiscal years, and after December 31, 2017 and interim periods beginning after December 31, 2018 for all other entities using the modified retrospective method for existing debt instruments. Early adoption is permitted. Our adoption of this standard did not have a material effect on our financial statements.

Accounting Standards Issued, Not Yet Adopted

In August 2016, the FASB issued ASU 2016-15,*Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments* ("ASC 2016-15"), which provides guidance on the classification of certain specific cash flow issues including debt prepayment or extinguishment costs, settlement of certain debt instruments, contingent consideration payments made after a business combination, proceeds from the settlement of certain insurance claims and distributions received from equity method investees. The standard requires the use of a retrospective approach to all periods presented, but may be applied prospectively if retrospective application would be impracticable. The guidance is effective for public entities for fiscal years beginning after December 15, 2017 and interim periods within those years, and after December 31, 2018 and interim periods beginning after December 31, 2019 for all other entities. Early adoption is permitted. The impact of its pending adoption of ASU 2016-15 is not expected to be material to our financial statements.

In February 2016, the FASB issued ASU No. 2016-02,*Leases* ("ASU 2016-02"), which applies to all leases. Under ASU 2016-02, a right-of-use asset and lease obligation will be recorded for all leases, whether operating or financing leases, while the statement of operations will reflect lease expense for operating leases and amortization and interest expense for financing leases. ASU 2016-02 is effective for public entities for fiscal years beginning after December 15, 2018 and interim periods within those years, and after December 31, 2019 and interim periods beginning after December 31, 2020 for all other entities. Early adoption is permitted. Entities are required to use a modified retrospective approach of adoption for leases that exist or are entered into after the beginning of the earliest comparative period in the financial statements. Full retrospective application is prohibited. The adoption of the new standard is not expected to have a material impact on our financial statements.

In June 2014, the FASB issued amended guidance, ASU No. 2014-09,*Revenue from Contracts with Customers* ("ASU 2014-09"), which is applicable to revenue recognition that will be effective for public entities for fiscal years beginning after December 31, 2017 and interim periods within those years, and after December 31, 2018 and interim periods beginning after December 31, 2019 for all other entities as a result of the deferral of the effective date adopted by the FASB in July 2015. A nonpublic entity may elect early adoption for fiscal years beginning after December 31, 2017 including interim periods within that reporting period. The new guidance must be adopted using either a full retrospective approach for all periods presented or a modified retrospective approach. For public entities, early adoption prior to the original adoption date (annual reporting periods beginning after December 15, 2016) of ASU 2014-09 is not permitted. The new guidance applies a more principles-based approach to revenue recognition. We will adopt the new standard, effective January 1, 2019, under the modified retrospective method. The impact of adopting the new standard is not expected to have a material impact on our financial statements.

BUSINESS

Overview

We are a company developing therapeutics for neurodegenerative disease inside and outside of the brain. We anticipate filing two INDs, for our lead programs with the FDA, in the first quarter of 2019.

Our lead programs utilize small molecule oral protein kinase inhibitors to treat PD, and its gastrointestinal complications. We have shown that our lead clinical candidate, IkT-148009, a potent, brain penetrant c-Abl protein kinase inhibitor, halts and/or reverses neurodegeneration in the brain and GI tract in preclinical models that mimic the human disease. We believe our therapeutic approach is disease-modifying.

In our opinion, the multi-decade failures in the treatment of neurodegenerative disease result from a lack of understanding of the biochemistry of the disease processes involved. Historically, symptoms of a neurodegenerative disease, like a “plaque” made up of a misfolded and/or aggregated protein(s), have been the development focus. To our knowledge, a “plaque”-focused strategy has not resulted in approval of a new medication that can alter the disease course for a neurodegenerative disease. We focus instead on the proteins that become dysfunctional in a disease pathway and seek to understand what leads to their dysfunction. In PD and other neurodegenerative diseases, our pathway-focused approach has enabled a deeper understanding of the origins of the disease and an understanding of how individual proteins are linked together to define the disease process. Using this strategy, we have discovered novel therapeutics for c-Abl, which we believe can alter the disease course for PD. Protein kinases are enzymes that modify proteins, including alpha-synuclein. Protein kinase inhibitors are small molecules that are believed to block the actions of protein kinases.

In addition to programs in neurodegeneration, our platform drug discovery and delivery technologies have identified additional opportunities, including potential treatment of bacterial or viral infections using a single agent at fixed dose and a potential near-term commercial opportunity in oncology that we believe will complete clinical development in 2019, subject to future FDA agreements, followed by completion of the FDA requirements for new drug approval in 2020. IkT-001Pro in oncology will seek to validate the pharmacology advantage of our prodrug delivery technology in a cancer patient population that is well understood. Following validation of IkT-001Pro in oncology, we believe the same pharmacology advantages could be applied to IkT-148009, our lead drug for neurodegenerative disease, to enhance clinical development. We believe we are one of the pioneers of the application of protein kinase inhibitors to non-oncology indications, including neurodegeneration and infectious diseases, as well as their more traditional role in the treatment of cancer.

Our scientific strategy is guided by our willingness to define the principles of the disease process and to do so by academic collaboration, bringing together multiple independent investigators to satisfy a common goal of understanding the origin of neurodegenerative disease. We believe that pathway identification and characterization will significantly increase the probability of success and will reduce the time to bring effective therapeutics to patients. Using this strategy, we believe the pathways governing the initiation and early stages of progression of Parkinson’s Disease were uncovered, enabling the focus on the c-Abl protein kinase as a therapeutic target. With c-Abl identified, we discovered novel protein kinase inhibitor therapeutics to block c-Abl and modify Parkinson’s and related alpha-synuclein disease and we then measured their efficacy in preclinical models.

In building and developing our portfolio, our product candidates were engineered to optimize target access and engagement. Using the RAMP drug discovery program, we believe we ‘pre-determine’ the human safety margin of our novel chemical entities. RAMP preserves the pharmacological properties of a template molecule with safety characteristics we desire, and then improves the potency of the template to create new chemical entities we intend to evaluate in clinical trials.

To increase the probability of success, we make parallel investments in several product candidates and back-up candidates, and plan to advance only those candidates to the later stages of clinical development that show strong preclinical and early clinical data. By developing a portfolio of product candidates for multiple, related indications, we can continuously apply learnings and tools across programs and leverage

economies of scale in our research and development organization. Our target indications include diseases with large patient populations, such as Parkinson's Disease, as well as orphan indications, such as Progressive, Multifocal Leukoencephalopathy, Multiple System Atrophy and Chronic Myelogenous Leukemia.

We currently have worldwide commercialization rights to all of our development programs and IP protection until 2032 or later.

Our Portfolio

IkT-148009: Our product candidate for Parkinson's Disease and related alpha-synuclein disorders

Market and Commercial Opportunity

Parkinson's disease (PD) is the second most prevalent neurodegenerative disorder, affecting approximately 1,000,000 persons in the United States, with 60,000 new cases and 38,000 deaths annually; worldwide, there could be as many as 10,000,000 cases of Parkinson's Disease. The compound annual growth rate for patients with PD is 4.5% and we expect that growth rate to continue through at least 2024. In the U.S. market, patients currently expend $15,000 to $25,000 per year to treat the symptoms of PD, creating a multi-billion dollar opportunity for disease-modification of this devastating disease. Moreover, since the same product would be used to treat both PD and its GI complications, we believe we have multiple opportunities to achieve commercial success in one or both treatment areas in this market.

c-Abl inhibition as a treatment focus in PD and related disease.

PD is a progressive disorder characterized by tremors, rigidity, difficulty in walking and an inability to maintain one's posture or keep oneself from falling.[(1)(2)] Pathologically, PD is characterized by degeneration of neurons in an area of the brain near the brainstem, coupled with the clumping and accumulation of mis-folded proteins in cell bodies known as Lewy bodies (LBs)[(3)(4)(5)]. The clinical and pathologic features of PD affect other areas of the brain in addition to the brainstem, resulting in a widespread pathology that is not adequately controlled with dopamine-replacement (i.e. levodopa) therapy.[(6)] Pathology of PD includes falling, freezing, neuropsychiatric disorders, GI complications, sensory problems, and cognitive impairment with dementia.[(6)]

Parkinson's Disease is initiated by a dysfunctional protein known as alpha-synuclein. In its dysfunctional form, alpha-synuclein is aggregated and likely to be misfolded, which collectively alter its physiological properties in the body. Dysfunctional alpha-synuclein, when taken up by a neuron, starts a cascade of events that are illustrated in Fig. 1. We believe that we can succeed in developing therapies that will slow or stop Parkinson's Disease and related disorders because we and our collaborators have characterized the pathways in Fig. 1 and believe the Abelson protein kinase, a.k.a. c-Abl, acts as a checkpoint on the pathway to neurodegeneration. The steps on the pathway illustrated in Fig. 1 have been validated in multiple contexts, multiple organ systems and by reproducing these results in two independent laboratories. Drawing from this knowledge, we believe inhibition of c-Abl will block the events downstream of c-Abl in these pathways and modify disease for PD and other alpha-synuclein related diseases.

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(6) A. H. V. Schapira, C. W. Olanow, J. T. Greenamyre, E. Bezard, Slowing of neurodegeneration in Parkinson's disease and Huntington's disease: future therapeutic perspectives. *Lancet* 384, 545-555 (2014).

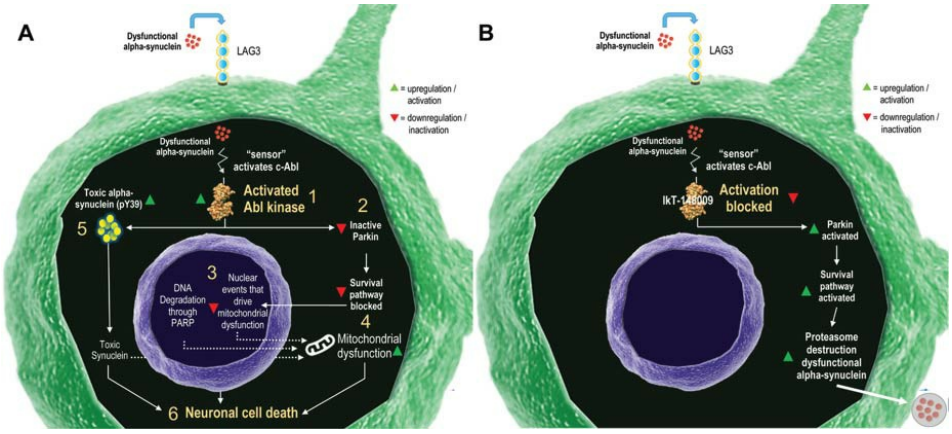

Fig. 1: A common pathway governs the process of neurodegeneration that initiates with dysfunctional alpha-synuclein. (A) Dysfunctional alpha-synuclein forms within a neuron as a consequence of chemical, environmental and/or genetic events. Once formed it can exit one neuron and into another through the Lag3 transporter.(7) Upon entering a neuron, dysfunctional alpha-synuclein is sensed by an unknown sensor that leads to activation of c-Abl. Once c-Abl is activated, c-Abl acts on dysfunctional alpha-synuclein to form what we believe is the true toxic entity of the disease created through phosphorylation by c-Abl on Tyr^{39} of alpha-synuclein (pY39).(8) pY39 goes on to influence the dysfunction of mitochondria and to drive cell death. C-Abl activation also phosphorylates a second protein, parkin. Parkin normally tags toxic proteins like dysfunctional alpha-synuclein so that they can be removed through the proteasome, which is the survival pathway that normally protects neurons from toxic proteins. But, when c-Abl acts on parkin, c-Abl inactivates it, shutting down the survival pathway and promoting nuclear and mitochondrial events that kill the neuron.**(B)** IkT-148009 acts systemically to block c-Abl activation, even when dysfunctional alpha-synuclein is present. Blocking c-Abl preserves the survival pathway leading to the removal of dysfunctional alpha-synuclein. In the presence of IkT-148009, toxic pY39 alpha-synuclein fails to form.

Development strategy for IkT-148009

Table 1

				Clinical Development				Biomarker		
Drug Target	Drug candidate	Modality	Disease indication	Preclinical Development	Phase 1	Phase 2	Phase 3	Preclinical target engagement	Clinical target engagement	Can be used for patient selection
Neurodegeneration										
c-Abl	IkT-148009	Small molecule	Parkinson's Disease: Treatment Naive		2019			Validated	Validating	Yes
c-Abl	IkT-148009	Small molecule	Parkinson's Disease: Early Stage		2019			Validated	Validating	Yes
c-Abl	IkT-148009	Small molecule	Neurogenic Constipation		2019			Validated	Validating	Yes
c-Abl	IkT-148009	Small molecule	Dysphagea		2019			Validated	Validating	Yes

IkT-148009 is a potent, selective and brain penetrant small molecule c-Abl inhibitor that we intend to use in clinical trials to treat two groups of PD patients and two additional groups to evaluate GI complications that arise early in the disease course for PD patients. We delineate the GI complications from PD because we will evaluate the GI complications using unique measurements and endpoints that are

(7) X. Mao, M. T. Ou, S. S. Karuppagounder, T. I. Kam, X. Yin, Y. Xiong, P. Ge, G. E. Umanah, S. Brahmachari, J. H. Shin, H. C. Kang, J. Zhang, J. Xu, R. Chen, H. Park, S. A. Andrabi, S. U. Kang, R. A. Gonçalves, Y. Liang, S. Zhang, C. Qi, S. Lam, J. A. Keiler, J. Tyson, D. Kim, N. Panicker, S. P. Yun, C. J. Workman, D. A. Vignali, V. L. Dawson, H. S. Ko, T. M. Dawson, Pathological α-synuclein transmission initiated by binding lymphocyte-activation gene 3. *Science* 353, (2016).

(8) S. Brahmachari, P. Ge, S. H. Lee, D. Kim, S. S. Karuppagounder, M. Kumar, X. Mao, J. H. Shin, Y. Lee, O. Pletnikova, J. C. Troncoso, V. L. Dawson, T. M. Dawson, H. S. Ko, Activation of tyrosine kinase c-Abl contributes to α-synuclein-induced neurodegeneration. *J Clin Invest.* 126, 2970-88 (2016).

distinct from PD itself. Thus, we believe we will have four opportunities to succeed with IkT-148009, lowering the risk of failure during the development program. We believe we have further lowered the risks associated with development of IkT-148009 because key aspects of the underlying pharmacology of IKT-148009 were "pre-determined" by preserving the ADME properties of the template molecule, Imatinib, from which IkT-148009 was chemically derived. IkT-148009 is a true new molecular entity and is subject to the regulatory guidance for new chemical entities from the FDA. The four indications to which IkT-148009 will be applied are listed in Table 1.

Efficacy of IkT-148009 in preclinical models of PD

1. Acute neurotoxicity model.

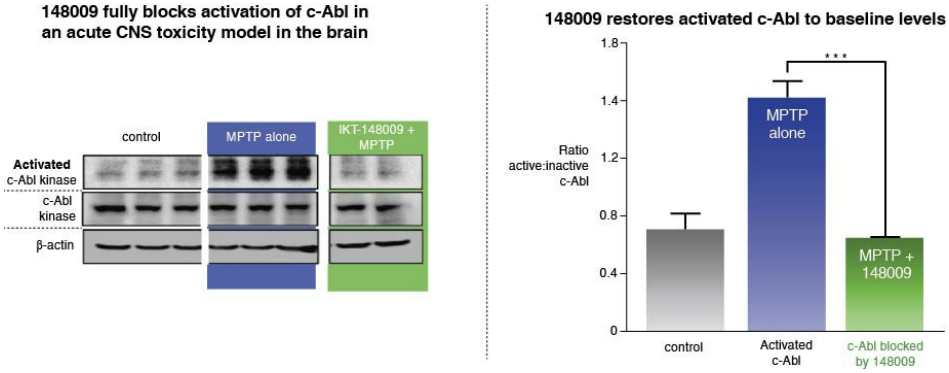

Fig. 2: IkT-148009 blocks activation of c-Abl by the acute neurotoxin MPTP. Orally delivered IkT-148009 at 50 mg/kg/day during a 14 day experiment blocks activation of c-Abl by MPTP in mouse brain. On the left is a Western Blot that enables quantification of the amount of inactive and activated c-Abl in mouse brain. On the right, the quantitation of the Western Blots for activated c-Abl demonstrates that IkT-148009 restores the level of activated c-Abl to baseline levels. The asterisks refer to the statistical analysis of the blots across three animals, with three asterisks representing a $P < 0.001$ in a Student's T-test.

This model uses a chemical neurotoxin, MPTP (1-methyl-4-phenyl-1,2,3,6- tetrahydropyridine), to stimulate c-Abl activation in the absence or presence of IkT-148009. Fig. 2 demonstrates that orally administered IkT-148009 at a human equivalent dose of 4 mg/kg/day completely blocks activation of c-Abl in the brain. Therefore, assuming a 60 kg adult, a human equivalent dose 240 mg is sufficient for IkT-148009 to prevent c-Abl activation induced by the acute neurotoxin MPTP in the mouse.

2. Progressive Disease Models. We have three newly described models and one model presently in development (Fig. 3) that recapitulate the formation of dysfunctional alpha-synuclein for both inherited PD in the brain or GI tract and for sporadic PD in the brain. Models 1 and 2 use an inherited mutation in alpha-synuclein, the Alanine-to-Threonine mutation at position 53 (A53T), that is found in Parkinson's patients. In Model 1, human A53T-alpha-synuclein is expressed in the mouse brain and in Model 2, human A53T-alpha-synuclein is expressed in the mouse GI tract and brain. For Models 3 and 4, we use laboratory prepared human alpha-synuclein that is treated in a manner to create dysfunctional alpha-synuclein which we refer to as a pre-formed fibrils or PFFs. The laboratory prepared PFFs mimic the aggregates of dysfunctional alpha-synuclein associated with sporadic PD in patients.

Evaluation of IkT-148009 is ongoing in Models 1, 2 and 3. We have recently completed readout from Model 2, the model that recapitulates GI tract complications of PD patients. We measure the GI tract complications in mice by measuring how fast food passes through the mouse GI tract. Normal mice process food and excrete solid waste in approximately 165 min. We call this measurement the Whole Gut Transit Time, or WGTT. Mice expressing A53T-alpha-synuclein in the neurons of the GI tract experience a progressive slowing of the WGTT as they age. The WGTT of mice at 3 months of age expressing A53T-alpha-synuclein averages 487 min (Fig. 4). By contrast, measurement of the WGTT in the presence of IkT-148009 results in restoration of nearly normal GI tract function. In fact, the drug treated mice (WGTT = 219 min) actually have GI function that is more similar to a normal mouse (WGTT = 165 min) and is superior to the GI function of mice expressing wildtype human alpha-synuclein (WGTT =

367.6 min). We refer to the drug treated mice as being “healthier than when they were born” because the WGTT is improved beyond that observed for the wildtype alpha-synuclein. This is remarkable in our view because the drug treated mice only express the dysfunctional form of alpha-synuclein in their bodies. We interpret the recovery of WGTT in mice as evidence of neuroprotection and/or neuro-regeneration in the mouse GI tract. This is a direct measure of likelihood of treatment success in patients in our view. We have also seen evidence of restoration of normal cognitive function in these mice using a proprietary approach that requires further validation prior to publication.

These results are consistent with previously published results by our collaborators where genetic deletion of c-Abl blocked all disease pathology associated with A53T-alpha-synuclein. Considering these model outcomes, we believe that inhibition of c-Abl in the GI tract will have a profound impact in patients treated with IkT-148009. We expect to complete analogous measures in mouse brain in the fourth quarter of 2018.

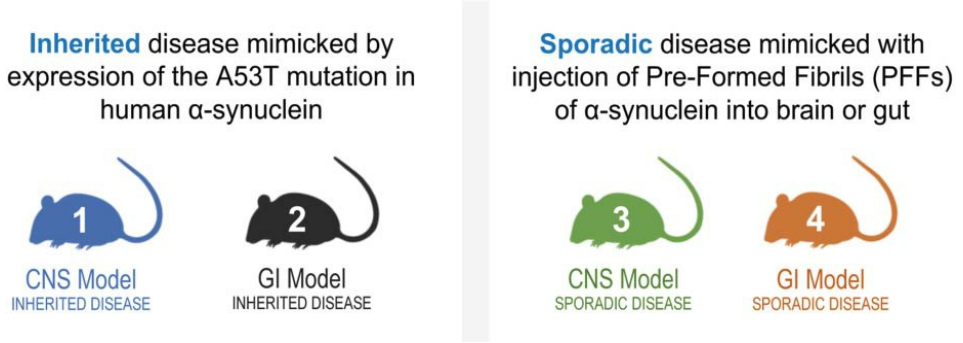

Fig. 3: Four progressive disease models used to evaluate drug efficacy against pathologic alpha-synuclein in mice. A53T alpha-synuclein is an inherited form of dysfunctional alpha-synuclein that leads to PD in some patients. Using mouse transgenics, A53T-alpha-synuclein can be introduced exclusively in the brain (Model 1) or in the brain and GI tract (Model 2). Models that mimic the most common form of PD use a laboratory prepared dysfunctional alpha-synuclein which we call a pre-formed fibril or PFF. PFFs can be injected into brain or the GI tract to induce PD or its GI complications in mice.

Pharmacology and toxicity of IkT-148009 in preclinical models

IkT-148009 is readily soluble in unbuffered water as a mesylate salt and readily absorbed from the GI tract following oral administration. In rodents, oral bioavailability is > 55% and at least 21% of the administered oral dose penetrates the blood brain barrier at peak absorption. IkT-148009 accumulates in the brain over 7 days, exceeding a total concentration in whole brain of 1 micromolar in rat. Given that the anticipated concentration required to saturate c-Abl in the brain is 188 nM, the accumulation of IkT-148009 in rodent brain over 7 days is more than sufficient to sustain a therapeutic concentration on daily dosing. We believe the pharmacology of IkT-148009 in rat is likely to be most similar to humans because rat pharmacology for Imatinib is closest to that found in humans.

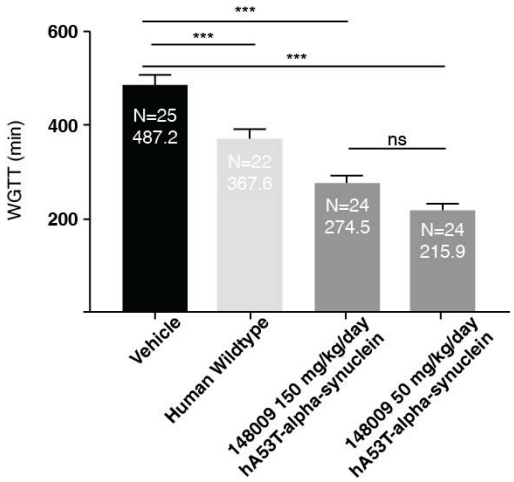

Fig. 4: The Whole Gut Transit Time (WGTT) measured in A53T and wildtype human alpha-synuclein transgenic mice in the presence or absence of IkT-148009. The WGTT was measured after 3 months at either 50 or 150 mg/kg/day in mice expressing A53T-alpha-synuclein. Two controls were used, either mice expressing A53T-alpha-synuclein administered a dosing solution without the drug (Vehicle) or a dosing solution without the drug administered to mice that expressed only human wildtype alpha-synuclein. These controls allowed comparison of drug treated mice to mice that express the normal human alpha-synuclein as the only source of alpha-synuclein in their bodies. Remarkably, both groups of drug treated mice display WGTT rates that are significantly faster than even the wildtype human synuclein mice. Note that because drugs like IkT-148009 have a half-life of just 2 hours in mice, a metabolic inhibitor was used (elacridar) to suppress premature loss of the drug during each 24 hour period of the experiment. It is not planned to use elacridar in large animal studies or in people. The inhibitor alone does not influence the WGTT in these mice. For each treatment group, the results were statistically significant relative to the no drug vehicle only treated controls and illustrated with three *** comparing the wildtype human synuclein or the drug treated mice to the vehicle control.

14-day toxicology studies in rats revealed no meaningful toxicity up to 200 mg/kg/day. The only notable observation was a very slight (at 50mg/kg/day) or slight (at 200 mg/kg/day) hyperplasia of the bile duct. When compared to Imatinib, the toxicity of IkT-148009 was comparable to or lower in nearly all other respects. Coupled with the lack of significant chronic or induced toxicity observed in the WGTT animals treated daily with IkT-148009 for 7 months, we anticipate human safety for IkT-148009 will be favorable. Similar evaluations are underway in monkey for IkT-148009.

Clinical Development Strategy for IkT-148009

Table 2: Trial designs for Proof-of-Concept Programs evaluating IkT-148009

	Treatment-Naïve	Early- to Mid-Stage*
# Patients	200	200
Centers	30 U.S. and/or ex-U.S.	≤ 10 U.S. and/or ex-U.S.
Enrollment	< 12 months	< 12 months
Measurement	• 12 months measurement using standard MDS-UPDRS outcome scores	• 12 months using standard MDS-UPDRS outcome scores + Novel Gait-related outcome measure
Regulatory status	FDA has approved this design for Phase 2/3 program	FDA engagement for this design under way
Budget	**$60K/patient = $12-15M max to POC**	**$60K/patient = $12-15M max to POC**

* Hoehn & Yahr 1-2.5

We intend to file two INDs for IkT-148009. One IND will be focused on clinical endpoints in the brain and a second IND focused on quantitative endpoints in the GI tract using proprietary tests. By using two distinct development approaches simultaneously, we believe we have a higher likelihood of developing a commercial product once the human safety of IkT-148009 is established in healthy volunteers. In the brain, two different trial approaches will be used simultaneously to achieve proof of concept for the majority of PD patients (Table 2). Treatment-naive patients can be accrued at a rate of 0.6 patients/center/month, requiring just 35 centers to enroll the Phase 2/3 trial in 10 – 12 months, followed by 12 months of measurement to reach the primary endpoint. The early stage patients on symptomatic therapy could be accrued from just 10 centers in 12 months in our estimation, followed by 12 months of measurement. We believe these trials could serve as registration trials for IkT-148009 to treat PD because the proposed primary endpoint, a 5 point reduction in the Universal Parkinson's Disease Rating Scale (UPDRS), is comparable to the reduction observed when treatment naïve patients initiate levodopa therapy. The difference will be that patients eventually lose response to levodopa. By contrast, we believe that patients treated with IkT-148009 will have their disease halted and/or reversed. Given the novelty of the approach and the underlying knowledge of mechanism of action, we anticipate achieving Breakthrough Designation during the clinical development program of IkT-148009.

The second IND for IkT-148009 would take a unique approach to seeking approval for the GI complications in PD patients. In the GI IND, prospective and retrospective data using a wireless capsule measuring WGTT along with high-resolution manometry would enable a direct measure of esophageal function (for dysphagia) and gut motility (for neurogenic constipation) in patients. The combination of these measures represent a new approach to evaluating neurological function in the GI tract. We believe these quantitative measures in the GI tract could facilitate proof-of-concept in 200 early stage PD patients, which could be accumulated from no more than 10 U.S. centers. We are initiating discussions with the Division of Neurology at FDA to seek their input to trial designs and primary endpoints as part of this proposed study.

IkT-001Pro: Validating our prodrug technology with what we believe is a near-term commercial opportunity in stable phase Chronic Myelogenous Leukemia (CML)

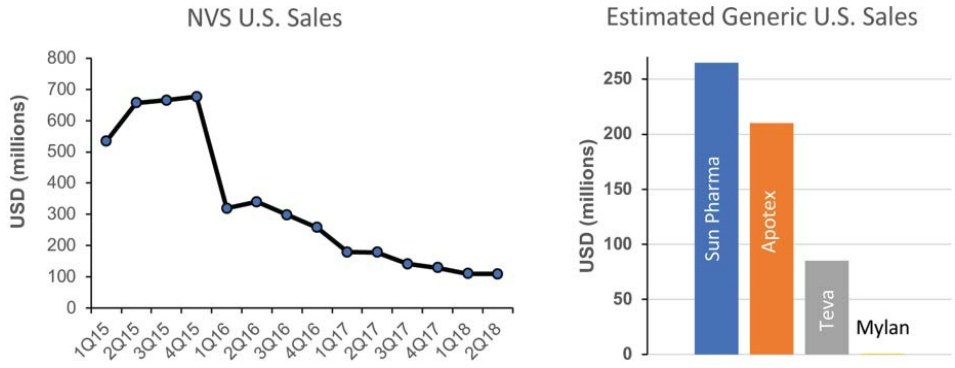

Fig. 5: Actual sales of branded Imatinib® and Estimated U.S. Sales of generic drug for 2016 – 2017. Sales of branded Imatinib for Novartis AG, or NVS, steadily declined over a 2 year period to approximately $400 million annually in 2018. At the same time, sales of generic drug grew for each of the four companies with approved ANDAs. To estimate generic sales for each company, the number of 400 mg units were identified by Symphony Health. To determine the actual price paid by patients, a survey of pharmacies prices for a 30 day supply was conducted through GoodRX®, setting the range of retail prices per pill between $34.20 and $115.10. Shown in the histogram on the right are the estimated maximum revenue by company over a two year period.

Market and Commercial Opportunity

IkT-001Pro is the first application of our prodrug technology that seeks to improve the oral absorption, reduce GI side effects and enhance the safety of active pharmaceutical ingredients. IkT-001Pro is an oncological prodrug of the anti-cancer agent Imatinib and approval will be sought from the FDA for IkT-001Pro in stable phase CML as an orphan indication. In 2016, Imatinib became generic and four

companies have been approved to sell generic Imatinib in the U.S. Current sales for branded Imatinib are approximately $440 million per year (Figure 5). Generic Imatinib sales add approximately $300 million more, indicative of a potentially robust commercial market for IkT-001Pro (Figure 5).

We believe IkT-001Pro will have superior safety and efficacy relative to branded and generic Imatinib. As a consequence, we believe we have an opportunity to capture a significant portion of the branded and generic Imatinib sales in the U.S. market, collectively we estimate to be worth more than $700 million annually. To achieve this commercial goal, we will require implementation of an appropriate commercial strategy for prescribers, pharmacy benefit managers and payors. Primary research to validate our strategy with pharmacy benefit managers and payors is now being conducted. We further believe that IkT-001Pro could capture market share from other first line therapies for CML. One of the approved indications for Nilotinib, for example, is for treatment of CML in patients that are Imatinib intolerant. For those patients whose Imatinib-intolerance arises from on-dosing side effects, we believe they would elect to take IkT-001Pro to relieve those side effects and avoid the serious cardiovascular risks associated with Nilotinib therapy.

Development Strategy for IkT-001Pro

CML is a proliferation of myeloid cells in the bone marrow with an incidence of 1 – 2 cases per 100,000 persons, and accounts for approximately 15% of newly diagnosed cases of leukemia in adults.[(9)] Prevalence of this disease has steadily grown over the past decade, with nearly 200,000 patients projected to be afflicted with this disease by 2050. Pathogenesis of CML is linked to a mutation in the c-Abl gene, referred to as BCR-Abl. BCR-Abl is a form of the c-Abl protein kinase that is always in the "on" state, and accounts for excessive accumulation of myeloid cells in the bone marrow and blood that we associate with leukemia. Inhibition of BCR-Abl with Imatinib suppresses tumor growth. In clinical practice, Imatinib is very successful at suppressing tumor burden with an 81% event-free survival rate and a 93% overall survival rate. However, 8-year follow-up studies revealed that only 55% of patients remained on therapy at 8 years, indicating that treatment failure grew over time. Treatment failures occur for a variety of reasons. We believe failure to adhere to the daily treatment regimen makes a significant contribution to treatment failure on Imatinib therapy. For example, nearly 50% of patients experience nausea, diarrhea and vomiting that are not well managed. Missing just 5 days of therapy in the first 12 months of treatment reduces the likelihood of reaching cure at the end of the fourth year of treatment by nearly 25%. Thus, while Imatinib remains the medication of choice for CML, GI distress and other on-dosing side effects of Imatinib therapy degrade patient adherence and lead to substantial additional medical costs, which can reach $100,000 per patient in the U.S. One of the key objectives for IkT-001Pro is to restore all patients to 100% treatment compliance by suppression of the GI and other on-dosing side effects for both branded and generic Imatinib.

Pharmacology of IkT-001Pro in preclinical models

We believe many of the side effects that degrade adherence to Imatinib therapy arise from GI distress on absorption, along with degradation that occurs at the gut wall (so-called first-pass metabolism). IkT-001Pro is a chemically modified form of Imatinib, that is absorbed intact and enzymatically releases Imatinib in the blood (Table 3). Evaluation of the prodrug absorption and distribution in rats demonstrated that the exposure to Imatinib is significantly higher overall (see the column labeled "AUC" in Table 3) even though the maximum concentration reached in the blood for Imatinib is the same for prodrug Imatinib and Imatinib alone (compare the C_{max} values). The higher distribution volume (Table 3) coupled with other studies led us to conclude that more Imatinib reaches the target tissue per unit mass of prodrug relative to the same mass of Imatinib administered alone. This has important implications to the safety of Imatinib in human patients.

(9) Jabbour E., Kantarjian H. (2014) Chronic myeloid leukemia: 2014 update on diagnosis, monitoring, and management. *Am. J. Hematol*. 89:548-556.

Table 3: Pharmacokinetic (PK) parameters in male rat at 3 mg/kg/day orally (n=3) and stability in human plasma for Imatinib prodrugs

Prodrug	T_{max} (hr)	C_{max} (nM)	AUC (nM-hr)	Elimination $T_{1/2}$ (h)	Distribution volume (L/kg)	Prodrug $t_{1/2}$ human plasma (min)[1]
Imatinib	2	323.3	1753	2.7	1.1	N/A
001Pro	4	387	2712	2.0	3	< 5

(1) The half-life of the prodrug is essentially the same in rat, monkey and human plasma.

Evaluation of the toxicity and tolerability of IkT-001Pro in monkeys revealed the unexpected property that the NOAEL, the dosing level at which no meaningful toxicity is observed, is 13-fold higher for IkT-001Pro relative to Imatinib given alone. We believe this is an unprecedented observation for a prodrug relative to the active ingredient from which it was derived. When we evaluated all the toxicities associated with the NOAEL of IkT-001Pro relative to the NOAEL of Imatinib, we also observed that all the GI and other on-dosing side effects were absent for IkT-001Pro.

Efficacy of IkT-001Pro in preclinical animal models of leukemia

Table 3 suggested to us that the exposure to Imatinib is higher per unit mass of prodrug than it is when Imatinib is administered alone. This suggested that we must be delivering more Imatinib into the target tissue when we deliver Imatinib via the prodrug. To evaluate whether this was true, we measured the efficacy of Imatinib therapy in a patient-derived model of leukemia by transferring the liquid tumor of a human patient into an immune-suppressed mouse. Fig 6. demonstrates that IkT-001Pro is as effective as Imatinib even though IkT-001Pro delivers 15% less Imatinib per unit mass.

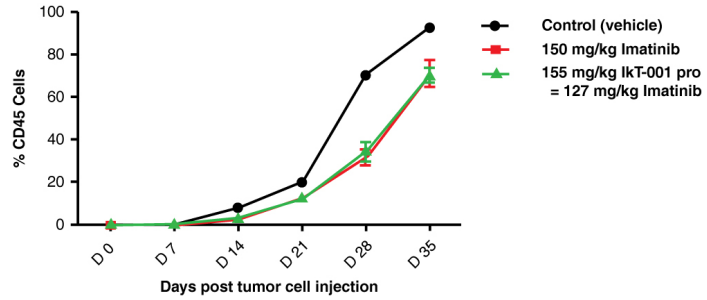

Fig. 6: Comparison of tumor control for Imatinib and IkT-001Pro. 150 mg/kg Imatinib or 155 mg/kg IkT-001Pro were dosed daily into 15 mice per group and compared to vehicle control. Dosing began on the 8th day after tumor cell inoculation into the tail vein with human, patient-derived leukemia cells, which could be followed with the cell surface marker CD45. As is readily apparent, tumor control by Imatinib and IkT-001Pro were identical even though IkT-001Pro delivered 15% less Imatinib relative to Imatinib alone. This is due to the mass difference between Imatinib and IkT-001Pro. These results confirm the observations made with respect to the AUC or drug exposure per unit mass of prodrug vs. Imatinib alone.

Clinical Development Strategy for IkT-001Pro in stable phase CML

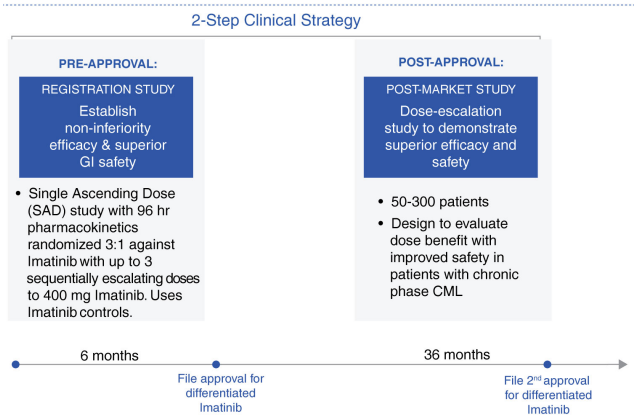

Fig. 7: Clinical development strategy of IkT-001Pro. The clinical development strategy follows the pre-IND discussion with the FDA for the development path of IkT-001Pro. First approval would occur for an orphan indication requiring just dose calibration to 400 mg generic Imatinib in healthy volunteers. The label at first approval would include the outcomes of the monkey comparative toxicology study to demonstrate the superior safety of IkT-001Pro. In a follow-up, post-market study, stable-phase CML patients will be treated with generic Imatinib and IkT-001Pro in a crossover study to show superior safety at lower Imatinib dose delivered.

Through pre-IND discussions with the FDA Division of Hematology, we believe approval of IkT-001Pro could be achieved through the 505(b)(2) regulation. The FDA has agreed to a highly truncated IND program involving only a single species comparative toxicology experiment in a non-human primate. The FDA has suggested that a single dose comparative clinical study to Imatinib with 96 hour pharmacokinetics measurement in healthy volunteers may be sufficient to calibrate the dose of IkT-001Pro to 400 mg Imatinib. For the IND, we are completing a single species comparative toxicology study requested by the FDA, comparing IkT-001Pro to Imatinib alone in monkeys. Following manufacturing of the clinical batch, we anticipate filing the IND for IkT-001Pro in the first quarter of 2019. To consider approval of IkT-001Pro, we believe the FDA is requiring a single dose comparison study to identify the dose of IkT-001Pro that leads to the same overall exposure as 400 mg branded Imatinib. We will attempt to demonstrate whether IkT-001Pro has superior safety relative to Imatinib in two-ways. In the monkey toxicology study, a dose of Imatinib alone will be compared to the mid-dose of IkT-001Pro to determine if Imatinib delivered by IkT-001Pro is safer than Imatinib alone using standard toxicology measures. In the clinic, the superior safety would be demonstrated in a post-market study of stable phase CML patients using a crossover design, wherein patients on 400 mg Imatinib would crossover to the equivalent dose of IkT-001Pro and the side effects experienced in these patients on daily dosing would be recorded for up to 12 months. The clinical development program is summarized in Fig. 7.

Our portfolio products for Progressive Multifocal Leukoencephalopathy, Multiple System Atrophy (MSA) and Dementia with Lewy Body (DLB)

We believe that the pathway described in Fig. 1 that governs the development of PD and its gastrointestinal complications is relevant to other indications that arise from dysfunctional alpha-synuclein. We are currently engaged in early stage research efforts to identify additional c-Abl inhibitors from the family of compounds from which IkT-148009 was derived that could be applied to other dysfunctional alpha-synuclein disease. We refer to those other c-Abl inhibitors as IkT-148x. DLB is a PD-like syndrome that involves the cognitive deficits common to PD, but without the loss of motor function PD patients experience. MSA is a more global disorder that results in multiple organ failure due to loss of both central and peripheral nervous system function. We are creating unique molecules for these indications that will incorporate our learnings from IkT-148009 in brain delivery and that we believe will have superior brain penetration and stability. We can evaluate these principles while preclinical models for these indications become more representative of the human disease. We intend to evaluate compounds in the IkT-148x family to identify potential therapeutics for each indication using these emerging models.

We have pursued a long-term research program in host-targeted anti-infectives, which have the potential to treat bacterial and viral infectious disease with a single agent at fixed dose. This type of anti-infective could be transformative, because it depends only on blocking a single target in the patient to interfere with bacterial or viral reproduction simultaneously. We are applying this strategy to the brain infection of the JC virus, the cause of Progressive Multifocal Leukoencephalopathy (PML).

Expertise and overall strategy

We have assembled a team of principals and advisors with deep scientific, clinical, business and leadership experience and expertise in drug development that includes neurodegenerative diseases. Our Founder and Chief Executive Officer, Milton H. Werner, Ph.D., is an internationally recognized scientist with a long history of conducting interdisciplinary research and executing on research programs in multiple therapeutic areas. Dr. Werner is a protein biochemist and structural biologist by training, enabling him and the team of principals and advisors we have assembled to develop and characterize a portfolio of novel c-Abl inhibitors and to rapidly determine their utility in a variety of model systems for specific diseases. Dr. Werner is joined by Terence Kelly, Ph.D., a 20-year veteran of medicinal chemistry at Boehringer-Ingelheim in Connecticut and Roger Rush, Ph.D., who has led IND-enabling programs for ground-breaking medications, like the Hepatitis C compound portfolio of Idenix, which was sold to Merck & Co. Inder Kaul, M.D., M.P.H. has extensive clinical development experience across multiple therapeutic areas, including neuroscience and is expert at clinical trial design and execution, a key component that prevents the uncoupling of the medical director from the actual execution of clinical development.

Collaborations are central components of our strategy to build and advance our pipeline of product candidates. Through NIH research grants awarded to Dr. Werner, we have subcontracted research projects in the biochemistry of neurodegeneration and neurological infection to Johns Hopkins University, University of Massachusetts Medical School — Worcester Campus, University of Alabama at Birmingham and the Louisiana State University Shreveport. In oncology, we have subcontracted research work to the University of California, San Francisco and consult with clinicians at the Memorial Sloan-Kettering Cancer Center. Our research endeavors have been validated by private and public granting agencies, to include the Michael J. Fox Foundation, and the National Institute of Neurological Disease and Stroke and the National Institute of Allergy and Infectious Disease. We believe that accessing external innovation is important to our success and we plan to remain active in accessing external innovation through business development activities and awarding of private, state and federal grants through institutions such as NIH and DoD.

Our leadership team is complemented by leading clinicians and research investigators in the areas of neurovirology (Drs. Joseph Berger and David Clifford) and neurodegeneration (Drs. Ted Dawson, Valina Dawson, Karl Kieburtz and C. Warren Olanow). We have research collaborations with Dr. Carrolee Barlow of the Parkinson's Institute, Sunnyvale, California and former head of CNS Clinical Development for Merck & Co., and with Dr. Robert Rissman of University of California, San Diego, an Alzheimer's and Parkinson's Disease expert, among others. Collectively, this group of collaborators and advisors represent what we believe is the cutting edge of the fields of neuroscience and neurodegeneration.

Our strategy is guided by three principles:

- **Identification and characterization of the pathway(s) governing neurodegenerative disease:** We select our therapeutic targets by identification and characterization of disease pathways that we believe drive neurodegenerative disease and elucidate the biochemistry of pathway proteins to enable small molecule targeting to treat PD and related disorders, often involving clinically validated targets.
- **Proprietary method of drug discovery in neurodegeneration:** We use our RAMP method to imprint the properties we desire from an approved medication onto a new molecular entity for treatment inside and outside of the brain. Using RAMP, we believe we can "pre-determine" the pharmacology and side effect profile of our product candidates using an existing medication as a template.
- **Delivering neurodegenerative treatments as a prodrug to improve pharmacology and safety:** A prodrug is a compound that, after administration, is metabolized by the body into a

pharmacologically active drug. Our prodrug technology has been shown in animal models to suppress GI and other adverse events commonly associated with oral kinase inhibitors and improve drug absorption from the GI tract. We believe this technology both reduces side effects and enhances drug distribution into the target tissues, which we believe will improve safety and tolerability of our kinase inhibitors for neurodegenerative and other diseases.

We believe that the application of these principles will significantly increase the probability of our success and will shorten the time required to bring effective therapeutics to patients with neurodegenerative and other diseases.

Drug discovery and Delivery Technologies

Engineering Small Molecule Brain Delivery

Our RAMP drug discovery program used Imatinib as a template to design and discover a family of novel chemical entities with high potency against c-Abl. We showed in preclinical models that a subset of the molecules that grew out of RAMP were more brain penetrant than Imatinib. We believe the specific modifications in the more brain penetrant RAMP molecules sterically hinder engagement of transporters that could suppress accumulation of drug in the brain. Thus, we believe RAMP could be further applied to predicting and developing next generation molecules with enhanced brain penetration without compromise of c-Abl inhibition. As part of our ongoing research and development effort, we have increased the ability to penetrate the blood-brain barrier by as much as 8-fold in preclinical models, enabling direct treatment in the brain following oral administration.

Enhance drug absorption through a prodrug technology

In addition to the design principles we have deployed to develop c-Abl inhibitors capable of maintaining therapeutic concentrations in the brain, we have also developed a delivery technology that suppresses GI side effects that occur on dosing with medications in this class. Using the anti-cancer agent Imatinib as a prototype, we believe that we have shown that formation of an ester-linked prodrug enables absorption of the active ingredient without induction of GI side effects, resulting in an increase in the NOAEL by 13-fold relative to Imatinib alone in preclinical models. The active ingredient we believe is more efficiently absorbed into the blood using this approach, which results in achieving therapeutic exposures with less drug administered. Since GI side effects can be common for drugs in this class and often discourage adherence to therapy, we believe this approach could be applied to any of the drugs we currently are developing for treatment of CNS disease and could be used to improve existing therapeutics in cancer as differentiated generics.

History of Business Operations and Key Events

We commenced operations in September 2008 as a Georgia limited liability company with in-licensed intellectual property relating protein kinase inhibitors to the control of bacterial and viral infectious diseases. By 2015, we had developed a portfolio of protein kinase inhibitors to treat bacterial and viral infections, including viral infections in the brain. During 2015, we also began our endeavors in developing product candidates for other diseases of the brain, including neurodegeneration. Key operational and financing milestones include:

- Between August 2008 and October 2008, the State of Georgia, through the Georgia Research Alliance, began financial support for the development of our underlying technologies for drug discovery and development of disruptive medications across multiple therapeutic indications, granting $205,550.
- In September 2009, the National Institute of Neurological Disease and Stroke, an Institute of the National Institutes of Health, awarded us $265,846 to begin development of small molecule treatments for viral infections in the brain.
- In September 2011, we executed a promissory note for the second tranche of a total of $250,000 of an economic development loan from the State of Georgia through the Georgia Research Alliance.

- In August 2012, we entered into a contract for $2,731,823 with the Department of Defense to develop our disruptive approach to treating infectious disease across viral and bacterial infections as a Medical Counter Measure (MCM).
- In September 2013, we amended our contract with the Department of Defense to increase the total value of the contract to $7,129,614 to expand our development of MCMs.
- In June 2015, the National Institute of Allergy and Infectious Disease, an Institute of the National Institutes of Health, awarded an additional $1,540,897 to continue our development of small molecule therapeutics to treat JC virus infection in the brain.
- In March 2017, the National Institute of Allergy and Infectious Disease, an Institute of the National Institutes of Health, awarded an additional $2,000,000 to continue our development of small molecule therapeutics to treat JC virus infection in the brain.
- In March 2017, the Michael J. Fox Foundation awarded us $433,729 to screen our novel c-Abl protein kinase inhibitors in a mouse model of Parkinson's Disease.
- In June 2017, we believe we came to an understanding with the FDA on the requirements for approval for IkT-001Pro, a prodrug of Imatinib, for the treatment of stable-phase patients with CML using a product with a potential for significant reduction of side effects under the FDA 505(b)(2) regulations.
- In September 2017, the National Institute of Neurological Disease and Stroke, an Institute of the National Institutes of Health, awarded us $3,108,583 to advance our novel c-Abl inhibitors as disease modifying therapies for Parkinson's Disease and related disorders.
- In May and September 2017, certain members of our board of directors invested an aggregate of $150,000 in convertible debt instruments to further advance the Parkinson's Disease programs.
- In March 2018, we opened our pre-IND discussion with the FDA for the application of our novel c-Abl inhibitor IKT-148009 for the treatment of Parkinson's Disease.
- In May 2018, outstanding convertible debt in the aggregate amount of $339,729 was converted into 81,081 shares of our common stock at $4.19/share.
- In May 2018, warrants were exercised for the purchase of 77,108 shares of our common stock, resulting in aggregate proceeds of $60,144. In a simultaneous transaction, 33,378 shares of our common stock by an investor were purchased at $4.19/share.
- In May 2018, 71,599 shares of our common stock were purchased by an investor at $4.19/share.
- In July 2018, 9,545 shares of our common stock were purchased by an investor at $4.19/share.

We do not have any products approved for sale and have not generated any product revenue since our inception. Historically, we funded our operations primarily with revenue from State of Georgia and Federal Contracts and Grants from NIH and DoD and loans from the State of Georgia through the Georgia Research Alliance. Since 2017, we augmented grant and contract revenue with equity sales of common stock to members of our board of directors and others. From inception through March 31, 2018, we have raised aggregate cash proceeds of approximately $15,466,000 from private, state and federal contracts and grants.

We have incurred significant operating losses to date and expect to continue to incur operating losses for the foreseeable future. Our ability to generate product revenue will depend on the successful development and eventual commercialization of IkT-001Pro, followed by successful development of IkT-148009 and related molecules for one or more of our product candidates in Parkinson's Disease and related indications. Our net losses were $436,075 and $628,737 for the years ended December 31, 2017 and 2016, respectively, and $124,984 for the three months ended March 31, 2018. As of March 31, 2018, we had an accumulated deficit of $4,433,278. We expect to continue to incur significant expenses and operating losses as we advance our c-Abl inhibitor programs through preclinical and clinical trials; broaden and improve our drug discovery and delivery technology platforms; acquire, discover, validate and develop

additional product candidates; obtain, maintain, protect and enforce our intellectual property portfolio; and hire additional personnel. In addition, upon the completion of this offering we expect to incur significant additional costs associated with operating as a public company.

Regulatory and Clinical Experiences

From September 2014 through September 2016, we conducted two non-interventional clinical studies to inform our research on the risk, development, and treatment of PML. The results of one of the studies was published in the Journal of Neurovirology.[(10)] In 2016, FDA approved protocols allowing us to conduct clinical trials with the use of non-Inhibikase marketed products to treat PD. We did not conduct these studies based on our decision to pursue development of IkT-148009.

Material Agreements

Emory University

In June 2010, we entered into a license agreement, or the Emory License, with Emory University, or Emory to develop one or more products. These products are related to patents filed by Emory directed to methods using the active ingredient in the anti-cancer agent Imatinib, as an anti-infective. We believe this ingredient is capable of treating bacterial and viral infections with a single agent at fixed dose through c-Abl protein kinase inhibition. In addition to a patent portfolio related to the use of Imatinib, additional patents were licensed to us that related to a portfolio of compounds, many of which were novel. These compounds were designed to inhibit c-Abl in patients for a therapeutic purpose. These patents formed the starting point for our RAMP drug discovery program, although none of the compounds described in the licensed patents are structurally similar to any of the molecules designed and developed through RAMP.

The Emory License grants us an exclusive, worldwide, sublicenseable license under patent rights related to the application of Imatinib or a series of novel analogs for the treatment of infections caused by both viruses and bacteria that utilize c-Abl protein kinase to reproduce in human hosts. The Emory License also includes a right of first offer for us to license from Emory certain improvement technologies related to the licensed subject matter. Unless sooner terminated as provided in the agreement, the term of the Emory License is the later of ten years or until the expiration of the patent rights.

We have certain obligations under the Emory License, which include using commercially reasonable efforts to develop and commercialize at least one licensed product under the patents and achieving certain milestones such as filing an IND, proof-of-concept clinical trial, Phase III trial and NDA filing for a licensed product. We are also obligated to reimburse Emory for pre-existing and ongoing costs incurred by Emory related to the filing, prosecution and maintenance of the licensed patents. These patents are controlled by Emory, although we have the right to review copies of all filings and correspondence related to such prosecution and maintenance. As of the date of this prospectus, we owe Emory approximately $356,000 for such incurred costs.

As partial consideration for the Emory License, we issued 450,000 shares of our common stock to Emory. In addition, we are obligated to pay to Emory a royalty of a low single-digit percentage of annual net sales by us, our affiliates and our sublicensees of licensed products and licensed services that are covered by a valid claim of the licensed patent rights at the time and in the country of sale, subject to a royalty stacking provision under which related intellectual property licensed from Duke University, or Duke, could reduce such royalties by up to 50%. Minimum annual royalties in the first three years after the first sale of a licensed product are $10,000, $20,000, and $40,000, respectively, and remain at $40,000 thereafter, for as long as the licensed patent rights are protected by valid claims in any such particular country of sale. On a country-by-country basis, upon expiration of the last valid claim of the licensed patent rights covering such licensed product or licensed service in such country, our license becomes royalty-free with respect to such country.

(10) Werner, M.H. and Huang, D. (2016) Natalizumab-treated patients at high risk for PML persistently excrete JC polyomavirus. *J. Neurovirol*. 22:871.

If one of the compounds subject to the Emory License proceeds to clinical development to treat infectious disease, we are obligated to pay potential total milestone payments of $280,000 based upon achievement of certain preclinical, clinical and regulatory milestones. In addition, we are required to pay to Emory a percentage of the payments that we receive from sublicensees of the patent rights licensed to us by Emory ranging from low single-digit to low double-digit percentages of the payments received by us under such sublicense and will be based upon the clinical stage of the product at the time of the sublicense.

In the event of third party infringement of the licensed patents, we have the right but not the obligation to file suit, at our cost, against the third party infringer. Upon settlement or judgement, any punitive or exemplary damages will be shared, after payment of costs, 70% to us, and 30% to Emory; compensatory damages, after payment of costs, will be treated as sales of licensed product, where we would pay Emory at the standard royalty rate. In the event we choose not to bring suit, Emory may do so, at their cost, and any damages would be shared 95% to Emory, 5% to us.

We entered into an additional license agreement with Emory on June 8, 2010, for intellectual property related to noscapine and noscapine derivatives for the treatment of infections caused by both viruses and bacteria that are sensitive to noscapine treatment. As partial consideration for the license, we issued 500,000 shares of our common stock to Emory. This license was terminated May 29, 2013 due to our inability to demonstrate any commercially viable applications of noscapine to treat any viral or bacterial infection. No financial obligations following the termination of this license remain.

Duke University

On June 18, 2010, we entered into a license agreement, or the Duke License, with Duke University to develop one or more products related to a patent filed and granted to Duke claiming methods of preventing or treating bacterial or viral infections through c-Abl protein kinase inhibition including Imatinib.

The Duke License grants us an exclusive, worldwide, sublicenseable patent license related to the application of an inhibitor of Abl tyrosine kinase to the treatment of bacterial infections, and a non-exclusive license to certain research and technical information not included in the patent rights.

Our obligations under the Duke License include using commercially reasonable efforts to develop and commercialize at least one licensed product under the patent rights and achieving certain milestones related to financing, filing of an IND, clinical trials and an NDA filing. We are also obligated to reimburse Duke for all reasonably and actually incurred costs by Duke related to the filing, prosecution and maintenance of the licensed patent. The patent is controlled by Duke, though we have the right to review and comment upon copies of all filings and correspondence related to such prosecution and maintenance. As of the date of this prospectus, we have reimbursed Duke for all such incurred costs.

As partial consideration for the license, we issued 700,000 shares of our common stock to Duke. In addition, we are obligated to pay to Duke an annual license fee of $5,000, a royalty of a low single-digit percentage of annual net sales by us or our sublicensees of licensed products and licensed services that are covered by a valid claim of the licensed patent rights at the time and in the country of sale, subject to certain accounting adjustments and a royalty stacking provision under which the Emory License could reduce such royalties payable under the Duke License by up to 50%. Minimum annual royalties in the first three years after the first sale of a licensed product range from $5,000 to $20,000, and remain at $20,000 thereafter. On a country-by-country basis, upon expiration of the last valid claim of the licensed patent rights covering such licensed product or licensed service in such country, our license becomes royalty-free with respect to such country.

We are obligated to pay potential total milestone payments of $280,000 based upon achievement of certain preclinical, clinical and regulatory milestones. In addition, we are required to pay to Duke a percentage of the payments that we receive from sublicensees of the patent rights licensed to us by Duke. This percentage varies from low single-digits to mid-single digits of the payments received by us under such sublicense and will be based upon the clinical stage of the product at the time of the sublicense.

In the event of third party infringement of the licensed patents, we have the right but not the obligation to file suit, at our cost, against the third party infringer. Upon settlement or judgement, any punitive or exemplary damages will be shared, after payment of costs, 75% to us, and 25% to Duke; compensatory

damages, after payment of costs, we will pay Duke an amount equal to a reasonable approximation of the royalties we would have owed Duke under the license, not to exceed 50% of the balance. In the event we choose not to bring suit, Duke may do so, at their cost, and Duke would pay us an amount equal to our lost profits or reasonable royalty (depending on the standard used by the court) less a reasonable approximation of royalties owed to Duke under the Duke License; punitive damages would be shared 75% to Duke and 25% to us.

Sphaera Pharma Pte. Ltd.

On March 2, 2012, we entered into a collaborative research and development agreement, or the Sphaera Agreement with Sphaera Pharma Pte. Ltd., or Sphaera, to collaborate on the development of the prodrug technology to be applied to protein kinase inhibitors for oncology and non-oncology indications. Under the terms of the Sphaera Agreement, each party would retain its pre-existing intellectual property, but any intellectual property conceived or reduced to practice under and certain results arising from the Sphaera Agreement would be assigned to us. On October 5, 2012, we and Sphaera amended the Sphaera Agreement to reflect joint patent applications in the U.S. and India by us and Sphaera for a series of novel compounds. While the underlying intellectual property would be jointly owned, we have the exclusive right to commercialize thirteen of the twenty-four linkers detailed in the filed patent applications, collectively, the Company Compounds, including the linker attached to Imatinib that comprises the 001Pro oncology product, with the remaining nine linkers owned by Sphaera, collectively, the Sphaera Compounds. Sphaera has the right to develop the Company Compounds for oncology indications, but may not commercialize the Company Compounds unless we abandon the Company Compounds. We have notified Sphaera that we do not intend to abandon the Company Compounds. We do not currently have the right to develop the Sphaera Compounds. Additionally, if either party files an IND for a Company Compound for an oncology indication in humans, the non-filing party is prohibited from developing such Company Compound.

The prosecution of patents related to the Company Compounds, which includes the prodrug technology, are the responsibility of the Company. As consideration for its services, Sphaera has received a fixed fee of $160,000 and is entitled to milestone payments upon the achievement of specified preclinical, clinical and regulatory milestones ranging from $250,000 upon the first dosing of a patient in a Phase 1 trial to $4,000,000 for FDA approval. Sphaera is also entitled to royalty payments of a percentage of annual net sales and sublicenses ranging in the mid-single digits. The parties did not contemplate the development of IkT-001Pro as a competitor to the generic Imatinib now on the market. As such, we and Sphaera are re-negotiating our financial obligations to ensure furtherance of the product to market.

Sponsored Research Agreements

We regularly enter into agreements with academic and research institutions under which the institution agrees to perform certain testing and research for us in exchange for incremental fee payments, or the Sponsored Research Agreements. Currently, we have Sponsored Research Agreements with Johns Hopkins University, University of Massachusetts Medical School — Worcester Campus, Louisiana State University, Shreveport, and the Parkinson's Institute, collectively, the Institutions. Incremental fee payments are due to the Institutions on a monthly or quarterly basis, and certain payments depend on the completion by the Institutions of testing and research milestones. The Sponsored Research Agreements contain estimated completion dates which may be extended by written agreement of the parties. The Sponsored Research Agreements may be terminated by either party on 30 days written notice, and upon termination we must reimburse the Institutions for all costs and reasonably incurred financial commitments, regardless of which party initiates the termination. Under the Sponsored Research Agreements, we retain all rights, title and interest in any information designated as purchaser property, as defined in the Sponsored Research Agreements. The Institutions retain all rights, title and interest to any inventions, discoveries, material and improvements conceived and/or developed by the Institutions or their personnel, but grant us an option to negotiate licenses or sub-licenses for the Institutions' rights, title and interest.

Manufacturing

We believe it is important to our business and success to have a reliable, high-quality preclinical and clinical drug supply. As we mature as a company and approach clinical and then commercial stage operations, securing reliable high-quality commercial drug supply will be critical.

We do not currently own or operate facilities for product manufacturing, storage, distribution or testing.

We rely on third party contract manufacturers, or CMOs, to manufacture and supply our preclinical and clinical materials to be used during the development of our product candidates. We have established relationships with several CMOs, including Agno Pharmaceuticals, LLC and PepTech Corporation, both in China, and we are in the process of contracting GMP manufacturing in the United States.

We do not currently need commercial manufacturing capacity. When and if this becomes relevant, we intend to evaluate both third party manufacturers as well as building out internal capabilities and capacity. We may choose one or both options, or a combination of the two.

Commercialization Plan

We do not currently have any approved drugs and we do not expect to have any approved drugs in the near term. Therefore, we have no sales, marketing or commercial product distribution capabilities and have no experience as a company in marketing drugs. However, members of our board of directors have commercial experience and we have conducted a full commercial opportunity assessment for our lead product for PD in the U.S. market.

Competition

The pharmaceutical industries, including in the neurodegenerative disease field, are characterized by rapidly advancing technologies, strong competition and an emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical companies, academic research institutions, governmental agencies and public and private research institutions. We believe that the key competitive factors affecting the success of any of our product candidates will include efficacy, safety profile, method of administration, cost, level of promotional activity and intellectual property protection.

Our product candidates for treatment of neurodegenerative diseases (PD, DLB, MSA and GI complications) will compete with other therapies in clinical development. However, approved treatments for PD and related disorders treat the symptoms of such diseases rather than halting or slowing the progression of the disease. Halting or slowing the progression of the disease is known as disease modification and our products are intended to modify disease. We believe that our product candidates, if approved by regulatory agencies in the U.S. and abroad, will compete with other potential therapies intended to halt or slow the progression of neurodegenerative disease that are being developed by a number of companies and institutions. Several large and specialty pharmaceutical companies, including Prothena Corporation plc, Roche Holdings AG, Biogen Inc., Neurimmune Holding AG, UCB S.A., Neuropore Therapies, Inc., Sanofi S.A., and Takeda Pharmaceutical Company Ltd. are developing potentially disease modifying therapeutics for PD and are in various stages of clinical trials. In addition, a number of companies have developed c-Abl inhibitors for oncology and any one of them could be in possession of an inhibitor that could be used for clinical development for neurodegenerative diseases. These include Novartis AG, Bristol-Meyers Squibb Company, Boehringer-Ingelheim GmbH and GlaxoSmithKline plc. In addition, we believe Botox® coupled with physical therapy is being explored in physician-led trials for neurogenic constipation, but we are not aware of any formal development programs by other companies.

Intellectual Property

The proprietary nature of, and protection for, our product candidates, processes, and know-how are important to our business. Our success depends in part on our ability to protect the proprietary nature of our product candidates, processes and know-how, to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary rights. We seek and maintain patent protection in the United States and internationally for our product candidates and other technology. We endeavor to patent or in-license technology, inventions and improvements that we consider important to the development of our business. In addition to patent protection, we intend to use other means to protect our proprietary rights, including pursuing terms of marketing or data exclusivity, orphan drug status (if applicable), and similar rights that are available under regulatory provisions in certain territories, including the United States, Europe and Japan. We also rely on trade secrets, know-how and continuing innovation to develop and maintain our competitive position.

For our product candidates, we generally pursue patent protection covering compositions of matter and methods of use. However, given that the development of our technology and product candidates is at an early stage, our intellectual property portfolio with respect to certain aspects of our technology and product candidates is also at an early stage. As further described below, we have filed or intend to file patent applications on various product candidates for composition of matter and other aspects of our technology and product candidates, and as we continue the development of our product candidates, we intend to identify additional means of obtaining patent protection that would potentially enhance commercial success, including protection for additional methods of use, formulation or manufacture.

We cannot be certain that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents granted to us in the future will be commercially useful in protecting our technology. Any of our intellectual property and proprietary rights could be challenged, invalidated, circumvented, infringed or misappropriated, or such intellectual property and proprietary rights may not be sufficient to permit us to take advantage of current market trends or otherwise to provide competitive advantages. For more information, please see "Risk Factors — Risks Relating to Intellectual Property."

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. The patent expirations reported below assume the patent is not rendered invalid or unenforceable by legal action and that all required fees are timely paid. In the United States, a patent may be entitled to Patent Term Adjustment for Patent Office delay. Where known, this has been included in the expiration dates described below. Further, in the United States, the patent term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to an approved drug may be extended and the extension can only be obtained for patents covering the approved drug, a method for using it, or a method for manufacturing it. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our eligible products receive FDA approval, we expect to apply for patent term extensions on patents covering those products. We plan to seek patent term extensions to any of our issued patents in any jurisdiction where these are available; however, there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extensions should be granted, and if granted, the length of such extensions. For more information regarding the risks related to our intellectual property, see "Risk Factors — Risks Related to Our Intellectual Property."

As of July 31, 2018, our patent portfolio included: (i) three issued patents and one pending patent application in the United States and (ii) one issued foreign patent and eight pending Patent Cooperation Treaty, or PCT, and foreign patent applications. Patents issuing from the applications in this portfolio, if granted, will expire between 2032 and 2037, not taking into account any potential patent-term adjustments or extensions that may be available in the future.

One family of patents and applications covers compositions of matter for IkT-001Pro and related chemical compounds, as well as methods of using those compounds. Patents issuing from the applications in this family, if granted, will expire between 2033 and 2034, not taking into account any potential patent-term adjustments or extensions that may be available in the future. This family includes two issued U.S. patents: U.S. Patent No. 9,487,500, which claims a genus of compounds including IkT-001Pro, and U.S. Patent No. 9,907,796, which claims methods of using this genus of compounds to treat tumoral disease and certain infectious diseases. Outside the U.S., this family includes one issued patent in Australia, and pending patent applications in Japan, Canada, and Europe. This family of patents and applications is jointly owned by us and Sphaera. Under the terms of our agreement with Sphaera, described above under "— Material Agreements — Sphaera Pharma Pte. Ltd." we have the exclusive right to commercialize certain compounds disclosed in these applications, including IkT-001Pro, for cancer treatments.

Two families of patents and applications cover compositions of matter for IkT-148009 and IkT-01427, the IkT-148x portfolio, and methods of use relating to those compositions. Patents issuing from the applications in these families, if granted, will expire between 2036 and 2037, not taking into account any potential patent-term adjustments or extensions that may be available in the future. These families include one issued U.S. patent and pending patent applications in the United States, Japan, Australia, Canada, and Europe, as well as one pending PCT patent application. The issued patent, U.S. Patent No. 9,828,370, will expire in 2036, not including any potential patent-term extensions, and includes claims that cover compositions of matter for IkT-148009 and IkT-01427. These families are solely owned by us.

We hold a license from Emory University to (i) two issued patents in the United States and (ii) eight issued foreign patents. These patents cover methods of treating pathogenic infections with certain tyrosine kinase inhibitors, not including IkT-148009 and IkT-01427, and will expire between 2025 and 2028.

We hold a license from Duke University to U.S. Patent No. 7,384,907. That patent covers methods of preventing or treating bacterial or viral infections with Abl tyrosine kinase inhibitors, and will expire in 2024, not taking into account any potential patent-term extensions that may be available in the future.

In addition to patent protection, we also rely on trade secrets, know how, other proprietary information and continuing technological innovation to develop and maintain our competitive position. We seek to protect and maintain the confidentiality of proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. Our agreements with employees also provide that all inventions conceived by the employee in the course of employment with us or from the employee's use of our confidential information are our exclusive property. However, such confidentiality agreements and invention assignment agreements can be breached and we may not have adequate remedies for any such breach. For more information regarding the risks related to our intellectual property, see "Risk Factors — Risks Related to Our Intellectual Property."

The patent positions of pharmaceutical companies like ours are generally uncertain and involve complex legal, scientific and factual questions. Our commercial success will also depend in part on not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third party patent would require us to alter our development or commercial strategies, or our drugs or processes, obtain licenses or cease certain activities. Our breach of any license agreements or our failure to obtain a license to proprietary rights required to develop or commercialize our future products may have a material adverse impact on us. If third parties prepare and file patent applications in the United States that also claim technology to which we have rights, we may have to participate in interference or derivation proceedings in the USPTO to determine priority of invention. For more information, see "Risk Factors — Risks Related to Our Intellectual Property."

Government Regulation

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an independent IRB at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with cGCPs, requirements to establish the safety and efficacy of the proposed drug product for each indication;
- submission to the FDA of an NDA;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practice requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with cGCPs and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post-approval requirements, including the potential requirement to implement a REMS, and the potential requirement to conduct post-approval studies.

Preclinical Studies

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess potential safety and efficacy. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. Some preclinical testing may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to initiate.

Clinical Trials

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with cGCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA. In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it initiates at that institution. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their www.clinicaltrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.

- Phase 2: The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

- Phase 3: The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Phase 1, Phase 2 and Phase 3 trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA, for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to FDA because the FDA has approximately two months to make a "filing" decision.

The FDA also may require submission of a REMS plan to ensure that the benefits of the drug outweigh its risks. The REMS plan could include medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

The FDA may refer an application for a novel drug to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with cGCP requirements.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response

letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Special FDA Expedited Review and Approval Programs

The FDA has various programs, including fast track designation, accelerated approval, priority review, and breakthrough therapy designation, which are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. The FDA may review sections of the NDA for a fast track product on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

The FDA may give a priority review designation to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. Under the new PDUFA agreement, these six and ten month review periods are measured from the "filing" date rather than the receipt date for NDAs for new molecular entities, which typically adds approximately two months to the timeline for review and decision from the date of submission. Most products that are eligible for fast track designation are also likely to be considered appropriate to receive a priority review.

In addition, products tested for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require a sponsor of a drug receiving accelerated approval to perform post-marketing studies to verify and describe the predicted effect on IMM or other clinical endpoint, and the drug may be subject to accelerated withdrawal procedures.

Moreover, under the provisions of the Food and Drug Administration Safety and Innovation Act, or FDASIA, passed in July 2012, a sponsor can request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. We may explore some of these opportunities for our product candidates as appropriate.

Accelerated Approval Pathway

The FDA may grant accelerated approval to a drug for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on IMM, and that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a drug.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a drug, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of drugs for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. As a result, a drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may designate a drug product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing

and making a drug product available in the United States for treatment of the disease or condition will be recovered from sales of the product). A company must request orphan product designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will be receiving orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances. If a drug or drug product designated as an orphan product ultimately receives marketing approval for an indication broader than what was designated in its orphan product application, it may not be entitled to exclusivity. Orphan exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There are continuing, annual user fee requirements for any marketed products and the establishments where such products are manufactured, as well as new application fees for supplemental applications with clinical data.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval of a drug or medical device is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;

- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs or devices may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

U.S. Healthcare Fraud and Abuse Laws and Compliance Requirements

We are subject to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our proposed sales and marketing programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our operations include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. The term "remuneration" has been broadly interpreted to include anything of value;
- federal false claims and civil monetary penalties laws, including the federal civil False Claims Act, which prohibits anyone from, among other things, knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services that are false or fraudulent;
- provisions of HIPAA, which created federal criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program or making false statements in connection with the delivery of or payment for healthcare benefits, items or services. In addition, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, impose certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- the federal Physician Payments Sunshine Act requirements, under the Patient Protection and Affordable Care Act, which require manufacturers of certain drugs and biologics to track and report to Centers for Medicare & Medicaid Services, or CMS, payments and other transfers of value they make to U.S. physicians and teaching hospitals as well as physician ownership and investment interests in the manufacturer.

Regulation Outside the United States

To the extent that any of our product candidates, once approved, are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws and implementation of corporate compliance programs and reporting of payments or other transfers of value to healthcare professionals.

To market our future products in the European Economic Area, or EEA (comprised of the 28 member states of the EU plus Norway, Iceland and Liechtenstein), and many other foreign jurisdictions, we must obtain separate regulatory approvals. More concretely, in the EEA, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations:

- The Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use of the

EMA and which is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products and medicinal products indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, autoimmune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU; and

- National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member State through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure.

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA assess the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

Data and Marketing Exclusivity

In the EEA, new products authorized for marketing, or reference products, qualify for eight years of data exclusivity and an additional two years of market exclusivity upon marketing authorization. The data exclusivity period prevents generic or biosimilar applicants from relying on the preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization in the EU during a period of eight years from the date on which the reference product was first authorized in the EU. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until 10 years have elapsed from the initial authorization of the reference product in the EU. The 10-year market exclusivity period can be extended to a maximum of eleven years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

Orphan Drug Designation

In the EEA, a medicinal product can be designated as an orphan drug if its sponsor can establish that the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the EU when the application is made, or that the product is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that the marketing of the drug in the EU would generate sufficient return to justify the necessary investment in development. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the drug will be of significant benefit to those affected by that condition.

In the EEA, an application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. Marketing authorization for an orphan drug leads to a ten-year period of market exclusivity. During this market exclusivity period, the EMA or the member state competent authorities, cannot accept another application for a marketing authorization, or grant a marketing authorization, for a similar medicinal product for the same indication. The period of market exclusivity is extended by two years for medicines that have also complied with an agreed pediatric investigational plan.

This period may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation, for example because the product is sufficiently profitable not to justify market exclusivity. Market exclusivity can be revoked only in very

selected cases, such as consent from the marketing authorization holder, inability to supply sufficient quantities of the product, demonstration of "clinical superiority" by a similar medicinal product, or, after a review by the Committee for Orphan Medicinal Products, requested by a member state in the fifth year of the marketing exclusivity period (if the designation criteria are believed to no longer apply). Medicinal products designated as orphan drugs are eligible for incentives made available by the EU and its Member States to support research into, and the development and availability of, orphan drugs.

Other U.S. Regulatory Matters

Manufacturing, sales, promotion and other activities following product approval are also subject to regulation by numerous regulatory authorities in the United States in addition to the FDA, including the Centers for Medicare & Medicaid Services, other divisions of the Department of Health and Human Services, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments.

For example, in the United States, sales, marketing and scientific and educational programs also must comply with state and federal fraud and abuse laws. These laws include the federal Anti-Kickback Statute, which makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. Moreover, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

Pricing and rebate programs must comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990 and more recent requirements in the ACA. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals or refusal to allow a firm to enter into supply contracts, including government contracts. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Prohibitions or restrictions on sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

U.S. Patent-Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of any future product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act permits restoration of the patent term of up to five years as compensation for

patent term lost during product development and FDA regulatory review process. Patent-term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent-term restoration period is generally half the time between the effective date of an IND and the submission date of an NDA or BLA plus the time between the submission date of an NDA or BLA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA or BLA.

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of a NDA for a new molecular entity. A drug is a new molecular entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for a NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Coverage and Reimbursement

Sales of our products will depend, in part, on the extent to which our products will be covered by third party payors, such as government health programs, commercial insurance and managed healthcare organizations. In the United States no uniform policy of coverage and reimbursement for drug products exists. Accordingly, decisions regarding the extent of coverage and amount of reimbursement to be provided for any of our products will be made on a payor-by-payor basis. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

The United States government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. For example, the ACA contains provisions that may reduce the profitability of drug products through increased rebates for drugs reimbursed by Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Adoption of general controls and measures, coupled with the tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceutical drugs.

The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. The ACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on

most branded prescription drugs from 15.1% of average manufacturer price, or AMP, to 23.1% of AMP and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The ACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization and by enlarging the population potentially eligible for Medicaid drug benefits. CMS has proposed to expand Medicaid rebate liability to the territories of the United States as well.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Unlike Medicare Part A and B, Part D coverage is not standardized. While all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval. However, any negotiated prices for our products covered by a Part D prescription drug plan likely will be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs. In addition, as 340B drug prices are determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

As noted above, the marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third party payors fail to provide adequate coverage and reimbursement. An emphasis on cost containment measures in the United States has increased, and we expect will continue to increase, the pressure on pharmaceutical pricing. Coverage policies and third party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In addition, in most foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower.

Scientific Advisory Board

We have assembled a highly qualified scientific advisory board who collectively have deep domain expertise in neurodegenerative diseases, infectious disease in the brain, drug development and translational medicine.

Joseph Berger, M.D. is Professor of Neurology and MS Division Chief in Department of Neurology, Perelman School of Medicine, University of Pennsylvania. Dr. Berger is a key opinion leader in the treatment of multiple sclerosis and other disorders of the central nervous system. Board Certified in Internal Medicine and Neurology, Dr. Berger was trained in Internal Medicine at Georgetown University and did Neurology residencies at the Hadassah Hospital in Jerusalem, Israel and the University of Miami School of Medicine before joining the Faculty at the University of Miami where he conducted research and practiced medicine. He subsequently moved to the University of Kentucky where he was chair of the Department of Neurology for 18 years. In 2014, Dr. Berger joined the Faculty at the University of Pennsylvania in the Department of Neurology. He has conducted extensive research in the treatment of multiple sclerosis and has been a leading investigator into the cause and treatment of Progressive Multifocal Leukoencephalopathy, which arises as a consequence of biologic treatments for MS. Dr. Berger is a member of the International Society for NeuroVirology, Member of the board of directors and Counselor, International Society for NeuroVirology, the board of directors of the International Society for NeuroVirology, Co-Chair of the "International NeuroAIDS Working Group" at the 5th International Symposium of NeuroVirology, Baltimore, Maryland.

Dr. David Clifford, M.D. is Melba and Forest Seay Professor of Clinical Neuropharmacology in Neurology, Washington University in St. Louis, and has a broad interest in neuropharmacology. Development of more successful medical management of neurological disease has been his clinical focus, and has included participation in studies of epilepsy, Parkinson's disease, multiple sclerosis, and virtually all neurologic complications of HIV. His present focus is on developing optimal treatments for neurologic complications of HIV, including HIV-associated dementia, painful peripheral neuropathies in HIV, progressive multifocal leukoencephalopathy, cryptococcal meningitis, toxoplasma encephalitis, primary CNS lymphoma, cytomegalovirus encephalitis/radiculomyelitis, and HIV myelopathy. Dr. Clifford leads a nationwide clinical research group, the Neurologic AIDS Research Consortium, funded by NINDS of the National Institute of Health, whose specific mission is to pursue better treatments for HIV associated neurologic complications. He is also Principal Investigator for the Washington University AIDS Clinical Trials Unit. He is working in international studies, particularly cooperating with medical schools of Ethiopia to further diagnosis and treatment of HIV in that country. Dr. Clifford has served as President of the medical staff of St. Louis ConnectCare, the corporation providing indigent health care in the St. Louis region and as Medical Director of Neurology for Barnes Jewish Hospital. On a national basis, Dr. Clifford has chaired the Neurology Section of the AIDS Clinical Trials Group, served on the Scientific Program Committees for the American Academy of Neurology and for the Conference on Retroviruses and Opportunistic Infections, as a Counsellor for the American Neurological Association, chairs the External Advisory Committee for the National NeuroAIDS Tissue Consortium and is a member of the American Federation for AIDS Research Scientific Advisory Board. He sits on the editorial boards of Journal of NeuroVirology and NeuroAIDS and is a frequent ad hoc reviewer for the major neurologic journals.

Dr. Ted Dawson, M.D., Ph.D., is a director at the Institute for Cell Engineering and Professor of Neurology at The John Hopkins University School of Medicine. He focuses on movement disorders, and many advances in neurobiology of disease have stemmed from Dr. Dawson's identification of the mechanisms of neuronal cell death and the elucidation of the molecular mechanisms of neurodegeneration. He pioneered the role of nitric oxide in neuronal injury in stroke and excitotoxicity and elucidated the molecular mechanisms by which nitric oxide and poly (ADP-ribose) polymerase kills neurons. His studies of nitric oxide led to major insights into the neurotransmitter functions of this gaseous messenger molecule. He co-discovered the neurotrophic properties of non-immunosuppressant immunophilin ligands. Dr. Dawson's discoveries have led to innovative approaches and enhanced the development of new agents to treat neurologic disorders, such as Parkinson's disease and Alzheimer's disease as well as other neurodegenerative disorders. For his participation on the Scientific Advisory Board, Dr. Dawson has received options for 150,000 shares of our common stock with an exercise price of $2.02 per share, which expire on December 31, 2027.

Dr. Valina Dawson, Ph.D., is a Professor of Neurology, Neuroscience, Physiology and the Graduate Program in Cellular & Molecular Medicine at the John Hopkins University School of Medicine. She is co-director of the Neuroregeneration and Stem Cell Programs in the Institute for Cell Engineering. Dr. Dawson's laboratory is actively engaged in discovering and defining cell signaling pathways that lead to either neuronal survival or neuronal death. She explores the role of the monogenic forms of Parkinson's disease with a focus on parkin, EIF4G1 and LRRK2 in order to begin to define the biochemical signaling important to Parkinson's disease. She has developed yeast, cellular, fly and mouse models to explore the Parkinson's disease causing mutations as well as studying human neuronal cultures and human postmortem tissue explore survival and disease signaling events relevant to Parkinson's disease. and stroke as well as to define neuron survival networks. For her participation on the Scientific Advisory Board, Dr. Dawson has received options for 150,000 shares of our common stock with an exercise price of $2.02 per share, which expire on December 31, 2027.

Dr. Warren Olanow, M.D., FRCPC is the Henry P. and Georgette Goldschmidt Professor and Chairman Emeritus of the Department of Neurology, and Professor in the Department of Neuroscience at the Mount Sinai School of Medicine in New York City. He received his medical degree from the University of Toronto, performed his neurology training at the New York Neurological Institute at Columbia Presbyterian Medical Center at Columbia University, and did post-graduate studies in neuroanatomy at Columbia University. He served on the faculties of McGill University, Duke University, and the University of South Florida prior to joining Mount Sinai. He was the recipient of the Movement Disorder Research Award from the American Academy of Neurology. He is a member of the executive committee of the Michael J Fox Foundation Scientific Advisory Board and has served on numerous additional medical and scientific advisory boards. He has served on several editorial boards including as Editor-in-Chief of the journal Movement Disorders. His clinical and basic science research efforts are directed toward defining more effective therapies for Parkinson's disease and other neurodegenerative disorders. Dr. Olanow has authored more than 350 publications, and was ranked #1 in the United States in citations for Parkinson's disease during the past quarter century. He has lectured on movement disorders at Universities and Conferences throughout the world.

Karl Kieburtz, M.D., M.P.H., is the Robert J. Joynt Professor in Neurology, Senior Associate Dean for Clinical Research and Director of the Clinical & Translational Science Institute at the University of Rochester Medical Center. He is also Professor of Public Health Sciences and of Environmental Medicine, and was the founding Director of the Center for Human Experimental Therapeutics (CHET). CHET conducts learning phase clinical trials in a wide spectrum of disorders in collaboration with investigators within the URMC as well as with colleagues throughout North America, Europe, Asia and Oceania. Dr. Kieburtz's primary clinical and research interests are neurodegenerative diseases affecting the basal ganglia, particularly Parkinson disease, Huntington disease, and HIV related neurologic disorders. He is the principal investigator for the NINDS sponsored trials of neuroprotective agents for PD (NET-PD) and directed the Coordination Center for an NEI-funded consortium in Neuro-opthalmology. He completed his M.D. and M.P.H. degrees at the University of Rochester, as well as his Neurology residency and a fellowship in Experimental Therapeutics.

Employees

As of August 17, 2018, we have two full-time employees, and five contractors that collectively comprise our management team. All but one of these individuals holds a Ph.D. or an M.D. Our employees and contractors are located in Boston, Connecticut and Atlanta. The Company is currently converting contract and consulting management team members into regular employees and expects to add five additional employees shortly after the completion of this offering. None of our employees are represented by a labor union or covered under a collective bargaining agreement.

Facilities

Our corporate headquarters are located in Atlanta, Georgia, where we lease a single corporate office. Additionally, we have offices in Cambridge and South Boston, Massachusetts which we use as conference spaces for our team, most of whom are based in the surrounding area. It is anticipated that these distant facilities will be consolidated in the Boston, Massachusetts area in early 2019.

Legal Proceedings

From time to time, we may become involved in litigation or other legal proceedings. We are not currently a party to any litigation or legal proceedings. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

MANAGEMENT

Executive Officers and Directors

The following table sets forth the names, ages and positions of our executive officers and directors as of August 17, 2018:

Name	Age	Position
Executive Officers:		
Milton H. Werner, Ph.D.	55	President, Chief Executive Officer and Director
Inder Kaul, M.D., M.P.H.	62	Interim Chief Medical Officer
Joseph Frattaroli	56	Chief Financial Officer
Non-Employee Directors:		
Peter Mueller, Ph.D.[(1)(2)(3)]	62	Chairperson of the board of directors
Lisa Evrén[(1)(4)]	63	Director
Richard F. Fante[(1)(2)(3)(5)]	53	Director
Hilary Malone, M.D.[(3)(6)]	53	Director

(1) Member of the audit committee

(2) Member of the compensation committee

(3) Member of the corporate governance and nominating committee

(4) Chair of audit committee

(5) Chair of compensation committee

(6) Chair of corporate governance and nominating committee

Executive Officers

Milton H. Werner, Ph.D. has been our President and Chief Executive Officer and a member of our board of directors since our formation as a Delaware corporation in June 2010. He founded our predecessor, Inhibikase Therapeutics, LLC in 2008 as an entrepreneurial start-up in Atlanta, Georgia with initial financial support from the Georgia Research Alliance. Prior to founding Inhibikase, from May 2007 until August 2008, Dr. Werner served as Director of Research at Celtaxsys, Inc., a cell-free immunotherapeutics company. From September 1996 until June 2007, Dr. Werner was a Head of the Laboratory of Molecular Biophysics at The Rockefeller University and departed the University at the rank of Associate Professor. While at The Rockefeller University, Dr. Werner focused on developing more complete understandings of mechanisms of human disease in immunology, oncology and infectious disease.

Dr. Werner is the author or co-author of more than 70 research articles, reviews and book chapters and has given lectures on his research work on more than 150 occasions throughout the world. He is the recipient of numerous private and public research grants totaling more than $10 million, and of several awards, including the Young Investigator Award from the Sidney Kimmel Cancer Foundation, the Research Chair from the Brain Tumor Society and a $1 million Distinguished Young Scholars in Medical Research award from the W. M. Keck Foundation. Dr. Werner received his Ph.D. in Chemistry from the University of California, Berkeley and his B.S. in Biochemistry from the University of Southern California.

We believe Dr. Werner is qualified to serve on our board of directors because of the perspective and experience he provides as our founder and as our President and Chief Executive Officer, as well as his experience within the pharmaceutical industry, particularly in the area of neuroscience, infectious disease and drug discovery and development.

Dr. Werner is an Adjunct Full Professor in the School of Biology at the Georgia Institute of Technology and a Member of the Winship Cancer Institute of Emory University, both in Atlanta, Georgia.

Inder Kaul, M.D., M.P.H. has served as our Interim Chief Medical Officer since July 2015 and as the President of Kaul Consulting, LLC since May 2015. From July 2009 until April 2015, Dr. Kaul served as President of Product Development and Chief Medical Officer of Asahi Kasei Pharma America (AKP America), a wholly-owned subsidiary of Asahi Kasei Pharma Corp. From July 2006 until June 2009, Dr. Kaul served as Vice President, Clinical Development, Medical & Regulatory Affairs of Oscient Pharmaceuticals. From July 1998 until June 2006, Dr. Kaul served as Division Vice President of Abt Associates Clinical Trials, a division of Abt Associates, Inc. From May 1995 until July 1998, he served as Vice President, Clinical & Medical Affairs of Medical and Technical Research Associates, Inc., which later merged with Applied Analytical Industries Inc. From April 1994 until May 1995, Dr. Kaul served as Medical Director for International Medical Operations at Searle, now Pfizer Inc. From January 1992 until April 1994, he was the Associate Director Clinical Research (Virology & Immunology) at Boehringer-Ingelheim, after having worked as Director Medical Services at Parexel International Corporation from March 1990 until January 1992. Dr. Kaul started his career in the industry with Candela Laser Corporation in July 1988 as a clinical research monitor. Dr. Kaul received his M.D. in 1983 and his M.P.H. in 1988 from Harvard University. We believe Dr. Kaul is qualified to serve on our board of directors because of his extensive experience in program development, project management, research and development leadership for multiple therapeutic areas which have resulted in successful product registrations and post marketing activities.

Joseph Frattaroli has served as our Chief Financial Officer since April 2018. Mr. Frattaroli is a certified public accountant with more than 15 years of experience in public company filings and compliance for Nasdaq and OTC Markets companies. He founded Flagship Consulting, Inc. in January 2010, through which he has provided chief financial officer and consulting services for several emerging biopharmaceutical and medical device companies, with responsibilities that included capital formation, deal structuring, and assisting private companies in their transition to becoming publicly traded SEC registrants. He has also served as an independent consultant to Danforth Advisors LLC since July 2015, providing interim chief financial officer and strategic advisory services to emerging public and private biotechnology and biopharmaceutical clients of Danforth Advisors LLC. Mr. Frattaroli received his BS in Accounting from Salem State University and was certified as a public accountant while employed by Ernst & Young, LLP.

Key Non-Executive Officers

Roger Rush, Ph.D., our Head of Preclinical Research is an experienced veteran of the pharmaceutical industry with over 30 years of experience working in the United Kingdom and U.S. for small and large pharmaceutical companies and contract research organizations, and is now based in the Greater Boston area. His major career focus has been on preclinical research and development, safety assessment and the translation of discovery research molecules into clinical development. He has contributed to over 20 IND, CTA and product license submissions and approved drugs including nicardipine (Cardene), ranolazine (Ranexa), Foscan, and zileuton (Zyflo CR). He has been a principal of Allon Preclinical Consulting, LLC since 2015. From March 2012 to December 2014, he was Vice President Preclinical Development for Idenix Pharmaceuticals, Inc., a wholly owned subsidiary of Merck & Company, Inc., where he managed the DMPK, toxicology and discovery research that lead to the identification of lead molecules to treat Hepatitis C virus. His work has spanned numerous therapeutic areas, including anti-inflammatory, anti-allergy, arthritis, anti-infectives, CNS, cardiovascular, oncology, genitourinary and anti-hyperlipidaemics. He received his B.Sc. and Ph.D. in Biochemistry from the University of Surrey in the United Kingdom.

Surendra Singh, Ph.D. currently serves as our head of Chemistry, Manufacturing and Controls (CMC). He is an expert on chemical process research & development, from lead optimization to launch, technology transfer and API manufacturing. From 2001 to 2011, he served various roles at Sunovion Pharmaceuticals Inc. and its predecessor, Sepracor Inc., including as a director of chemical process research, and has served as a chemistry, manufacturing, and control consultant for AVEO Oncology, RestorGenex Corporation, Verastem Oncology, and Syner-G Pharma Consulting, LLC. Dr. Singh received his doctoral degree from the Indian Institute of Technology from 1991, and was a post-doctoral fellow at The Ohio State University. Dr. Singh establishes and manages the commercial process, global outsourcing, and global vendor management, as well as participates in all aspects of the drafting and review of regulatory documents from the IND to NDA.

Terence Kelly, Ph.D., currently serves as our Head of Medicinal Chemistry and Drug Discovery. He is a 30-year pharmaceutical industry veteran and has served as a member of the board of directors of Cardax, Inc. since June 2014. He is a founder of Kelly Pharma Research Consulting, LLC and has served as its President since January 2010. From June 2010 to July 2017, he held several positions at CoMentis, including most recently President and CEO. From July 2002 to December 2009, he served as Vice-President of Medicinal Chemistry at Boehringer Ingelheim Pharmaceuticals, Inc. At the Company, Dr. Kelly developed processes for the RAMP drug discovery program.

Non-Employee Directors

Lisa Evrén is the former Vice President and Treasurer at Talisman-Energy in Calgary, Alberta, Canada, serving from 2010 until 2013. From 2007 until 2010, Ms. Evrén served as the Executive Vice President and Chief Financial Officer of Merrimack Pharmaceuticals, Inc. in Cambridge, Massachusetts, a privately held specialty pharmaceutical company focused on oncology. Prior to Merrimack, Ms. Evrén held roles of increasing responsibility in finance at Amgen Inc. from 2002 until 2007 and Pfizer Inc. from 1991 until 1997. Ms. Evrén received her B.A. in Philosophy from Pomona College and her J.D. and L.L.M. (Taxation) from New York University. Ms. Evrén's qualifications to serve on the board of directors include her experience as a chief financial officer in the pharmaceutical industry and in the structuring and execution of global capital markets and private funding transactions

Richard Fante has served as Chief Commercial Officer and Head of Business Development for Innocoll, Inc. since August 2015 and as President of RF Consulting LLC since April 2013. Prior to founding RF Consulting LLC, Mr. Fante spent over nineteen years at AstraZeneca plc, or AstraZeneca, in the United States. He served in a number of roles, most recently as President of its U.S. business, CEO of North America and Regional Vice President of the Americas. Prior to assuming his role as President of AstraZeneca US in November 2008, Mr. Fante served as the head of Brand Strategy and Portfolio Operations at AstraZeneca. From January 1992 until December 1994, Mr. Fante worked at Lederle Laboratories where his roles included sales representative and brand manager. Mr. Fante served as Board Chairman of the National Pharmaceutical Council (2012) and was a member of the Institute of Medicine of the National Academies of Science Roundtable on Value and Science. Mr. Fante received his B.A. in Biology from Princeton University and his M.B.A. from the University of North Carolina at Chapel Hill. Mr. Fante's qualifications to serve on the board of directors include his experience as a pharmaceutical executive and his leadership in the development and commercialization of pharmaceutical products.

Dr. Hilary Malone is an experienced business leader in the pharmaceutical industry with specialist expertise in global regulatory affairs and compliance spanning the development, registration, and marketing of innovative prescription drugs and vaccines. Dr. Malone currently serves as Chief Regulatory Officer and Head of Global Regulatory Affairs for Sanofi, S.A., a position she has held since July 2013. Prior to joining Sanofi, S.A., Dr. Malone served as the Chief Regulatory & Compliance Officer for Reata Pharmaceuticals, a small U.S.-based biotech company from 2011 to 2013. Dr. Malone also held roles as the Senior Vice President and Head of Worldwide Regulatory Strategy for both Wyeth, from 2006 to 2009 and Pfizer Inc., from 2009 to 2011, overseeing all products across the human health businesses (pharmaceuticals and vaccines, consumer health brands, generics, and infant nutritionals). Earlier in her career, Dr. Malone held positions of increasing responsibility at GlaxoSmithKline plc and its predecessor SmithKline Beecham plc from 1993 to 2001 and AstraZeneca from 2011 to 2006. Dr. Malone earned her B.Sc. in Physiology and her Ph.D. in Molecular Neuropharmacology from the University of Dundee, Scotland, U.K. and also spent time in postdoctoral research at the University of California, San Francisco prior to joining the pharmaceutical industry. Dr. Malone's qualifications to serve on the board of directors include her experience in global regulatory affairs and compliance spanning the development, registration, marketing and commercialization of innovative prescription drugs and vaccines.

Dr. Peter Mueller has served as the President of the Mueller Health Foundation, a private foundation dedicated to combatting globally lethal infectious diseases such as tuberculosis, since January 2017. Previously, he served as President of R&D and Chief Scientific Officer of Axcella Health from November 2014 until January 2017. From July 2003 to October 2014, Dr. Mueller served as Executive Vice President Global Research and Development & Chief Scientific Officer for Vertex Pharmaceuticals, where he provided strategic oversight for Vertex's worldwide drug discovery research programs, pharmaceutical

development, quality assurance and control, and pharmaceutical operations as well as clinical and nonclinical development, regulatory, patient safety, and medical affairs. Prior to his tenure at Vertex, Dr. Mueller served as Senior Vice President, Research and Development, for Boehringer Ingelheim from April 1997 until June 2003, where he was responsible for the development of all drug candidates of the company's worldwide portfolio in North and South America, Canada and Japan. He also led research programs in the areas of immunology, inflammation, cardiovascular disease and gene therapy on a global basis. During his time at Boehringer Ingelheim, Dr. Mueller oversaw the discovery of numerous development candidates and held several positions in basic research, medicinal chemistry, and management at various locations of Boehringer Ingelheim worldwide.

Dr. Mueller received both his undergraduate degree and his Ph.D. in Chemistry at the Albert Einstein University of Ulm, Germany, where he also received a Professorship in Theoretical Organic Chemistry. He completed fellowships in Quantum Pharmacology at Oxford University and in Biophysics at the University of Rochester.

His special fields of studies include synthetic organic chemistry, computational chemistry (cheminformatics and bioinformatics), RNA-biophysics, atherosclerosis research, IMID (Immune Mediated Inflammatory Diseases), neurodegenerative diseases, infection, oncology, gene/epigenetic technology, artificial intelligence and management/business strategies (IMD).

He is a member of various scientific and political societies, including the Gesellschaft Deutscher Chemiker (GDCh) and Verband Chemische Industrie (Germany), Royal Society of Chemistry (UK), IRI, RNA-Society, ASAP, AAAS (USA). He currently also serves as the Chairman of the board of directors of BioXcel Therapeutics, Inc. and as a director on the board of the US-India Chamber of Commerce Biotech. He also serves as a chair of the Scientific Advisory Board for BioXcel Corporation, is an advisor to the University Iowa (CBB) and is a faculty member of the GLG Institute. Dr. Mueller served on the Advisor Committee at the Harvard Accelerator Fund, and SAB for Keystone Symposia. Before he left Connecticut to join Vertex, Dr. Mueller was also a member of Governor Roland's Council on Economic Competitiveness and Technology for the State of Connecticut (USA).

Dr. Mueller's qualifications to serve on the board of directors include his experience as a pharmaceutical executive and his leadership in the development and commercialization of pharmaceutical products.

Family Relationships

No family relationships exist between any director, executive officer or person nominated or chosen to be a director or officer.

Board of Directors Composition

Our board of directors currently consists of five members. After the completion of this offering, the number of directors will be fixed by our board of directors, subject to the terms of our amended and restated certificate of incorporation and amended and restated bylaws. Each of our current directors will continue to serve as a director until the election and qualification of his or her successor, or until his or her earlier death, resignation or removal.

Our amended and restated certificate of incorporation will provide that our board of directors will be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of stockholders, with the other classes continuing for the remainder of their respective three-year terms. Our current directors will be divided among the three classes as follows:

- the Class I director will be Dr. Werner, and his term will expire at the annual meeting of stockholders to be held in 2019;
- the Class II directors will be Ms. Evrén and Mr. Fante, and their terms will expire at the annual meeting of stockholders to be held in 2020; and
- the Class III directors will be Drs. Malone and Mueller, and their terms will expire at the annual meeting of stockholders to be held in 2021.

At each annual meeting of stockholders, upon the expiration of the term of a class of directors, the successor to each such director in the class will be elected to serve from the time of election and qualification until the third annual meeting following his or her election and until his or her successor is duly elected and qualified, in accordance with our amended and restated certificate of incorporation. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of our directors.

This classification of our board of directors may have the effect of delaying or preventing changes in control of our company.

In addition, under the terms of our amended and restated certificate of incorporation and our amended and restated bylaws, members of our board of directors may only be removed for cause. This may also have the effect of delaying or preventing changes in control of our company.

Director Independence

Upon the completion of this offering, our common stock will be listed on the NASDAQ Capital Market, or NASDAQ. Under the rules of NASDAQ, independent directors must comprise a majority of a listed company's board of directors within one year of the completion of its initial public offering. In addition, the rules of NASDAQ require that, subject to specified exceptions, each member of a listed company's audit, compensation and corporate governance and nominating committees be independent. Audit committee members and compensation committee members must also satisfy the independence criteria set forth in Rule 10A-3 and Rule 10C-1, respectively, under the Exchange Act. Under the rules of NASDAQ, a director will only qualify as an "independent director" if, in the opinion of that company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

To be considered to be independent for purposes of Rule 10A-3 and under the rules of NASDAQ, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board of directors committee: (1) accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries; or (2) be an affiliated person of the listed company or any of its subsidiaries.

To be considered independent for purposes of Rule 10C-1 and under the rules of NASDAQ, the board of directors must affirmatively determine that the member of the compensation committee is independent, including a consideration of all factors specifically relevant to determining whether the director has a relationship to the company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (i) the source of compensation of such director, including any consulting, advisory or other compensatory fee paid by the company to such director; and (ii) whether such director is affiliated with the company, a subsidiary of the company or an affiliate of a subsidiary of the company.

Our board of directors undertook a review of its composition, the composition of its committees and the independence of our directors and considered whether any director has a material relationship with us that could compromise his or her ability to exercise independent judgment in carrying out his or her responsibilities. Based upon information requested from and provided by each non-employee director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that none of our directors have relationships that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that each of these directors is "independent" as that term is defined under the rules of NASDAQ. Only Dr. Werner is not independent under NASDAQ's independence standards.

In making these determinations, our board of directors considered the current and prior relationships that each non-employee director has with our company and all other facts and circumstances our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director, and the transactions involving them described in the section titled "Certain Relationships and Related Party Transactions."

Board of Directors Leadership Structure

Upon completion of this offering, our board of directors will be chaired by Dr. Mueller. As a general policy, our board of directors believes that separation of the positions of Chairperson and Chief Executive Officer reinforces the independence of our board of directors from management, creates an environment that encourages objective oversight of management's performance and enhances the effectiveness of our board of directors as a whole. As such, Dr. Werner serves as our President and Chief Executive Officer, while Dr. Mueller will serve as the Chairperson of our board of directors, but will not be an officer. We expect and intend the positions of Chairperson of our board of directors and Chief Executive Officer to continue to be held by two separate individuals in the future.

Board of Directors Committees

Upon completion of this offering our board of directors will have an audit committee, a compensation committee and a corporate governance and nominating committee, each of which will have the composition and the responsibilities described below.

Audit Committee

Upon completion of this offering the members of our audit committee will be Ms. Evrén, Dr. Mueller and Mr. Fante. Ms. Evrén will be the chair of our audit committee, and will be our audit committee financial expert, as that term is defined under the applicable SEC rules, and possesses financial sophistication, as defined under the rules of NASDAQ. All of the members of our audit committee will be independent, as that term is defined under the rules of NASDAQ. Our audit committee will oversee our corporate accounting and financial reporting process and assists our board of directors in monitoring our financial systems. Our audit committee will also:

- select and hire the independent registered public accounting firm to audit our financial statements;
- help to ensure the independence and performance of the independent registered public accounting firm;
- approve audit and non-audit services and fees;
- review financial statements and discuss with management and the independent registered public accounting firm our annual audited and quarterly financial statements, the results of the independent audit and the quarterly reviews and the reports and certifications regarding internal controls over financial reporting and disclosure controls;
- prepare the audit committee report that the SEC requires to be included in our annual proxy statement;
- review reports and communications from the independent registered public accounting firm;
- review the adequacy and effectiveness of our internal controls and disclosure controls and procedure;
- review our policies on risk assessment and risk management;
- review related party transactions; and
- establish and oversee procedures for the receipt, retention and treatment of accounting related complaints and the confidential submission by our employees of concerns regarding questionable accounting or auditing matters.

Our audit committee will operate under a written charter, to be effective prior to the completion of this offering, which will satisfy the applicable rules of the SEC and the listing standards of NASDAQ.

Compensation Committee

Upon completion of this offering the members of our compensation committee will be Mr. Fante and Drs. Malone and Mueller. Mr. Fante will be the chair of our compensation committee. All of the members of our compensation committee will be independent, as that term is defined under the rules of NASDAQ. Our compensation committee oversees our compensation policies, plans and benefits programs. The compensation committee will also:

- oversee our overall compensation philosophy and compensation policies, plans and benefit programs;
- review and approve or recommend to our board of directors for approval compensation for our executive officers and directors;
- prepare the compensation committee report that the SEC would require to be included in our annual proxy statement if we were no longer deemed to be an emerging growth company or a smaller reporting company; and
- administer our equity compensation plans.

Our compensation committee will operate under a written charter, to be effective prior to the completion of this offering, which will satisfy the applicable rules of the SEC and the listing standards of NASDAQ.

Corporate Governance and Nominating Committee

Upon completion of this offering the members of our corporate governance and nominating committee will be Drs. Malone and Mueller and Mr. Fante. Dr. Malone will be the chair of our corporate governance and nominating committee. All will be independent, as that term is defined under the rules of NASDAQ. Our corporate governance and nominating committee oversees and assists our board of directors in reviewing and recommending nominees for election as directors. Specifically, the corporate governance and nominating committee will:

- identify, evaluate and make recommendations to our board of directors regarding nominees for election to our board of directors and its committees;
- consider and make recommendations to our board of directors regarding the composition of our board of directors and its committees;
- review developments in corporate governance practices;
- evaluate the adequacy of our corporate governance practices and reporting; and
- evaluate the performance of our board of directors and of individual directors.

Our corporate governance and nominating committee will operate under a written charter, to be effective prior to the completion of this offering, which will satisfy the applicable rules of the SEC and the listing standards of NASDAQ.

Director Compensation

Directors are compensated for each meeting. We reimburse our directors for expenses associated with attending meetings of our board of directors and its committees and we issue non-qualified stock options on an annual basis.

The following table presents the total compensation received by each of our non-employee directors during the year ended December 31, 2017.

Name	Fees Earned or Paid in Cash ($)	Option Awards ($)[1]	Total ($)
Ms. Lisa Evrén[2]	4,800	40,140	44,940
Mr. Richard Fante[3]	4,800	40,140	44,940
Dr. Hilary Malone[4]	4,800	40,140	44,940
Dr. Peter Mueller[5]	4,800	40,140	44,940

(1) The amounts disclosed represent the aggregate grant date fair value of the award as calculated in accordance with FASB Accounting Standards Codification Topic 718, or ASC 718. The assumptions used in calculating the grant date fair value of the award disclosed in this column are set forth in the notes to our audited financial statements included elsewhere in this prospectus. These amounts do not correspond to the actual value that may be recognized by the directors upon vesting of the applicable awards.

(2) As of December 31, 2017, Ms. Evrén held options to purchase 175,000 shares of our common stock and 152,083 shares subject to such options were vested as of such date.

(3) As of December 31, 2017, Mr. Fante held options to purchase 89,583 shares of our common stock and 66,666 shares subject to such options were vested as of such date.

(4) As of December 31, 2017, Dr. Malone held options to purchase 75,000 shares of our common stock and 52,083 shares subject to such options were vested as of such date.

(5) As of December 31, 2017, Dr. Mueller held options to purchase 175,000 shares of our common stock and 152,083 shares subject to such options were vested as of such date.

In August 2018, our board of directors adopted our outside director compensation policy. Members of our board of directors who are not employees are eligible for compensation under our outside director compensation policy. Our outside director compensation policy will be effective as of the effective date of the registration statement of which this prospectus forms a part. Under our outside director compensation policy, each non-employee director will be eligible to receive compensation for his or her service consisting of annual cash retainers and equity awards as described below. Our board of directors may revise outside director compensation as it deems necessary or appropriate.

Cash Compensation

Under our outside director compensation policy, all non-employee directors will be entitled to receive the following cash compensation for their services following the effective date of the registration statement of which this prospectus forms a part:

- $40,000 per year for service as a board member;
- $30,000 per year additionally for service as non-executive Chairperson of the Board;
- $20,000 per year additionally for service as chair of the audit committee;
- $5,000 per year additionally for service as member of the audit committee (excluding committee chair);
- $10,000 per year additionally for service as chair of the compensation committee;
- $5,000 per year additionally for service as member of the compensation committee (excluding committee chair);
- $5,000 per year additionally for service as chair of the corporate governance and nominating committee;

- $3,000 per year additionally for service as member of the corporate governance and nominating committee (excluding committee chair);

All cash payments to non-employee directors who served in the relevant capacity at any point during the immediately preceding prior fiscal quarter will be paid quarterly in arrears on a prorated basis. A non-employee director who served in the relevant capacity during only a portion of the prior fiscal quarter will receive a pro-rated payment of the quarterly payment of the applicable cash retainer.

Employee Directors will receive $25,000 per year as a member of the Board of Directors.

Equity Compensation

All non-employee Directors will receive 25,000 non-qualified stock options with a 12 month vesting period for each year of service. The Chairperson will receive an additional 10,000 non-qualified stock options and Committee Chairs will receive 5,000 non-qualified stock options in recognition of their service. Each such grant would be subject to the terms, conditions and any applicable limits as set forth in the 2018 Plan as described below.

Scientific Advisory Board Compensation

Each member of our scientific advisory board earns $400 per hour for his or her service as a member of our scientific advisory board. We also reimburse each member of our scientific advisory board for all reasonable and necessary expenses in connection with the performance of his or her services. Members of the scientific advisory board who are also our employees or directors receive no additional compensation for their service on the scientific advisory board.

Compensation Committee Interlocks and Inside Participation

None of the members of our compensation committee are or have been an officer or employee of our company. None of our executive officers currently serve, or in the past fiscal year has served, on the board of directors or compensation committee (or other board of directors' committee performing equivalent functions) of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. Following the completion of this offering, the code of business conduct and ethics will be available on our website at www.inhibikase.com. We intend to disclose future amendments to such code, or any waivers of its requirements, applicable to any principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions or our directors on our website identified above. The inclusion of our website address in this prospectus does not include or incorporate by reference the information on our website into this prospectus. We will provide any person, without charge, upon request, a copy of our code of conduct and ethics. Such requests should be made in writing to the attention of Dr. Milton Werner, President and CEO at Inhibikase Therapeutics, Inc., 3350 Riverwood Parkway SE, Suite 1900, Atlanta, GA 30339.

Limitation of Liability and Indemnification

Our amended and restated certificate of incorporation and amended and restated bylaws, each to be effective upon the completion of this offering, will provide that we will indemnify our directors and officers, and may indemnify our employees and other agents, to the fullest extent permitted by Delaware law. Delaware law prohibits our amended and restated certificate of incorporation from limiting the liability of our directors for the following:

- any breach of the director's duty of loyalty to us or to our stockholders;
- acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;

- unlawful payment of dividends or unlawful stock repurchases or redemptions; and
- any transaction from which the director derived an improper personal benefit.

If Delaware law is amended to authorize corporate action further eliminating or limiting the personal liability of a director, then the liability of our directors will be eliminated or limited to the fullest extent permitted by Delaware law, as so amended. Our amended and restated certificate of incorporation does not eliminate a director's duty of care and, in appropriate circumstances, equitable remedies, such as injunctive or other forms of non-monetary relief, remain available under Delaware law. This provision also does not affect a director's responsibilities under any other laws, such as the federal securities laws or other state or federal laws. Under our amended and restated bylaws, we will also be empowered to purchase insurance on behalf of any person whom we are required or permitted to indemnify.

In addition to the indemnification required in our amended and restated certificate of incorporation and amended and restated bylaws, we have entered into an indemnification agreement with each director and each of our officers. These agreements provide for the indemnification of our directors and officers for certain expenses and liabilities incurred in connection with any action, suit, proceeding or alternative dispute resolution mechanism, or hearing, inquiry or investigation that may lead to the foregoing, to which they are a party or other participant, or are threatened to be made a party or other participant, by reason of the fact that they are or were a director, officer, employee, agent or fiduciary of our company, by reason of any action or inaction by them while serving as an officer, director, agent or fiduciary, or by reason of the fact that they were serving at our request as a director, officer, employee, agent or fiduciary of another entity. In the case of an action or proceeding by or in the right of our company or any of our subsidiaries, no indemnification will be provided for any claim where a court determines that the indemnified party is prohibited from receiving indemnification. We believe that these charter and bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against directors and officers, even though an action, if successful, might benefit us and our stockholders. Moreover, a stockholder's investment may be harmed to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to our directors, officers and controlling persons pursuant to the foregoing provisions, or otherwise, we have been advised that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable. There is no pending litigation or proceeding naming any of our directors or officers as to which indemnification is being sought, nor are we aware of any pending or threatened litigation that may result in claims for indemnification by any director or officer.

EXECUTIVE COMPENSATION

Our named executive officers for 2018, which consist of our principal executive officer and the next two most highly compensated executive officers, are:

- Milton H. Werner, Ph.D., our President and Chief Executive Officer;
- Inder Kaul, M.D., M.P.H., our Interim Chief Medical Officer;
- Joseph Frattaroli, C.P.A., our Chief Financial Officer.

Summary Compensation Table

The following table sets forth information regarding the compensation of our named executive officers for the year ended December 31, 2017.

Name and Principal Position	Salary ($)	Option Awards[1]	All Other Compensation ($)	Total ($)
Milton H. Werner, Ph.D. *President and Chief Executive Officer*	$280,800	$40,140	$ 21,419[2]	$342,359
Inder Kaul, M.D., M.P.H. *Interim Chief Medical Officer*	$156,000[3]	—	—	$156,000
Joseph Frattaroli, C.P.A.[4] *Chief Financial Officer*	—	—	—	—

(1) The amount reported represents the aggregate grant date fair value of the award as calculated in accordance with ASC 718. The assumptions used in calculating the grant date fair value of the award disclosed in this column are set forth in the notes to our audited financial statements included elsewhere in this prospectus. The amount does not correspond to the actual value that may be recognized by the named executive officer upon vesting and/or exercise of the applicable awards.

(2) The amount reported represents (i) $12,084 for automobile expenses; (ii) $2,615 for life insurance policy premiums; and (iii) $6,720 for Company contributions to the Simple IRA Plan on Dr. Werner's behalf.

(3) The amount reported represents payments to Dr. Kaul for the year ended December 31, 2017 pursuant to his consulting agreement with the Company.

(4) Mr. Frattaroli became our Chief Financial Officer in February 2018 and did not receive any compensation from the Company in the year ended December 31, 2017.

Outstanding Equity Awards at Fiscal Year-End

The following table sets forth information concerning outstanding equity awards held by each of our named executive officers as of December 31, 2017:

	Option Awards					
Name	Grant Date[1]	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Equity incentive awards: number of securities underlying unexercised unearned options (#)	Option Exercise Price ($)[2]	Option Expiration Date
Milton H. Werner, Ph.D.	6/1/2011	50,000	—	—	0.33	6/1/2021
	6/1/2013	25,000	—	—	1.77	6/1/2023
	3/1/2015	25,000	—	—	1.77	3/1/2025
	11/1/2015	25,000	—	—	2.02	11/1/2025
	11/1/2016	25,000	—	—	2.02	11/1/2026
	11/1/2017	2,083	22,917[3]	—	2.02	11/1/2027
Inder Kaul, M.D., M.P.H.	—	—	—	—	—	—
Joseph Frattaroli, C.P.A.	—	—	—	—	—	—

(1) Each of the outstanding options to purchase shares of our common stock was granted pursuant to our 2011 Plan.

(2) This column represents the fair market value of a share of our common stock on the date of grant, as determined by our board of directors or its authorized committee.

(3) The unvested shares underlying this option vest in 11 substantially equal monthly installments on the first day of each month.

Employment Arrangements with Our Named Executive Officers

Milton H. Werner, Ph.D.

Employment Agreement

On April 1, 2014, the Company entered into a written employment agreement, or the CEO Agreement, with Dr. Werner at an initial base annual salary of $224,000, subject to adjustment by the board of directors. His current base annual salary is $292,800. The CEO Agreement provides an initial ten year fully vested option to purchase 50,000 shares of stock of the Company at an exercise price of $0.33 per share. For so long as he remains employed by the Company, the Company agrees to grant an annual option to purchase 25,000 shares of stock of the Company at an exercise price equal to the fair market value of the shares at the date of the grant and to be vested pro rata in monthly installments over twelve months from the date of the grant, with vesting accelerating upon a change in control of the Company, subject generally to his continued service on such date and/or event. Bonuses, additional stock option grants or other compensation can be awarded from time to time at the sole discretion of the Company's board of directors. As of December 31, 2017, Dr. Werner had received options to purchase up to 175,000 shares of common stock of the Company.

The CEO Agreement provides that Dr. Werner would be eligible to participate in the benefit plans generally made available to other executive officers of the Company. In addition, he is entitled to (i) three weeks of paid vacation per year, (ii) reimbursement for discretionary expenditures (including life insurance premiums, automobile expenses, and country club memberships) up to a maximum of $13,000 annually and

(iii) reimbursement of certain relocation expenses in the event that the Company's headquarters is relocated by more than 25 miles from Atlanta, Georgia, including house hunting expenses, three months of interim housing expenses, and reimbursement for four round trip airline tickets between Atlanta and the new Company headquarters.

The CEO Agreement provides that it shall continue until terminated (i) by mutual agreement; (ii) due to death or disability of Dr. Werner; (iii) by Dr. Werner upon four weeks written notice to the Company; (iv) by the Company without cause (as defined in the CEO Agreement) upon four weeks written notice to Dr. Werner; (v) by Dr. Werner for good reason (as defined in the CEO Agreement); or (vi) by the Company for cause. In the event of a termination for good reason or without cause, Dr. Werner is entitled to six months of salary continuation at his then-current base annual salary, reimbursement of COBRA premiums for six months, accelerated vesting on options that would have vested in the six months following termination had he remained employed, and extended exercise periods for vested stock options. If the termination for good reason or without causes arose in connection with a change in control, the six months of salary continuation and reimbursement of COBRA premiums is extended to 12 months, and all options would become fully vested with extended exercise periods. The receipt of any benefits described above are subject to Dr. Werner's execution of a release.

In addition, the CEO Agreement provides for an excise tax gross-up in the event that Dr. Werner is subject to an excise tax under Sections 280G and 4999 of the Code upon a change in control.

Inder Kaul, M.D., M.P.H.

Dr. Kaul is currently a consultant who will convert to full-time employment following the closing of this offering. Upon conversion, he will be an "at-will" employee who will be able to participate in our 2018 Plan, be provided life and health insurance and can participate in a contributory Simple IRA program through Fidelity Investments.

Joseph Frattaroli, C.P.A.

Mr. Frattaroli is currently a consultant who will convert to full-time employment following the closing of this offering. Upon conversion, he will be an "at-will" employee who will be able to participate in our 2018 Plan, be provided life and health insurance and can participate in a contributory Simple IRA program through Fidelity Investments.

Potential Payments upon Termination or Change in Control

The following chart summarizes the total benefits that would have been payable to the named executive officers upon a termination of employment or a change in control that occurred on December 31, 2017.

Name	Cash Severance Payment ($)	Accelerated Option Vesting ($)	Health Insurance Coverage ($)	Total ($)
Dr. Milton Werner				
Voluntary termination for good reason or involuntary termination without cause	140,400[1]	27,125[2]	10,000[3]	177,525
No termination following a change in control	—	45,208[4]	—	45,208[5]
Voluntary termination for good reason or involuntary termination without cause following a change in control	280,800[6]	45,208[4]	20,000[7]	346,008[5]
Dr. Inder Kaul[8]	—	—	—	—
Mr. Joseph Frattaroli[8]	—	—	—	—

(1) These cash severance payments are made over 6 months.

(2) This amount reflects accelerated vesting on unvested stock options that would have vested within the six months following termination if Dr. Werner had remained employed during that time. It was calculated based on the spread between the price of our common stock as of December 31, 2017 of $4.19 and the exercise price applicable to each such option.

(3) This amount represents COBRA continuation for a period of 6 months for Dr. Werner and any eligible dependents.

(4) This amount reflects accelerated vesting on all unvested stock options. It was calculated based on the spread between the price of our common stock as of December 31, 2017 of $4.19 and the exercise price applicable to each such option.

(5) While Dr. Werner has a contractual entitlement to an excise tax gross-up under his 2014 CEO Agreement, no gross-up would have been triggered had a change in control occurred on December 31, 2017, and therefore no such amounts are included in the table.

(6) These cash severance payments are made over 12 months.

(7) This amount represents COBRA continuation for a period of 12 months for Dr. Werner and any eligible dependents.

(8) As of December 31, 2017, Dr. Kaul and Mr. Frattaroli were not entitled to any of the benefits described in the table upon a termination or change in control.

Employee Benefit and Stock Plans

Simple IRA Plan

We maintain a Simple IRA retirement savings plan for the benefit of our employees, including our named executive officers, who satisfy certain eligibility requirements. Under the Simple IRA, eligible employees may elect to defer a portion of their compensation, within the limits prescribed by the Code, on a pre-tax basis through contributions to the Simple IRA plan. The Simple IRA plan authorizes employer safe harbor contributions. We contribute 3% of gross salary for eligible employees. The Simple IRA plan is intended to qualify under Sections 401(a) and 501(a) of the Code. As a tax-qualified retirement plan, pre-tax contributions to the Simple IRA plan and earnings on those pre-tax contributions are not taxable to the employees until distributed from the Simple IRA plan.

2018 Equity Incentive Plan

On August 21, 2018, our Board of Directors approved the Inhibikase Therapeutics, Inc. 2018 Equity Incentive Plan, or the 2018 Plan, which became effective immediately prior to the closing of the Company's initial public offering described herein. The principal provisions of the 2018 Plan are summarized below.

Administration

The 2018 Plan vests broad powers in a committee to administer and interpret the 2018 Plan. Our Board of Directors will designate the compensation committee to administer the 2018 Plan. Except when limited by the terms of the 2018 Plan, the compensation committee has the authority to, among other things: select the persons to be granted awards; determine the type, size and term of awards; establish performance objectives and conditions for earning awards; determine whether such performance objectives and conditions have been met; and accelerate the vesting or exercisability of an award. In its discretion, the compensation committee may delegate all or part of its authority and duties with respect to granting awards to one or more of our officers, subject to certain limitations and provided applicable law so permits.

Our Board of Directors may amend, alter or discontinue the 2018 Plan and the compensation committee may amend any outstanding award at any time; provided, however, that no such amendment or termination may adversely affect awards then outstanding without the holder's permission. In addition, any amendments seeking to increase the total number of shares reserved for issuance under the 2018 Plan or modifying the classes of participants eligible to receive awards under the 2018 Plan will require ratification

by our stockholders in accordance with applicable law. Additionally, as described more fully below, neither the compensation committee nor the Board of Directors is permitted to reprice outstanding options or stock appreciation rights without shareholder consent.

Eligibility

Any of our employees, directors, consultants, and other service providers, or those of our affiliates, are eligible to participate in the 2018 Plan and may be selected by the compensation committee to receive an award. As of August 17, 2018, the Company had two employees, five directors (including one employee director), and five contractors.

Vesting

The compensation committee determines the vesting conditions for awards. These conditions may include the continued employment or service of the participant, the attainment of specific individual or corporate performance goals, or other factors as determined in the compensation committee's discretion (collectively, "Vesting Conditions").

Shares of Stock Available for Issuance

Subject to certain adjustments, the maximum number of shares of common stock that may be issued under the 2018 Plan in connection with awards is 8,770,834, plus any shares that are available or become available under the 2011 Plan. All of these shares may be utilized toward the grant of any type of award, including incentive stock options. The 2018 Plan imposes a $250,000 limitation on the total grant date fair value of awards granted to any non-employee director in any single calendar year.

In the event of any merger, consolidation, reorganization, recapitalization, stock split, reverse stock split, split up, spin-off, combination of shares, exchange of shares, stock dividend, dividend in kind, or other like change in capital structure (other than ordinary cash dividends) to stockholders of the company, or other similar corporate event or transaction that affects our common stock, the compensation committee shall make appropriate adjustments in the number and kind of shares authorized by the 2018 Plan and covered under outstanding awards as it determines appropriate and equitable.

Shares subject to awards that expire without being fully exercised or that are otherwise forfeited, cancelled or terminated may again be made available for issuance under the 2018 Plan. However, shares withheld in settlement of a tax withholding obligation, or in satisfaction of the exercise price payable upon exercise of an option, will not again become available for issuance under the 2018 Plan.

We expect the initial public offering price for our common stock will be between $[•] and $[•] per share.

Types of Awards

The following types of awards may be granted to participants under the 2018 Plan: (i) incentive stock options, or ISOs; (ii) nonqualified stock options, or NQOs and together with ISOs, options, (iii) stock appreciation rights, (iv) restricted stock, or (v) restricted stock units.

Stock Options. An option entitles the holder to purchase from us a stated number of shares of common stock. An ISO, may only be granted to an employee of ours or our eligible affiliates. The compensation committee will specify the number of shares of common stock subject to each option and the exercise price for such option, provided that the exercise price may not be less than the fair market value of a share of common stock on the date the option is granted. Notwithstanding the foregoing, if ISOs are granted to any 10% stockholder, the exercise price shall not be less than 110% of the fair market value of common stock on the date the option is granted.

Generally, options may be exercised in whole or in part through a cash payment. The compensation committee may, in its sole discretion, permit payment of the exercise price of an option in the form of previously acquired shares based on the fair market value of the shares on the date the option is exercised or through means of "net settlement," which involves the cancellation of a portion of the option to cover the cost of exercising the balance of the option.

All options shall be exercisable in accordance with the terms of the applicable award agreement. The maximum term of an option shall be determined by the compensation committee on the date of grant but shall not exceed 10 years (5 years in the case of ISOs granted to any 10% stockholder). In the case of ISOs, the aggregate fair market value (determined as of the date of grant) of common stock with respect to which such ISOs become exercisable for the first time during any calendar year cannot exceed $100,000. ISOs granted in excess of this limitation will be treated as non-qualified stock options.

Stock Appreciation Rights. A stock appreciation right represents the right to receive, upon exercise, any appreciation in a share of common stock over a particular time period. The base price of a stock appreciation right shall not be less than the fair market value of a share of common stock on the date the stock appreciation right is granted. This award is intended to mirror the benefit the participant would have received if the compensation committee had granted the participant an option. The maximum term of a stock appreciation right shall be determined by the compensation committee on the date of grant but shall not exceed 10 years. Distributions with respect to stock appreciation rights may be made in cash, shares of common stock, or a combination of both, at the compensation committee's discretion.

Unless otherwise provided in an award agreement or determined by the compensation committee, if a participant terminates employment with us (or our affiliates) due to death or disability, the participant's unexercised options and stock appreciation rights may be exercised, to the extent they were exercisable on the termination date, for a period of twelve months from the termination date or until the expiration of the original award term, whichever period is shorter. If the participant terminates employment with us (or our affiliates) for cause, (i) all unexercised options and stock appreciation rights (whether vested or unvested) shall terminate and be forfeited on the termination date, and (ii) any shares in respect of exercised options or stock appreciation rights for which we have not yet delivered share certificates will be forfeited and we will refund to the participant the option exercise price paid for those shares, if any. If the participant's employment terminates for any other reason, any vested but unexercised options and stock appreciation rights may be exercised by the participant, to the extent exercisable at the time of termination, for a period of ninety days from the termination date (or such time as specified by the compensation committee at or after grant) or until the expiration of the original option or stock appreciation right term, whichever period is shorter. Unless otherwise provided by the compensation committee, any options and stock appreciation rights that are not exercisable at the time of termination of employment shall terminate and be forfeited on the termination date.

Restricted Stock. A restricted stock award is a grant of shares of common stock, which are subject to forfeiture restrictions during a restriction period. The compensation committee will determine the price, if any, to be paid by the participant for each share of common stock subject to a restricted stock award. The restricted stock may be subject to Vesting Conditions. If the specified Vesting Conditions are not attained, the participant will forfeit the portion of the restricted stock award with respect to which those conditions are not attained, and the underlying common stock will be forfeited to us. At the end of the restriction period, if the Vesting Conditions have been satisfied, the restrictions imposed will lapse with respect to the applicable number of shares. During the restriction period, a participant will have the right to vote the shares underlying the restricted stock. However, unless otherwise provided by the applicable award agreement or the compensation committee, a participant generally will not have the right to receive any cash distributions or dividends with respect to the restricted stock prior to the lapse of the restriction period. Unless otherwise provided in an award agreement or determined by the compensation committee, upon termination a participant will forfeit all restricted stock that then remains subject to forfeiture restrictions.

Restricted Stock Units. Restricted stock units are granted in reference to a specified number of shares of common stock and entitle the holder to receive, on the achievement of applicable Vesting Conditions, shares of common stock. Unless otherwise provided in an award agreement or determined by the Compensation committee, upon termination a participant will forfeit all restricted stock units that then remain subject to forfeiture.

Change in Control

In the event of a change in control, the compensation committee may, on a participant-by-participant basis: (i) cause any or all outstanding awards to become vested and immediately exercisable (as applicable), in whole or in part; (ii) cause any outstanding option or stock appreciation right to become fully vested and

immediately exercisable for a reasonable period in advance of the change in control and, to the extent not exercised prior to that change in control, cancel that option or stock appreciation right upon closing of the change in control; (iii) cancel any unvested award or unvested portion thereof, with or without consideration; (iv) cancel any award in exchange for a substitute award; (v) redeem any restricted stock or restricted stock unit for cash and/or other substitute consideration with value equal to the fair market value of an unrestricted share on the date of the change in control; (vi) cancel any outstanding option or stock appreciation right with respect to all common stock for which the award remains unexercised in exchange for a cash payment equal to the excess (if any) of the fair market value of the common stock subject to the option or stock appreciation right over the exercise price of the option or stock appreciation right; (vii) take such other action as the compensation committee shall determine to be reasonable under the circumstances; and/or (viii) in the case of any award subject to Section 409A of the Code, such award shall vest and be distributed only in accordance with the terms of the applicable award agreement and the compensation committee shall only be permitted to use discretion to the extent that such discretion would be permitted under Section 409A of the Code.

Repricing

Neither our Board of Directors nor the compensation committee may, without obtaining prior approval of our stockholders: (i) implement any cancellation/re-grant program pursuant to which outstanding options or stock appreciation rights under the 2018 Plan are cancelled and new options or stock appreciation rights are granted in replacement with a lower exercise per share; (ii) cancel outstanding options or stock appreciation rights under the 2018 Plan with an exercise price per share in excess of the then current fair market value per share for consideration payable in our equity securities; or (iii) otherwise directly reduce the exercise price in effect for outstanding options or stock appreciation rights under the 2018 Plan.

Federal Tax Consequences

Under the Code as currently in effect, a grant under the 2018 Plan of options, stock appreciation rights, restricted stock or restricted stock units would have no federal income tax consequence at the time of grant. All amounts taxable as ordinary income to participants under the 2018 Plan in respect of awards are expected to be deductible by the Company as compensation at the same time the participant recognizes the ordinary income, subject to the limitations of Section 162(m) of the Code.

Options and Stock Appreciation Rights. Upon exercise of a nonqualified stock option, the excess of the fair market value of the stock at the date of exercise over the exercise price is taxable to a participant as ordinary income. Similarly, upon exercise of a Stock Appreciation Right, the value of the shares or cash received is taxable to the participant as ordinary income. Upon exercise of an ISO, the participant will not have taxable income, except that alternative minimum tax may apply. When there is a disposition of the shares subject to the ISO, the difference, if any, between the sale price of the shares and the exercise price of the option, is treated as long-term capital gain or loss if the participant has held for at least two years after the date of grant and at least one year after the date of exercise. If the participant does not satisfy these holding period requirements, a “disqualifying disposition” occurs and the participant will recognize ordinary income in the year of the disposition in an amount equal to the excess of the fair market value of the shares at the time the option was exercised over the exercise price of the option. Any gain realized in excess of the fair market value at the time of exercise will be short or long-term capital gain, depending on whether the shares were sold more than one year after the option was exercised.

Restricted Stock. Unless the participant elects to recognize its value as income at the time of the grant, restricted stock is taxable to a participant as ordinary income when it becomes vested.

Restricted Stock Units. When shares of common stock with respect to restricted stock unit awards are delivered to the participant, the value of the shares is taxable to the participant as ordinary income.

Miscellaneous

Generally, awards granted under the 2018 Plan shall be nontransferable except by will or by the laws of descent and distribution. No participant shall have any rights as a stockholder with respect to shares covered by options or restricted stock units, unless and until such awards are settled in shares of common

stock. No option shall be exercisable, no shares of common stock shall be issued, no certificates for shares of common stock shall be delivered and no payment shall be made under the 2018 Plan except in compliance with all applicable laws. The awards will be subject to our recoupment and stock ownership policies, as may be in effect from time to time. The 2018 Plan will expire ten years after it becomes effective.

Equity Compensation Plan Information

The table below sets forth information with respect to compensation plans under which equity securities of the Company are authorized for issuance as of December 31, 2017:

Plan Category	Number of Securities to be issued upon exercise of outstanding options, warrants and rights (a)	Weighted-average exercise price of outstanding options, warrants and rights (b)	Securities available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity Compensation Plans approved by stockholders	3,204,166	$ 0.85	145,834
Equity Compensation Plans not approved by stockholders			

We maintain the 2011 Plan, a stock option plan, which was initially approved by our stockholders on May 27, 2011. Employees, officers, directors, consultants and advisors are eligible to participate in the 2011 Plan. As of December 31, 2017, there were 145,834 shares reserved for issuance under the 2011 Plan that remain available. We will cease making new grants under the 2011 Plan, effective upon the completion of this offering. However, options that were previously granted under the 2011 Plan will remain subject to the terms and conditions contained in that plan.

New Plan Benefits

The benefits that will be awarded or paid under the 2018 plan are in the discretion of the Compensation Committee, and are not determinable at this time. Additionally, no options or other awards have previously been granted under the 2018 Plan.

2011 Equity Incentive Plan

Prior to the completion of this offering, we maintained the 2011 Plan, pursuant to which we made grants of non-qualified stock options to eligible employees and other service providers. Subject to specific contractual entitlements of the grantees, generally, options granted under the 2011 Plan had a term of ten years or less, vested monthly over a 12 month period and remained exercisable for 30 days after the date of grantee's cessation of service with the Company, and three months upon disability or death. Options could be exercised upon the delivery of written notice to the Company by the optionee, along with payment in cash or check, or such other method as the committee administering the 2011 Plan allowed in its discretion. Under the 2011 Plan, there was no automatic acceleration of vesting of the options on a change in control, but the committee had the discretion to, among other things, accelerate the vesting of outstanding options, provide that unexercised options would expire on the change in control, require the acquiror to grant replacement awards in lieu of the existing options, or terminate the options in exchange for a cash payment.

Dr. Werner currently has 175,000 options outstanding under the 2011 Plan. In addition, as described under the Equity Compensation Plan Information table above, there are 3,204,166 shares that remain outstanding under the 2011 Plan. Effective upon the completion of this offering, we will cease making new grants under the 2011 Plan, and will make future grants under the 2018 Plan.

Other Benefits

Our named executive officers who are full time employees are eligible to participate in our employee benefit plans, including our health and welfare plans, which are currently fully paid by us. Such benefits will be paid by the Company at 80% as of the completion of this offering, with the remainder to be paid by the eligible employee.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

In addition to the compensation arrangements, including employment, termination of employment and change in control arrangements, with our directors and executive officers, including those discussed in the sections titled "Management" and "Executive Compensation," and the registration rights described in the section titled "Description of Capital Stock — Registration Rights," the following is a description of each transaction since January 1, 2015 or any currently proposed transaction in which:

- we have been or are to be a party to;
- the amount involved exceeded or exceeds $120,000 or 1% of the average of our total assets as of the end of the last two completed fiscal years; and
- any of our directors, executive officers or holders of more than 5% of our outstanding capital stock, or any immediate family member of, or person sharing the household with, any of these individuals or entities, had or will have a direct or indirect material interest.

For information on our compensation arrangements, including employment, termination of employment and change in control arrangements, with our directors and executive officers, see the sections titled "Management" and "Executive Compensation," and the registration rights described in the section titled "Description of Capital Stock — Registration Rights."

Convertible Promissory Notes

In May 2017, we issued a convertible promissory note for an aggregate principal amount of $100,000 to Dr. Mueller, a member of our board of directors. In May 2018, the unpaid principal amount and accrued interest of $112,920 was converted into 26,950 shares of our common stock.

In September 2017, we issued a convertible promissory note for an aggregate principal amount of $50,000 to Mr. Fante, a member of our board of directors. In June 2018, the unpaid principal amount and accrued interest of $54,797 was converted into 13,078 shares of our common stock.

Consulting Agreements

In April 2018, we entered into a consulting agreement with Flagship Consulting, Inc., pursuant to which Mr. Frattaroli performs his duties as Chief Financial Officer. We pay Flagship Consulting, Inc. $12,500 per month, with an additional $12,500 per month accruing on a convertible revolving demand promissory note. As of August 17, 2018, the aggregate outstanding principal amount is $62,500 with accrued interest of $435, which Mr. Frattaroli can opt to convert into shares of our common stock at 80% of the then-fair market value per share.

In July 2015, we entered into a consulting agreement with Mr. Kaul, our Chief Medical Officer, pursuant to which Mr. Kaul performs services as the medical director and development of clinical plans and FDA engagements for the Company. Mr. Kaul is compensated at $13,000 per month.

Indemnification Agreements

We have entered, and intend to continue to enter, into separate indemnification agreements with each of our directors and executive officers, in addition to the indemnification that will be provided for in our amended and restated certificate of incorporation and amended and restated bylaws. The indemnification agreements and our amended restated certificate of incorporation and amended and restated bylaws that will be in effect upon the completion of this offering require us to indemnify our directors, executive officers and certain controlling persons to the fullest extent permitted by Delaware law. See the section titled "Executive Compensation — Limitation of Liability and Indemnification" for additional information.

Stockholder Loans

In November 2010, we advanced to Dr. Werner a total of $103,950 for the purpose of enabling Dr. Werner to repay loan proceeds personally borrowed by him to cover certain of our initial start-up expenses. $69,388 of this amount was characterized as a loan to Dr. Werner with the remainder characterized as a return of capital. The loan was subsequently modified in November 2013 to provide that the unpaid principal amount of the loan bears interest at an annual rate of 1.92% compounded semi-annually. Interest on the loan is capitalized to principal and payable at the maturity date. The maturity date occurs on the earlier of (i) ten years from the loan issuance date or (ii) separation of Dr. Werner from the Company. The loan amount includes $6,526 paid by the Company in April 2011 on behalf of Dr. Werner for his personal tax return, which was characterized as a loan to Dr. Werner. As of March 31, 2018, the total outstanding amount of the loan was $87,478.

We anticipate that such amounts will be repaid in full prior to the closing of this offering, potentially through a cash bonus to be paid to Dr. Werner upon approval by our board of directors.

PRINCIPAL STOCKHOLDERS

The following table sets forth the beneficial ownership of our common stock as of August 17, 2018 by:

- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock;
- each of the named executive officers;
- each of our directors; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC, and thus it represents sole or shared voting or investment power with respect to our securities. Unless otherwise indicated below, to our knowledge, the persons and entities named in the table have sole voting and sole investment power with respect to all shares that they beneficially owned, subject to community property laws where applicable. The information does not necessarily indicate beneficial ownership for any other purpose, including for purposes of Sections 13(d) and 13(g) of the Exchange Act.

We have based our calculation of the percentage of beneficial ownership prior to this offering on 9,205,455 shares of our common stock outstanding as of August 17, 2018, plus 18,774 shares of our common stock issuable pursuant to the conversion of an outstanding convertible note in an aggregate principal amount of $62,500 and accrued interest of approximately $435 into our common stock immediately prior to the completion of this offering, as if such conversion had occurred as of August 17, 2018. We have based our calculation of the percentage of beneficial ownership after this offering on [•] shares of our common stock outstanding immediately after the completion of this offering, assuming no exercise by the underwriters of their option to purchase additional shares. We have deemed shares of our common stock subject to stock options that are currently exercisable or exercisable within 60 days of August 17, 2018, to be outstanding and to be beneficially owned by the person holding the stock option for the purpose of computing the percentage ownership of that person. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the address of each beneficial owner listed in the table below is c/o Inhibikase Therapeutics, Inc., 3350 Riverwood Parkway SE, Suite 1900, Atlanta, GA 30339.

	Shares Beneficially Owned Prior to this Offering		Shares Beneficially Owned After this Offering	
Name of Beneficial Owner	**Shares**	**Percentage**	**Shares**	**Percentage**
Named Executive Officers and Directors				
Milton H. Werner, Ph.D.[1]	6,172,917	65.8%		%
Inder Kaul, M.D., M.P.H.	—	—		%
Joseph Frattaroli, CPA[2]	18,645	*		%
Lisa Evrén[3]	172,917	1.8%		%
Peter Mueller, Ph.D.[4]	199,867	2.1%		%
Richard Fante[5]	100,578	1.1%		
Hillary Malone[6]	72,917	*		%
All executive officers and directors as a group (7 persons)	6,719,194	71.8%		
5% Stockholders				%
Duke University	700,000	7.6%		
Emory University	950,000	10.3%		
Daniel Kalman, Ph.D.[7]	2,000,000	17.8%		

* Represents beneficial ownership of less than one percent.

(1) Consists of (a) 6,000,000 shares held of record by Milton H. Werner, Ph.D. and (b) 172,917 shares subject to options exercisable within 60 days of August 17, 2018.

(2) Consists of 18,645 shares of issuable to Flagship Consulting, Inc. upon conversion of the Convertible Revolving Demand Promissory Note held by Flagship Consulting, Inc. as of August 17, 2018. Flagship Consulting, Inc. is controlled by Joseph Frattaroli and, as such, may be deemed to indirectly beneficially own the shares beneficially owned by Flagship Consulting, Inc.

(3) Consists of 172,917 shares subject to options exercisable within 60 days of August 17, 2018.

(4) Consists of (a) 26,950 shares held of record by Peter Mueller 2006 Revocable Trust, for which Dr. Mueller serves as a trustee and (b) 172,917 shares subject to options exercisable within 60 days of August 17, 2018.

(5) Consists of (a) 13,078 shares held of record by Richard Fante and (b) 87,500 shares subject to options exercisable within 60 days of August 17, 2018.

(6) Consists of 72,917 shares subject to options exercisable within 60 days of August 17, 2018.

(7) Consists of 2,000,000 shares subject to options exercisable within 60 days of August 17, 2018.

DESCRIPTION OF CAPITAL STOCK

The following descriptions of our capital stock and certain provisions of our amended and restated certificate of incorporation and amended and restated bylaws are summaries and are qualified by reference to the amended and restated certificate of incorporation and the amended and restated bylaws that will be in effect upon completion of this offering. Copies of these documents will be filed with the SEC as exhibits to our registration statement, of which this prospectus forms a part. The descriptions of the common stock and preferred stock reflect changes to our capital structure that will occur upon the completion of this offering.

Upon the completion of this offering and the filing of our amended and restated certificate of incorporation to be effective upon completion of this offering, our authorized capital stock will consist of 110,000,000 shares of capital stock, par value $0.001 per share, of which:

- 100,000,000 shares are designated as common stock; and
- 10,000,000 shares are designated as preferred stock.

As of August 17, 2018, there are 9,205,455 shares of our common stock outstanding held by 17 stockholders of record.

Authorized Capitalization

Common Stock

Voting Rights

Each holder of common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Our amended and restated certificate of incorporation and amended and restated bylaws to be in effect upon the completion of this offering do not provide for cumulative voting rights. Because of this, the holders of a plurality of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose. With respect to matters other than the election of directors, at any meeting of the stockholders at which a quorum is present or represented, the affirmative vote of a majority of the voting power of the shares present in person or represented by proxy at such meeting and entitled to vote on the subject matter shall be the act of the stockholders, except as otherwise required by law. The holders of a majority of the stock issued and outstanding and entitled to vote, present in person or represented by proxy, shall constitute a quorum for the transaction of business at all meetings of the stockholders.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of our common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and Preferences

Holders of our common stock have no preemptive, conversion, subscription or other rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate in the future.

Fully Paid and Nonassessable

All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering, upon payment and delivery in accordance with the underwriting agreement, will be fully paid and nonassessable.

Preferred Stock

Upon the closing of this offering, our board of directors will have the authority, without further action by the stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, redemption rights, liquidation preferences, sinking fund terms and the number of shares constituting any series or the designation of such series, any or all of which may be greater than the rights of common stock. Upon closing of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Options

As of March 31, 2018, we had outstanding options to purchase an aggregate of 3,204,166 shares of our common stock, with a weighted-average exercise price of approximately $0.85 per share, under our 2011 Plan. Subsequent to March 31, 2018, we have not issued any additional options to purchase our common stock.

Registration Rights

After the completion of this offering, under our founder's registration rights agreement, as amended, Dr. Werner, the holder of 6,000,000 shares of our common stock, or his transferees, has the right to require us to register the offer and sale of his shares, or to include his shares in any registration statement we file, in each case as described below.

Demand Registration Rights

After the completion of this offering, Dr. Werner will be entitled to certain demand registration rights. At any time following the first anniversary of the closing of this offering, Dr. Werner can request that we file a registration statement to register the offer and sale of his shares on either Form S-1, or any similar long-form registration ("Long-Form Registration"), or on Form S-3, or any similar short-form registration ("Short-Form Registration"). We are obligated to file two Long-Form Registrations and an unlimited number of Short-Form Registrations. These demand registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under certain circumstances. If we determine that it would be seriously detrimental to us and our stockholders to effect such a demand registration, we have the right to defer such registration, not more than twice in any 12-month period, for a period of up to 60 days.

Piggyback Registration Rights

After the completion of this offering, Dr. Werner will be entitled to certain "piggyback" registration rights. If we propose to register the offer and sale of shares of our common stock under the Securities Act, Dr. Werner can request that we include his shares in such registration, subject to certain marketing and other limitations, including the right of the underwriters to limit the number of shares included in any such registration statement under certain circumstances. As a result, whenever we propose to file a registration statement under the Securities Act, other than with respect to (1) a registration related to any employee benefit plan or a corporate reorganization or other transaction covered by Rule 145 promulgated under the Securities Act, (2) a registration in which the only stock being registered is common stock issuable upon conversion of debt securities also being registered or (3) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of our common stock, Dr. Werner is entitled to notice of the registration and has the right, subject to certain limitations, to include his shares in the registration.

Rights of Certain Stockholders

In June 2018, we entered into a side letter agreement with an investor in connection with the purchase of 81,145 shares of our common stock. In the event that we issue and sell shares of our common stock or securities convertible into shares of our common stock in a transaction intended to be exempt from registration under the Securities Act, for cash at a price per share less than that paid by such investor, the agreement provides such investor the right to participate in such transaction. The right of participation will terminate upon the closing of this offering.

In the event that we issue shares of our common stock or securities convertible into shares of our common stock to investors who are not our affiliates pursuant to an effective registration statement at a price per share of less than $4.19, the agreement provides that such investor has the right to receive warrants to purchase shares of our common stock in an amount equal to the aggregate purchase price paid by the investor divided by the lowest price paid by such other investors, with an exercise price of 80% of such lowest price paid by such other investors. In the event that this offering closes after March 31, 2019, such investor has the right to receive warrants to purchase 35,800 shares of our common stock, with an exercise price equal to the initial public offering price. The right to receive warrants in either case will terminate upon the closing of this offering.

Anti-Takeover Effects of Certain Provisions of Delaware Law, Our Amended and Restated Certificate of Incorporation and Our Amended and Restated Bylaws

Certain provisions of Delaware law and certain provisions that will be included in our amended and restated certificate of incorporation and amended and restated bylaws summarized below may be deemed to have an anti-takeover effect and may delay, deter, or prevent a tender offer or takeover attempt that a stockholder might consider to be in its best interests, including attempts that might result in a premium being paid over the market price for the shares held by stockholders.

Preferred Stock

Our amended and restated certificate of incorporation will contain provisions that permit our board of directors to issue, without any further vote or action by the stockholders, shares of preferred stock in one or more series and, with respect to each such series, to fix the number of shares constituting the series and the designation of the series, the voting rights (if any) of the shares of the series, and the powers, preferences or relative, participation, optional and other special rights, if any, and any qualifications, limitations or restrictions, of the shares of such series. The issuance of preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing change in our control or other corporate action.

Classified board of directors

Our amended and restated certificate of incorporation will provide that our board of directors is divided into three classes, designated Class I, Class II, and Class III. Each class will be an equal number of directors, as nearly as possible, consisting of one-third of the total number of directors constituting our entire board of directors. The term of initial Class I directors shall terminate on the date of the 2019 annual meeting, the term of the initial Class II directors shall terminate on the date of the 2020 annual meeting, and the term of the initial Class III directors shall terminate on the date of the 2021 annual meeting. At each annual meeting of stockholders beginning in 2019, successors to the class of directors whose term expires at that annual meeting will be elected for a three-year term.

Removal of Directors

Our amended and restated certificate of incorporation will provide that stockholders may only remove a director for cause by a vote of no less than a majority of the shares present in person or by proxy at the meeting and entitled to vote.

Director Vacancies

Our amended and restated certificate of incorporation will authorize only our board of directors to fill vacant directorships.

No Cumulative Voting

Our amended and restated certificate of incorporation will provide that stockholders do not have the right to cumulate votes in the election of directors.

Special Meetings of Stockholders

Our amended and restated certificate of incorporation and amended and restated bylaws will provide that, except as otherwise required by law, special meetings of the stockholders may be called only by an officer at the request of a majority of our board of directors, by the chairperson or president of our board of directors, or by our Chief Executive Officer.

Advance Notice Procedures for Director Nominations

Our bylaws will provide that stockholders seeking to nominate candidates for election as directors at an annual or special meeting of stockholders must provide timely notice thereof in writing. To be timely, a stockholder's notice generally will have to be delivered to and received at our principal executive offices before notice of the meeting is issued by the secretary of the company, with such notice being served not less than 90 nor more than 120 days before the meeting. Although the amended and restated bylaws will not give our board of directors the power to approve or disapprove stockholder nominations of candidates to be elected at an annual meeting, the amended and restated bylaws may have the effect of precluding the conduct of certain business at a meeting if the proper procedures are not followed or may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect its own slate of directors or otherwise attempting to obtain control of the company.

Action by Written Consent

Our amended and restated certificate of incorporation and amended and restated bylaws will provide that any action to be taken by the stockholders must be effected at a duly called annual or special meeting of stockholders and may not be effected by written consent.

Amending our Certificate of Incorporation and Bylaws

Our amended and restated certificate of incorporation may be amended or altered in any manner provided by the DGCL. Certain provisions of our amended and restated certificate of incorporation may only be amended or altered in any manner by the affirmative vote of 66⅔% of the then-outstanding common stock. Our amended and restated bylaws may be not be amended by stockholders. Additionally, our amended and restated certificate of incorporation will provide that our bylaws may be amended, altered, or repealed by our board of directors.

Authorized but Unissued Shares

Our authorized but unissued shares of common stock and preferred stock will be available for future issuances without stockholder approval, except as required by the listing standards of NASDAQ, and could be utilized for a variety of corporate purposes, including future offerings to raise additional capital, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could render more difficult or discourage an attempt to obtain control of the company by means of a proxy contest, tender offer, merger or otherwise.

Exclusive Jurisdiction

Our amended and restated bylaws will provide that, unless we consent to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of fiduciary duty, any action asserting a claim arising pursuant to the DGCL, any action regarding our amended and restated certificate of incorporation or our amended and restated bylaws or any action asserting a claim against us that is governed by the internal affairs doctrine. Our amended and restated certificate of incorporation further provides that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act.

Business Combinations with Interested Stockholders

Subject to certain exceptions, Section 203 of the DGCL prohibits a public Delaware corporation from engaging in a business combination (as defined in such section) with an "interested stockholder" (defined generally as any person who beneficially owns 15% or more of the outstanding voting stock of such corporation or any person affiliated with such person) for a period of three years following the time that such stockholder became an interested stockholder, unless (i) prior to such time the board of directors of such corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder; (ii) upon consummation of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of such corporation at the time the transaction commenced (excluding for purposes of determining the voting stock of such corporation outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (A) by persons who are directors and also officers of such corporation and (B) by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer); or (iii) at or subsequent to such time the business combination is approved by the board of directors of such corporation and authorized at a meeting of stockholders (and not by written consent) by the affirmative vote of at least 66⅔% of the outstanding voting stock of such corporation not owned by the interested stockholder.

Our amended and restated certificate of incorporation and our amended and restated bylaws will provide that we must indemnify our directors and officers to the fullest extent authorized by the DGCL. We are expressly authorized to carry, and we intend to carry, directors' and officers' insurance providing coverage for our directors, officers and certain employees for some liabilities. We believe that these indemnification provisions and insurance are useful to attract and retain qualified directors and executive directors.

The limitation on liability and indemnification provisions in our certificate of incorporation and bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duty. These provisions may also have the effect of reducing the likelihood of derivative litigation against directors and officers, even though such an action, if successful, might otherwise benefit us and our stockholders. In addition, your investment may be adversely affected to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

Listing

Our common stock has been approved for listing on the NASDAQ Capital Market under the symbol "IKT."

Transfer Agent and Registrar

Upon completion of this offering, the transfer agent and registrar for our common stock will be American Stock Transfer & Trust Company. The transfer agent and registrar's address is 6201 15th Ave, Brooklyn, NY 11219.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock, and we cannot assure investors that there will be an active public market for our common stock following this offering. We cannot predict what effect, if any, sales of our shares in the public market or the availability of shares for sale will have on the market price of our common stock. Future sales of substantial amounts of common stock in the public market, including shares issued upon exercise of outstanding options, or the perception that such sales may occur, however, could adversely affect the market price of our common stock and also could adversely affect our future ability to raise capital through the sale of our common stock or other equity-related securities of ours at times and prices we believe appropriate.

Upon completion of this offering, based on our shares outstanding as of August 17, 2018, [•] shares of our common stock will be outstanding, or [•] shares of common stock if the underwriters exercise their option to purchase additional shares in full. All of the shares of common stock expected to be sold in this offering will be freely tradable without restriction or further registration under the Securities Act unless held by our "affiliates," as that term is defined in Rule 144 under the Securities Act. The remaining outstanding shares of our common stock will be deemed "restricted securities" as that term is defined under Rule 144. Restricted securities may be sold in the public market only if their offer and sale is registered under the Securities Act or if the offer and sale of those securities qualify for an exemption from registration, including exemptions provided by Rules 144 and 701 under the Securities Act, which are summarized below.

As a result of the lock-up agreements described below and the provisions of Rules 144 or 701 and assuming no exercise of the underwriters' option to purchase additional shares, the shares of our common stock that will be deemed "restricted securities" will be available for sale in the public market following the completion of this offering as follows:

- [•] shares will be eligible for sale on the date of this prospectus; and
- [•] shares will be eligible for sale upon expiration of the lock-up agreements beginning more than 180 days after the date of this prospectus.

Lock-Up Agreements

Our officers, directors and the holders of substantially all of our capital stock and options have entered into lock-up agreements with the underwriters under which they have agreed, subject to certain exceptions, not to dispose of or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior consent of *[insert names of lead underwriters*]. See the section titled "Underwriting" for additional information.

Rule 144

Rule 144, as currently in effect, generally provides that, once we have been subject to the public company reporting requirements of Section 13 or Section 15(d) of the Exchange Act for at least 90 days, a stockholder who is not deemed to have been one of our affiliates at any time during the preceding 90 days and who has beneficially owned the shares of our capital stock proposed to be sold for at least six months is entitled to sell such shares in reliance upon Rule 144 without complying with the volume limitation, manner of sale or notice conditions of Rule 144. If such stockholder has beneficially owned the shares of our capital stock proposed to be sold for at least one year, then such person is entitled to sell such shares in reliance upon Rule 144 without complying with any of the conditions of Rule 144.

Rule 144 also provides that a stockholder who is deemed to have been one of our affiliates at any time during the preceding 90 days and who has beneficially owned the shares of our common stock proposed to be sold for at least six months is entitled to sell such shares in reliance upon Rule 144 within any three-month period beginning 90 days after the date of this prospectus a number of shares that does not exceed the greater of the following:

- 1% of the number of shares of our capital stock then outstanding, which will equal [•] shares immediately after the completion of this offering; or

- the average weekly trading volume of our common stock during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Sales of our capital stock made in reliance upon Rule 144 by a stockholder who is deemed to have been one of our affiliates at any time during the preceding 90 days are also subject to the current public information, manner of sale, and notice conditions of Rule 144.

Rule 701

In general, under Rule 701 of the Securities Act, most of our employees, consultants or advisors who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement are eligible to resell those shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the holding period or certain other restrictions contained in Rule 144.

Registration Rights

After the completion of this offering, Dr. Werner, the holder of 6,000,000 shares of our common stock will be entitled to certain rights with respect to the registration of such shares under the Securities Act. The registration of these shares of our common stock under the Securities Act would result in these shares becoming eligible for sale in the public market without restriction under the Securities Act immediately upon the effectiveness of such registration statement, subject to the Rule 144 limitations applicable to affiliates. See the section titled “Description of Capital Stock — Registration Rights” for a description of these registration rights.

Registration Statement for Equity Awards

In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act to register all of the shares of our common stock subject to equity awards outstanding or reserved for issuance under our equity compensation plans. The shares of our common stock covered by such registration statement will be eligible for sale in the public market without restriction under the Securities Act immediately upon the effectiveness of such registration statement, subject to vesting restrictions, the conditions of Rule 144 applicable to affiliates and any applicable lock-up agreements. See the section titled “Executive Compensation — Employee Benefit and Stock Plans” for a description of our equity compensation plans.

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF OUR COMMON STOCK

The following is a summary of the material U.S. federal income tax consequences of the ownership and disposition of our common stock acquired in this offering by a "non-U.S. holder" (as defined below), but does not purport to be a complete analysis of all the potential tax considerations relating thereto. This summary is based upon the provisions of the United States Internal Revenue Code of 1986, as amended, or the Code, Treasury Regulations promulgated thereunder, administrative rulings and judicial decisions, all as of the date hereof. These authorities may be changed, possibly retroactively, so as to result in U.S. federal income tax consequences different from those set forth below. We have not sought, and do not intend to seek, any ruling from the Internal Revenue Service, or IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS or a court will agree with such statements and conclusions.

This summary also does not address the tax considerations arising under the laws of any state or local or non-U.S. jurisdiction or under U.S. federal gift and estate tax rules, or rising out of other non-income tax rules, except to the limited extent set forth below. In addition, this discussion does not address tax considerations applicable to an investor's particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

- banks, insurance companies, regulated investment companies, real estate investment trusts or other financial institutions;
- persons subject to the alternative minimum tax or the tax on net investment income;
- persons subject to special tax accounting rules as a result of any item of gross income with respect to our common stock being taken into account in an applicable financial statement;
- tax-exempt organizations or governmental organizations;
- pension plans and tax-qualified retirement plans;
- controlled foreign corporations, passive foreign investment companies and corporations that accumulate earnings to avoid U.S. federal income tax;
- partnerships or other entities or arrangements treated as partnership for U.S. federal income tax purposes (and investors therein);
- brokers or dealers in securities or currencies;
- traders in securities that elect to use a mark-to-market method of accounting for their securities holdings;
- persons that own, or are deemed to own, more than five percent of our capital stock (except to the extent specifically set forth below);
- certain former citizens or long-term residents of the United States;
- persons who hold our common stock as a position in a hedging transaction, "straddle," "conversion transaction" or other risk reduction transaction or integrated investment;
- persons who hold or receive our common stock pursuant to the exercise of any option or otherwise as compensation;
- persons who do not hold our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, property held for investment); and
- persons deemed to sell our common stock under the constructive sale provisions of the Code.

In addition, if a partnership, entity or arrangement classified as a partnership or flow-through entity for U.S. federal income tax purposes holds our common stock, the tax treatment of a partner generally will depend on the status of the partner and upon the activities of the partnership or other entity. A partner in a partnership or other such entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of the ownership and disposition of our common stock through a partnership or other such entity, as applicable.

This summary is for informational purposes only and is not tax advice. Each non-U.S. holder is urged to consult its own tax advisor with respect to the application of the U.S. federal income tax laws to its particular situation, as well as any tax consequences of the purchase, ownership and disposition of our common stock arising under the U.S. federal gift or estate tax rules or under the laws of any state, local, non-U.S. or other taxing jurisdiction or under any applicable tax treaty.

Non-U.S. Holder Defined

For purposes of this discussion, a "non-U.S. holder" is a beneficial owner of our common stock that, for U.S. federal income tax purposes, is neither a "U.S. person" nor an entity (or arrangement) treated as a partnership. A "U.S. person" is any person that, for U.S. federal income tax purposes, is or is treated as any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation or other entity taxable as a corporation created or organized in the United States or under the laws of the United States or any political subdivision thereof, or otherwise treated as such for U.S. federal income tax purposes;
- an estate whose income is subject to U.S. federal income tax regardless of its source; or
- a trust (x) whose administration is subject to the primary supervision of a U.S. court and that has one or more U.S. persons who have the authority to control all substantial decisions of the trust or (y) that has made a valid election under applicable Treasury Regulations to be treated as a U.S. person.

Distributions

As described in the section titled "Dividend Policy," we have never declared or paid cash dividends on our common stock, and we do not anticipate paying any dividends on our common stock following the completion of this offering. However, if we do make distributions of cash or property on our common stock to non-U.S. holders, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. To the extent those distributions exceed both our current and our accumulated earnings and profits, the excess will first constitute a return of capital and will reduce each non-U.S. holder's adjusted tax basis in our common stock, but not below zero. Any additional excess will then will be treated as capital gain from the sale of stock, as discussed under "Gain on Disposition of common stock."

Subject to the discussions below on effectively connected income, and backup withholding and Foreign Account Tax Compliance Act, or FATCA, withholding, any dividend paid to a non-U.S. holder generally will be subject to U.S. federal withholding tax either at a rate of 30% of the gross amount of the dividend or such lower rate as may be specified by an applicable income tax treaty between the United States and such non-U.S. holder's country of residence. In order to receive a reduced treaty rate, such non-U.S. holder must provide the applicable withholding agent with an IRS Form W-8BEN or W-8BEN-E or other appropriate version of IRS Form W-8 certifying qualification for the reduced treaty rate. A non-U.S. holder of shares of our common stock eligible for a reduced rate of U.S. federal withholding tax pursuant to an income tax treaty may obtain a refund of any excess amounts withheld by filing an appropriate claim for refund with the IRS. If such non-U.S. holder holds our common stock through a financial institution or other agent acting on the non-U.S. holder's behalf, the non-U.S. holder will be required to provide appropriate documentation to such agent, which then will be required to provide certification to the applicable withholding agent, either directly or through other intermediaries. Each non-U.S. holder should consult its own tax advisors regarding their entitlement to benefits under any applicable income tax treaty.

Dividends received by a non-U.S. holder that are treated as effectively connected with such non-U.S. holder's conduct of a trade or business within the United States (and, if an applicable income tax treaty so provides, such non-U.S. holder maintains a permanent establishment or fixed base in the United States to which such dividends are attributable) are generally exempt from the 30% U.S. federal withholding tax, subject to the discussion below on backup withholding and FATCA withholding. To claim this exemption, a non-U.S. holder must provide the applicable withholding agent with a properly executed IRS

Form W-8ECI or other applicable IRS Form W-8 properly certifying such exemption. Such effectively connected dividends, although not subject to U.S. federal withholding tax, are taxed at the same graduated rates applicable to U.S. persons, net of certain deductions and credits, subject to an applicable income tax treaty providing otherwise. In addition, if a non-U.S. holder is a corporation, dividends such non-U.S. holder receives that are effectively connected with its conduct of a U.S. trade or business may also be subject to a branch profits tax at a rate of 30% or such lower rate as may be specified by an applicable income tax treaty between the United States and such non-U.S. holder's country of residence. Each non-U.S. holder should consult its own tax advisor regarding the tax consequences of the ownership and disposition of our common stock, including any applicable tax treaties that may provide for different rules.

Gain on Disposition of Common Stock

Subject to the discussion below regarding backup withholding and FATCA withholding, a non-U.S. holder generally will not be required to pay U.S. federal income tax on any gain realized upon the sale or other disposition of our common stock unless:

- the gain is effectively connected with such non-U.S. holder's conduct of a U.S. trade or business (and, if an applicable income tax treaty so provides, such non-U.S. holder maintains a permanent establishment or fixed base in the United States to which such gain is attributable);
- such non-U.S. holder is an individual who is present in the United States for an aggregate 183 days or more during the taxable year in which the sale or disposition occurs and certain other conditions are met; or
- our common stock constitutes a United States real property interest, or USRPI, by reason of our status as a "United States real property holding corporation," or USRPHC, for U.S. federal income tax purposes.

We believe that we are not currently and will not become a USRPHC for U.S. federal income tax purposes, and the remainder of this discussion so assumes. However, because the determination of whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our U.S. and worldwide real property interests plus our other business assets, there can be no assurance that we will not become a USRPHC in the future. Even if we become a USRPHC, however, as long as our common stock is regularly traded on an established securities market, your common stock will be treated as U.S. real property interests only if you actually (directly or indirectly) or constructively hold more than five percent of such regularly traded common stock at any time during the shorter of the five-year period preceding your disposition of, or your holding period for, our common stock.

A non-U.S. holder described in the first bullet above will be required to pay U.S. federal income tax on the gain derived from the sale (net of certain deductions and credits) under regular graduated U.S. federal income tax rates. Such a non-U.S. holder that is a corporation may be subject to the branch profits tax at a 30% rate on a portion of its effectively connected earnings and profits for the taxable year that are attributable to such gain, as adjusted for certain items. A lower rate may be specified by an applicable income tax treaty.

A non-U.S. holder described in the second bullet above will be subject to tax at 30% (or such lower rate specified by an applicable income tax treaty) on the gain derived from the sale, which gain may be offset by U.S. source capital losses of such non-U.S. holder for the taxable year, provided such non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses.

Each non-U.S. holder should consult its own tax advisor regarding any applicable income tax or other treaties that may provide for different rules.

Information Reporting and Backup Withholding

Generally, we or an applicable withholding agent must report annually to the IRS the amount of dividends paid to a non-U.S. holder, such non-U.S. holder's name and address, and the amount of tax withheld, if any. A similar report is sent to such non-U.S. holder. Pursuant to any applicable income tax treaty or other agreement, the IRS may make such report available to the tax authority in such non-U.S. holder's country of residence.

Dividends paid by us (or our paying agent) to a non-U.S. holder may also be subject to backup withholding at a current rate of 24%.

Such information reporting and backup withholding requirements may be avoided, however, if such non-U.S. holder establishes an exemption by providing a properly executed, and applicable, IRS Form W-8, or otherwise establishes an exemption. Generally, such information reporting and backup withholding requirements will not apply to a non-U.S. holder where the transaction is effected outside the United States, through a non-U.S. office of a non-U.S. broker. Notwithstanding the foregoing, backup withholding and information reporting may apply, however, if the applicable withholding agent has actual knowledge, or reason to know, that such non-U.S. holder is a U.S. person.

Backup withholding is not an additional tax; rather, the U.S. federal income tax liability of persons subject to backup withholding will be reduced by the amount of tax withheld. If withholding results in an overpayment of taxes, a refund or credit may generally be obtained from the IRS, provided that the required information is furnished to the IRS in a timely manner.

Foreign Account Tax Compliance Act (FATCA)

Sections 1471 to 1474 of the Code, Treasury Regulations issued thereunder and related official IRS guidance, commonly referred to as FATCA, generally impose a U.S. federal withholding tax of 30% on dividends on, and the gross proceeds from a sale or other disposition of, our common stock paid to a "foreign financial institution" (as defined under FATCA, and which may include banks, traditional financial institutions, investment funds, and certain holding companies), unless such institution enters into an agreement with the U.S. Department of the Treasury to, among other things, identify accounts held by certain "specified United States persons" or "United States-owned foreign entities" (each as defined under FATCA), report annually substantial information about such accounts, and withhold on certain payments to non-compliant foreign financial institutions and certain other account holders. FATCA also generally imposes a U.S. federal withholding tax of 30% on dividends on, and the gross proceeds from a sale or other disposition of, our common stock paid to a "non-financial foreign entity" (as specially defined under FATCA), unless such entity provides identifying information regarding each direct or indirect "substantial United States owners" (as defined under FATCA), certifies that it does not have any substantial United States owners, or otherwise establishes an exemption. Accordingly, the institution or entity through which our common stock is held will affect the determination of whether such withholding is required.

The withholding obligations under FATCA generally apply to dividends on our common stock and will apply to the payment of gross proceeds of a sale or other disposition of our common stock made on or after January 1, 2019. Such withholding will apply regardless of whether the beneficial owner of the payment otherwise would be exempt from withholding pursuant to an applicable tax treaty with the United States, the Code, or other exemptions described above. Under certain circumstances, a non-U.S. holder might be eligible for refunds or credits of such taxes.

An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this section. Prospective investors are encouraged to consult with their own tax advisors regarding the application of FATCA withholding to their investment in, and ownership and disposition of, our common stock.

The preceding discussion of U.S. federal tax considerations is for general information only. It is not tax advice to investors in their particular circumstances. Each prospective investor should consult its own tax advisor regarding the particular U.S. federal, state and local and non-U.S. tax consequences of purchasing, holding and disposing of our common stock, including the consequences of any proposed change in applicable laws.

UNDERWRITING

[To come.]

LEGAL MATTERS

The validity of the issuance of our common stock offered in this prospectus will be passed upon for us by Pepper Hamilton LLP, New York, New York. [•] is acting as counsel for the underwriters.

EXPERTS

CohnReznick LLP, independent registered public accounting firm, has audited our financial statements at December 31, 2017 and 2016, and for each of the two years in the period ended December 31, 2017, as set forth in their report, which includes an explanatory paragraph relating to our ability to continue as a going concern. We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on CohnReznick LLP's report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of our common stock offered by this prospectus. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement, as permitted by the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement, including the exhibits filed as a part of the registration statement. Statements contained in this prospectus concerning the contents of any contract or any other document are not necessarily complete. If a contract or document has been filed as an exhibit to the registration statement, please see the copy of the contract or document that has been filed. Each statement in this prospectus relating to a contract or document filed as an exhibit is qualified in all respects by the filed exhibit. You may obtain copies of this information by mail from the Public Reference Section of the SEC, 100 F Street, N.E., Room 1580, Washington, D.C. 20549, at prescribed rates or view them online. You may obtain information on the operation of the public reference rooms by calling the SEC at 1-800-SEC-0330. The SEC also maintains an Internet website that contains the registration statement of which this prospectus forms a part, as well as the exhibits thereto. These documents, along with future reports, proxy statements and other information about us, are available at the SEC's website, www.sec.gov.

As a result of this offering, we will become subject to the information and reporting requirements of the Exchange Act, as amended, and, in accordance with this law, will file periodic reports, proxy statements and other information with the SEC. These periodic reports, proxy statements and other information will be available for inspection and copying at the SEC's public reference facilities and the website of the SEC referred to above. We also maintain a website at www.inhibikase.com. Upon the completion of this offering, you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

GLOSSARY

Abelson protein kinase (c-Abl)	a protooncogene that encodes a protein tyrosine kinase involved in a variety of cellular processes, including cell division, adhesion, differentiation, and response to stress
Alpha-synuclein	a protein that is found primarily in neurons and accumulates to form Lewy bodies in people affected with Parkinson's disease and some forms of dementia
AMP	average manufacturer price
ANDA	abbreviated new drug application to the FDA
BBB	blood-brain barrier
Biomarker	a biological molecule found in blood, other bodily fluids or tissues that is a sign of a normal or abnormal process or of a condition or disease
BLA	biologics license application to the FDA
c-Abl1 (c-Abl)	The ABL1 gene provides instructions for making a protein involved in many processes in cells throughout the body. The ABL1 protein functions as a kinase, which is an enzyme that changes the activity of other proteins by adding a cluster of oxygen and phosphorus atoms (a phosphate group) at specific positions. The ABL1 kinase is normally turned off (inactive) and must be turned on (activated) to perform its functions. Abelson murine leukemia viral oncogene homolog 1 also known as ABL1 is a protein that, in humans, is encoded by the ABL1 gene (previous symbol ABL) located on chromosome 9.
c-Abl protein kinase inhibitor	a potential therapeutic treatment in PD and other neurodegenerative disease that could improve motor behavior, prevent the loss of dopamine neurons, inhibit phosphorylation of Cdk5, regulate α-synuclein phosphorylation and clearance, inhibit the tyrosine phosphorylation of parkin and decrease parkin substrate
Central Nervous System (CNS)	the portion of the vertebrate nervous system consisting of the brain and spinal cord.
cGCPs	current good clinical practices promulgated by the FDA
Cmax	measured maximum concentration
CML	Chronic Myelogenous Leukemia
CMO	third party contract manufacturer
CMS	Centers for Medicare & Medicaid Services
CRO	contract research organizations
CTA	clinical trial application to the EMA
Dementia with Lewy Body (DLB)	A type of dementia, whose underlying mechanism involves the buildup of Lewy bodies, clumps of alpha-synuclein protein in neurons
EMA	European Medicines Agency
FDA	U.S. Food and Drug Administration
Imatinib	marketed as Gleevec®, developed to treat chronic myelogenous leukemia (CML).
IMM	irreversible morbidity or mortality
IND	investigational new drug
Investigational New Drug Applications (IND)	a request for Food and Drug Administration (FDA) authorization to administer an investigational drug to humans. Such authorization must be secured prior to interstate shipment and administration of any new drug that is not the subject of an approved new drug application.

Kinase	an enzyme that catalyzes the addition of a phosphate group to substrates, usually proteins
Lewy bodies	clumps of alpha-synuclein protein in neurons
Liquid tumor	Cancers that do not result in the formation of solid tumors, including cancers occurring in the blood, bone marrow, blood cells and lymphatic system
MA	Marketing Authorization
MAA	marketing authorization application
Misfolded and/or aggregated protein	Misfolded protein intermediates form large polymers of unwanted aggregates and are involved in the pathogenesis of many human diseases
Multiple System Atrophy (MSA)	A neurological disorder; This combined parkinsonian and autonomic disorder is referred to as the Shy-Drager syndrome. In addition to orthostatic hypotension, other features of autonomic failure include impotence, loss of sweating, dry mouth and urinary retention and incontinence
NDA	new drug application to the FDA
Neurodegenerative	resulting in or characterized by degeneration of the nervous system, especially the neurons in the brain.
NOAEL	NO Adverse Event Level
Oncology	branch of medicine that deals with tumors, including study of their development, diagnosis, treatment, and prevention.
Orange Book	Approved Drug Products with Therapeutic Equivalence Evaluations
Pathway	a chain of nerve fibers along which impulses normally travel; a sequence of enzymatic or other reactions by which one biological material is converted to another.
Peripheral nervous system	The part of the vertebrate nervous system constituting the nerves outside the central nervous system and including the cranial nerves, spinal nerves, and sympathetic and parasympathetic nervous systems.
PD	Parkinson’s Disease
pharmacokinetics	the activity of drugs in the body over a period of time, including the processes by which drugs are absorbed, distributed in the body, localized in the tissues, and excreted
Prodrug	a compound that, on administration, must undergo chemical conversion by metabolic processes before becoming an active pharmacological agent; a precursor of a drug.
Progressive Multifocal Leukoencephalopathy (PML)	a rapidly progressive neuromuscular disease caused by opportunistic infection of brain cells (oligodendrocytes and astrocytes) by the JC virus (JCV)
RAMP	Re-engineering Approach with Metabolism Preserved
REMS	Risk Evaluation and Mitigation Strategy
Small molecule	a low molecular weight organic compound that may regulate a biological process
Therapeutic target	a protein or nucleic acid whose activity can be modified by an external stimulus
Toxic protein	a protein that forms when the individual proteins malfunction and start to bind together.

INHIBIKASE THERAPEUTICS, INC.
INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Stockholders and board of directors of Inhibikase Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Inhibikase Therapeutics, Inc. (the "Company") as of December 31, 2017 and 2016, and the related statements of operations, stockholders' deficit, and cash flows for the years then ended, and the related notes (collectively referred to as the financial statements). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2016, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Going Concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has suffered recurring losses from operations, has a working capital deficit, and has an accumulated deficit that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. Federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ CohnReznick LLP

We have served as the Company's auditor since 2018.

Boston, Massachusetts

August 30, 2018

Inhibikase Therapeutics, Inc.

Balance Sheets

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash	$ 16,665	$ 12,036
Accounts receivable	180,780	66,878
Prepaid expenses and other current assets	625	1,250
Total current assets	198,070	80,164
Due from shareholder	87,097	82,763
Total assets	$ 285,167	$ 162,927
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 353,521	$ 431,791
Accrued expenses and other current liabilities	907,051	753,904
Deferred revenue	5,641	—
Notes payable	459,962	328,942
Total current liabilities	1,726,175	1,514,637
Commitments and contingencies (see Note 10)		
Stockholders' deficit:		
Common stock, $0.001 par value; 30,000,000 shares authorized; 8,919,665 shares issued and outstanding at December 31, 2017 and December 31, 2016	8,920	8,920
Additional paid-in capital	2,858,366	2,511,589
Accumulated deficit	(4,308,294)	(3,872,219)
Total stockholders' deficit	(1,441,008)	(1,351,710)
Total liabilities and stockholders' deficit	$ 285,167	$ 162,927

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Statements of Operations

	Year ended December 31,	
	2017	**2016**
Revenue:		
Grant revenue	$ 2,059,871	$ 967,386
Consulting revenue	1,066	—
Total revenue	2,060,937	967,386
Costs and expenses:		
Research and development	1,755,692	846,386
Selling, general and administrative	710,375	734,288
Total costs and expenses	2,466,067	1,580,674
Loss from operations	(405,130)	(613,288)
Interest expense, net	(30,945)	(15,449)
Net loss	$ (436,075)	$ (628,737)
Net loss per share – basic and diluted	$ (0.05)	$ (0.07)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	8,919,665	8,919,665

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Statements of Stockholders' Deficit

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount			
Balance at January 1, 2016	8,919,665	$8,920	$2,201,501	$(3,243,482)	$ (1,033,061)
Stock-based compensation expense	—	—	310,088	—	310,088
Net loss	—	—	—	(628,737)	(628,737)
Balance at December 31, 2016	8,919,665	8,920	2,511,589	(3,872,219)	(1,351,710)
Stock-based compensation expense	—	—	300,659	—	300,659
Issuance of warrants	—	—	46,118	—	46,118
Net loss	—	—	—	(436,075)	(436,075)
Balance at December 31, 2017	8,919,665	$8,920	$2,858,366	$(4,308,294)	$ (1,441,008)

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Statements of Cash Flows

	Year ended December 31,	
	2017	**2016**
Operating activities		
Net loss	$(436,075)	$(628,737)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	300,659	310,088
Non-cash interest income from shareholder	(4,334)	(1,140)
Warrant expense	46,118	—
Changes in operating assets and liabilities:		
Accounts receivable	(113,901)	94,450
Prepaid expenses and other assets	625	(300)
Accounts payable	(78,271)	96,854
Accrued expenses and other current liabilities	153,147	99,212
Deferred revenue	5,641	—
Net cash used in operating activities	(126,391)	(29,573)
Financing activities		
Proceeds from notes payable	150,000	—
Repayments of note payable	(18,980)	(23,769)
Net cash provided by (used in) financing activities	131,020	(23,769)
Net increase (decrease) in cash	4,629	(53,342)
Cash at beginning of year	12,036	65,378
Cash at end of year	$ 16,665	$ 12,036
Supplemental disclosures of cash flow information		
Cash paid for interest	$ 10,322	$ 4,596

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Notes to Financial Statements

1. Nature of Business

Inhibikase Therapeutics, Inc. (the "Company"), incorporated on June 3, 2010 as a Delaware corporation with its headquarters in Atlanta, Georgia, is developing therapeutics for neurodegenerative disease inside and outside of the brain. The Company anticipates filing two investigational new drug applications, or INDs for its lead program with the U.S. Food and Drug Administration, or FDA, in the first quarter of 2019.

The Company's lead programs utilize small molecule oral protein kinase inhibitors to treat Parkinson's disease, or PD and its gastrointestinal complications. The Company has shown that its lead clinical candidate, IkT-148009, a potent, brain penetrant c-Abl protein kinase inhibitor, halts and/or reverses neurodegeneration in the brain and gastrointestinal tract in preclinical models that mimic the human disease.

Historically, symptoms of a neurodegenerative disease, like a "plaque" made up of a misfolded and/or aggregated protein(s), have been the development focus. The Company focuses instead on the proteins that become dysfunctional in a disease pathway and seeks to understand what leads to their dysfunction. In PD and other neurodegenerative diseases, the Company's pathway-focused approach has enabled a deeper understanding of the origins of the disease and an understanding of how individual proteins are linked together to define the disease process. Using this strategy, the Company has discovered novel therapeutics for c-Abl.

In addition to programs in neurodegeneration, the Company's platform drug discovery and delivery technologies have identified additional opportunities, including treatment of bacterial or viral infections using a single agent at fixed dose and a potential near-term commercial opportunity in oncology that it believes will complete clinical development in 2019, subject to FDA agreements, followed by completion of the FDA requirements for new drug approval in 2020. IkT-001Pro in oncology will seek to validate the pharmacology advantage of the Company's prodrug delivery technology in a cancer patient population. Following validation of IkT-001Pro in oncology, the Company believes the same pharmacology advantages could be applied to IkT-148009, our lead drug for neurodegenerative disease, to enhance clinical development.

Liquidity and Going Concern

The Company has recognized recurring losses. At December 31, 2017, the Company had a working capital deficit of $1,528,105, an accumulated deficit of $4,308,294, cash of $16,665 and accounts payable and accrued expenses of $1,260,572. As of March 31, 2018, the Company had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. This amount represents additional resources to pay for certain prescribed expenses incurred after March 31, 2018 pursuant to our various notices of award from the National Institute of Health.

The future success of the Company is dependent on its ability to successfully obtain additional working capital, obtain regulatory approval for and successfully launch and commercialize its product candidates and to ultimately attain profitable operations. Historically, the Company has funded its operations primarily through cash received in connections with revenue from its various grants.

The Company is subject to a variety of risks similar to other early stage life science companies including, but not limited to the successful development, regulatory approval and market acceptance of the Company's product candidates, development by its competitors of new technological innovations, protection of proprietary technology, and raising additional working capital. Working capital is defined as current assets less current liabilities. The Company has incurred significant research and development expenses and general and administrative expenses related to its product candidate programs. The Company anticipates costs and expenses to increase in the future as the Company continues to develop its product candidates.

The Company may seek to fund its operations through public equity or private equity or debt financings, as well as other sources. However, the Company may be unable to raise additional working capital, or if it is able to raise additional working capital it may be unable to do so on commercially favorable terms. The Company's failure to raise capital or enter into such other arrangements if and when needed would have a negative impact on the Company's business, results of operations and financial condition and the Company's ability to continue to develop its product candidates.

As of March 31, 2018, the Company has active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. However, as certain elements of the Company's operating plan are outside of the Company's control, including the receipt of anticipated grants and funding from a future capital raise, they cannot be considered probable. If the Company does not receive additional working capital from future anticipated grants and future anticipated capital raises, its existing resources are projected to be sufficient to fund its operations through May 31, 2019.

These conditions raise substantial doubt regarding the Company's ability to continue as a going concern for a period of one year after the date the financial statements are issued. Management's plans to alleviate the conditions that raise substantial doubt include delaying certain research projects and capital expenditures and eliminating certain future operating expenses in order to fund operations at reduced levels for the Company to continue as a going concern for a period of 12 months from the date the financial statements are issued. Management has concluded the likelihood that its plan to obtain sufficient funding from one or more of these sources or adequately reduce expenditures will be successful, while reasonably possible, is less than probable. Accordingly, substantial doubt exists about the Company's ability to continue as a going concern for a period of at least 12 months from the date of issuance of these financial statements.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainties described above.

2. Summary of Significant Accounting Policies

Basis of Presentation

The Company's financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

Use of Estimates

The preparation of the Company's financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. The Company utilizes certain estimates in the determination of the fair value of its stock options and warrants, deferred tax valuation allowances, revenue recognition, to record expenses relating to research and development contracts and accrued expenses. The Company bases its estimates on historical experience and other market-specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results could differ from such estimates.

Off-Balance Sheet Risk and Concentrations of Credit Risk

The Company has no significant off-balance sheet risks, such as foreign exchange contracts, option contracts, or other foreign hedging arrangements. Cash accounts are maintained at financial institutions that potentially subject the Company to concentrations of credit risk. At December 31, 2017 and 2016, substantially all of the Company's cash was deposited in accounts at one financial institution. The Company maintains its cash deposits, which at times may exceed the federally insured limits, with a large financial institution and, accordingly, the Company believes such funds are subject to minimal credit risk.

For the years ended December 31, 2017 and 2016, the Company derived more than 90% of its total revenue from a single source, the United States Government, in the form of Federal research grants.

Accounts Receivable

The Company's accounts receivable consists of amounts due to the Company in connection with its various research grants. At each reporting period, management reviews all outstanding balances to determine if the facts and circumstances of each customer relationship indicate the need for a reserve. The Company does not require collateral and did not have an allowance for doubtful accounts at December 31, 2017 or 2016.

Fair Value Measurements

For certain financial instruments, including accounts receivable and accounts payable, the carrying amounts approximate their fair values as of December 31, 2017 and 2016 because of their short-term nature. At December 31, 2017 and 2016, the carrying value of the Company's debt also approximated fair value.

Revenue Recognition

The Company generates revenue from research and development grants with third parties. Accordingly, the Company recognizes revenue when all of the following criteria have been met:

i. Persuasive evidence of an arrangement exists

ii. Delivery has occurred or services have been rendered

iii. The seller's price to the buyer is fixed or determinable

iv. Collectability is reasonably assured

If any of the above criteria have not been met, the Company defers revenue until such time each of the criteria has been satisfied.

Revenue earned from activities performed pursuant to research and development grants is reported as grant revenue in the statements of operations, using the proportional performance method as the work is completed, limited to payments earned, and the related costs are expensed as incurred as research and development expense. The timing of receipt of cash from the Company's research and development grants generally differs from when revenue is recognized.

Research and Development Costs

Costs incurred in the research and development of the Company's product candidates are expensed as incurred. Research and development expenses consist of costs incurred in performing research and development activities, including activities associated with performing services under grant revenue and include salaries and benefits, stock compensation, research-related subcontractors and consultants, supplies and overhead costs.

Stock-Based Compensation

The Company has a stock-based compensation plan which is more fully described in Note 6. The Company records stock-based compensation for options granted to employees and to members of the board of directors for their services on the board of directors, based on the grant date fair value of awards issued, and the expense is recorded on a straight-line basis over the applicable service period, which is generally one to two years. The Company accounts for non-employee stock-based compensation arrangements based upon the fair value of the consideration received or the equity instruments issued, whichever is more reliably measurable. The measurement date for non-employee awards is generally the date that the performance of services required for the non-employee award is complete. Stock-based compensation costs for non-employee awards is recognized as services are provided, which is generally the vesting period.

The Company uses the Black-Scholes-Merton option pricing model to determine the fair value of stock options. The use of the Black-Scholes-Merton option-pricing model requires management to make assumptions with respect to the expected term of the option, the expected volatility of the common stock consistent with the expected life of the option, risk-free interest rates and expected dividend yields of the common stock. The Company has concluded that its historical share option exercise experience does not provide a reasonable basis upon which to estimate expected term. Therefore, the expected term was determined according to the simplified method, which is the average of the vesting tranche dates and the contractual term. Due to the lack of company specific historical and implied volatility data, we have based our estimate of expected volatility primarily on the historical volatility of a group of similar companies that are publicly traded. For these analyses, companies with comparable characteristics are selected, including enterprise value and position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. The Company computes the historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of its stock-based awards. The risk-free interest rate is determined by reference to U.S. Treasury zero-coupon issues with remaining maturities similar to the expected term of the options. The Company has not paid, and does not anticipate paying, cash dividends on shares of common stock.

Income Taxes

The Company provides for income taxes using the asset and liability method. The Company provides deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the Company's financial statement carrying amounts and the tax basis of assets and liabilities using enacted tax rates expected to be in effect in the years in which the differences are expected to reverse. A valuation allowance is provided to reduce the deferred tax assets to the amount that will more likely than not be realized.

The Company does not have any material uncertain tax positions for which reserves would be required. The Company will recognize interest and penalties related to uncertain tax positions, if any, in income tax expense.

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting the weighted-average number of shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, warrants to purchase common stock and stock options are considered to be common stock equivalents, but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share applicable to common stockholders were the same for all periods presented.

Recent Accounting Standards

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and are generally adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its financial position or results of operations upon adoption.

We qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act. The JOBS Act permits an emerging growth company such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

Accounting Standards Adopted

In March 2016, the FASB released ASU No. 2016-09,*Improvements to Employee Share-Based Payment Accounting* ("ASU 2016-09"), which is intended to simplify income tax accounting for excess tax benefits, accounting for forfeitures, and employer statutory withholding. Under the current guidance, excess tax benefits that result from an award vesting or settling are recognized in additional paid-in capital in the period that they reduce cash taxes payable. This requires the provision to be computed on a with and without option basis and may result in net operating loss and credit carryforwards on the balance sheet being less than what is available on the tax return. Under the new guidance, the income tax effects of awards will be recognized as a component of income tax expense when the awards vest or are settled (regardless if cash taxes are reduced). For interim reporting purposes, companies will account for excess tax benefits and tax deficiencies as discrete items in the period during which they occurred. The guidance is effective for public entities for fiscal years beginning after December 15, 2016 and interim periods within those years, and after December 31, 2017 and interim periods beginning after December 31, 2018 for all other entities. Early adoption is permitted, however all of the guidance included in the update must be applied when adopted. The Company must use a modified retrospective transition method for adopting and record the cumulative effect of all unrecognized benefits and any change in valuation allowances at the end of the prior tax period as an adjustment to retained earnings. The Company's adoption of this standard did not have a material effect on its financial statements.

In March 2016, the FASB issued ASU No. 2016-06,*Derivatives and Hedging (Topic 815): Contingent Put and Call Options in Debt Instruments (*"ASU 2016-06"), which applies to all issuers of or investors in debt instruments with embedded call or put options. ASU 2016-06 clarifies the requirements for assessing whether contingent call or put options that can accelerate the payment of principal on debt instruments are clearly and closely related to their debt hosts. Entities performing the assessment under the guidance of ASU 2016-06 are required to assess the embedded call or put options solely in accordance with the four-step decision process. In addition, ASU 2016-06 clarifies what steps are required when assessing whether the economic characteristics and risks of call or put options are clearly and closely related to the economic characteristics and risks of their debt hosts. ASU 2016-06 is effective for public entity financial statements issued for fiscal years beginning after December 15, 2016 and interim periods within those fiscal years, and after December 31, 2017 and interim periods beginning after December 31, 2018 for all other entities using the modified retrospective method for existing debt instruments. Early adoption is permitted. The Company's adoption of this standard did not have a material effect on its financial statements.

Accounting Standards Issued, Not Yet Adopted

In August 2016, the FASB issued ASU 2016-15,*Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments* ("ASC 2016-15"), which provides guidance on the classification of certain specific cash flow issues including debt prepayment or extinguishment costs, settlement of certain debt instruments, contingent consideration payments made after a business combination, proceeds from the settlement of certain insurance claims and distributions received from equity method investees. The standard requires the use of a retrospective approach to all periods presented, but may be applied prospectively if retrospective application would be impracticable. The guidance is effective for public entities for fiscal years beginning after December 15, 2017 and interim periods within those years, and after December 31, 2018 and interim periods beginning after December 31, 2019 for all other entities. Early adoption is permitted. The impact of its pending adoption of ASU 2016-15 is not expected to be material to the Company's financial statements.

In February 2016, the FASB issued ASU No. 2016-02,*Leases* ("ASU 2016-02"), which applies to all leases. Under ASU 2016-02, a right-of-use asset and lease obligation will be recorded for all leases, whether operating or financing leases, while the statement of operations will reflect lease expense for operating leases and amortization and interest expense for financing leases. ASU 2016-02 is effective for public entities for fiscal years beginning after December 15, 2018 and interim periods within those years, and after December 31, 2019 and interim periods beginning after December 31, 2020 for all other entities. Early adoption is permitted. Entities are required to use a modified retrospective approach of adoption for leases that exist or are entered into after the beginning of the earliest comparative period in the financial statements. Full retrospective application is prohibited. The Company is evaluating the new guidance and the expected effect on the Company's financial statements. The adoption of the new standard is not expected to have a material impact on its financial statements.

In June 2014, the FASB issued amended guidance, ASU No. 2014-09,*Revenue from Contracts with Customers* ("ASU 2014-09"), which is applicable to revenue recognition that will be effective for public entities for fiscal years beginning after December 31, 2017 and interim periods within those years, and after December 31, 2018 and interim periods beginning after December 31, 2019 for all other entities as a result of the deferral of the effective date adopted by the FASB in July 2015. The new guidance must be adopted using either a full retrospective approach for all periods presented or a modified retrospective approach. For public entities, early adoption prior to the original adoption date (annual reporting periods beginning after December 15, 2016) of ASU 2014-09 is not permitted. The new guidance applies a more principles-based approach to revenue recognition. The Company will adopt the new standard, effective January 1, 2019, under the modified retrospective method. The adoption of the new standard is not expected to have a material impact on its financial statements.

3. Supplemental Balance Sheet Information

Accrued expenses and other current liabilities consist of the following:

	December 31, 2017	December 31, 2016
Accrued consulting	$ 13,200	$ 42,921
Accrued legal	350,038	290,984
Accrued professional services	—	127,549
Accrued research and development	326,988	60,178
Accrued interest	118,060	102,235
Accrued other	98,765	130,037
Total accrued expenses and other current liabilities	$ 907,051	$753,904

4. Notes Payable

Future principal payments on the notes payable as of December 31, 2017 are as follows:

Year ended December 31,	
2018	$459,962
2019	—
2020	—
2021	—
2022	—
Total notes payable	$459,962

Subordinated Notes

The Company's subordinated promissory notes issued at face value to ATDC Seed Capital Fund, LLC, a Georgia Research Alliance Funds, Inc. entity (the "GRA"), on August 12, 2010 in the amount of $150,000 and on September 29, 2011 in the amount of $100,000 (collectively, the "GRA Notes") are classified under current liabilities on the balance sheets at December 31, 2017 and 2016, respectively, based on the Company's consideration of the probability of violating covenants included in the GRA Notes. The net proceeds of approximately $243,000 were utilized as working capital by the Company. The total unpaid principal on the GRA Notes included in Notes Payable is $188,734 at December 31, 2017 and $207,713 at December 31, 2016.

The GRA Notes carry a five-year maturity, maturing in 2015 and 2016. They carry a stated interest rate of five percent per annum and contain provisions for contingent accelerated payments of principal and interest in the event of the Company having two consecutive profitable years, or if it closed on a working capital raise of $7 million or greater, or if it entered into a qualified sales transaction. The likelihood of these events was considered remote. The agreements also contain customary affirmative and negative covenants for a credit facility of this size and type. At December 31, 2017 and 2016, the fair value of these

features was not material due to the remote likelihood of the occurrence of the events; therefore, they were not recorded as separate items on the balance sheets.

Upon maturity in 2015 and 2016, the GRA agreed to accept payment of the unpaid principal plus accrued but unpaid interest over a term of 60 months from the dates of maturity at the same interest rate of five percent per annum contained in the GRA Notes. During 2018, the GRA Notes unpaid balance and accrued unpaid interest were converted into shares of stock of the Company at the election of the GRA. Refer to Note 12 Subsequent Events — GRA Notes Conversion.

During 2016, the Company failed to pay certain amounts falling due under the 60-month term agreement. During January 2017, in consideration of GRA's forbearance of a default, the Company issued the GRA a warrant to purchase up to 25,000 shares of the Company's stock. The warrant is exercisable at any time prior to January 2027 at a price of $2.02 per share. The warrant is classified within stockholders' deficit at its fair value and was treated as a standalone instrument. The fair value of the warrant was determined to be $46,118 utilizing the Black-Scholes Merton option-pricing model. Refer to Note 12, Subsequent Events — GRA Notes Conversion.

Convertible Notes

On May 5 and September 8, 2017, the Company issued two convertible promissory notes (the "Notes") in the face amount of $100,000 with Director Mueller and $50,000 with Director Fante, respectively (individually, each "Holder"). Issuance costs were approximately $8,000 and the Company netted approximately $142,000 from the issuance. The net proceeds were used as working capital by the Company. The total unpaid principal on the convertible notes was $150,000 at December 31, 2017.

The Notes bear simple interest at the rate of twelve percent per annum. The unpaid principal balance plus all accrued but unpaid interest thereon (the "Unpaid Balance") shall become due and payable on the first to occur of a) the third anniversary of the issue date, b) on demand by the Holder any time 60 days following the sale of substantially all of the assets of the Company or c) upon the closing by the Company on a private placement financing of preferred stock (the "Preferred Stock") of not less than $5 million in net proceeds of new money, excluding any amounts attributable to the Notes (the "Private Placement").

In the event of the Maturity Date occurring as a result of the Private Placement, the Unpaid Balance may be converted, at the option of the Holder, into the Preferred Stock. The conversion price per share is equal to the price per share of the Preferred Stock but not greater than the price per share based upon a $15 million valuation and the Company capitalization as determined immediately before the Private Placement.

The Company assessed the terms and features of the Notes, including the contingent acceleration of the obligations under the Notes under an event of default and the contingent conversion feature in order to identify any potential embedded features that would require bifurcation. The Company concluded that these features are not clearly and closely related to the host instrument, and represent derivative instruments required to be re-measured at fair value on a quarterly basis. At December 31, 2017 and 2016, the Company determined that the value of these features was not material and, therefore, were not recorded as a separate item on the balance sheets.

During 2018 Directors Mueller and Fante elected to convert the unpaid principal of their respective notes plus accrued and unpaid interest into stock of the Company. Refer to Note 12. Subsequent Events — Notes Conversion — Mueller and Fante.

Revolving Demand Promissory Note

The Company issued a revolving demand promissory note in 2009 in exchange for legal services. The total fair value of the legal services rendered to the Company in exchange for the note was $121,228. The balance of the unpaid principal on the note was $121,228 at December 31, 2017 and 2016. The note originally matured in January 2011. The Company has an unwritten arrangement with the holder to continue to accrue interest at the default interest rate of 5% on the unpaid principal until the Company is able to repay the note in full. The holder is under no obligation to continue this arrangement and may suspend or cancel it at any time at his sole discretion.

5. Stockholders' Deficit

Each share of common stock is entitled to one vote. The holders of common stock are also entitled to receive dividends whenever funds are legally available and when declared by the board of directors, subject to the prior rights of holders of all classes of stock outstanding. As of December 31, 2017, a total of 3,306,274 and 43,726 shares of common stock were reserved for issuance upon (i) the exercise of outstanding stock options and warrants under the 2011 Equity Incentive Plan and (ii) the future issuance of stock awards under the Company's 2011 Equity Incentive Plan, respectively.

6. Stock-Based Compensation

2011 Equity Incentive Plan

The Company's 2011 Equity Incentive Plan (the "2011 Plan") was established for granting stock incentive awards to directors, officers, employees and consultants to the Company.

Stock Options

During the years ended December 31, 2017 and 2016, the Company granted options with an aggregate fair value of $200,699 and $696,901, respectively, which are being amortized into expense over the vesting period of the options as the services are being provided.

The following is a summary of option activity under the Plan:

	Number of Shares	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (In Years)	Aggregate Intrinsic Value
Outstanding at December 31, 2016	3,079,166	$ 0.81	4.75	$ 1,999,497
Granted	125,000	2.02	9.83	200,699
Exercised	—	—	—	—
Forfeited	—	—	—	—
Canceled	—	—	—	—
Outstanding at December 31, 2017	3,204,166	0.85	4.95	2,200,196
Exercisable at December 31, 2017	2,859,583	0.71	4.44	1,636,569
Vested or expected to vest at December 31, 2017	3,204,166	0.85	4.95	2,200,196

There are no options to purchase stock that vest upon the achievement of performance conditions at December 31, 2017.

The weighted-average fair values of options granted in the years ended December 31, 2017 and 2016 were $1.606 and $1.640, per share, respectively, and were calculated using the following estimated assumptions:

	Year ended December 31,	
	2017	2016
Weighted-average risk-free interest rate	2.01%	1.30%
Expected dividend yield	0.00%	0.00%
Expected volatility	105.31%	107.25%
Expected terms	5.5 years	5.5 years

The total fair values of stock options that vested during the years ended December 31, 2017 and 2016 were $300,659 and $310,088, respectively.

As of December 31, 2017, there was $563,627 of total unrecognized compensation cost related to non-vested stock options granted under the Stock Incentive Plan. Total unrecognized compensation cost will be adjusted for future changes in the estimated forfeiture rate. The Company expects to recognize that cost over a remaining weighted-average period of 1.9 years as of December 31, 2017.

Restricted Stock Units

During the years ended December 31, 2017 and 2016 there were no restricted stock units issued or outstanding.

Stock-Based Compensation Expense

The following table summarizes the stock-based compensation expense for stock options granted to employees and non-employees:

	Year ended December 31,	
	2017	2016
Research and development	$125,609	$ 46,539
Selling, general and administrative	175,050	263,549
Total stock-based compensation expense	$300,659	$310,088

7. Warrants

In connection with the GRA Notes, during January 2017, the Company issued to the GRA a warrant to purchase up to 25,000 shares of the Company's stock. The warrant is exercisable at any time prior to January 2027 at an exercise price of $2.02 per share. This warrant was issued in consideration of a 2016 suspension of certain monthly payments of principal and interest under the GRA Notes. Interest continued to accrue on the unpaid balance during this period at the stated rate of five percent. The warrants are classified within stockholders' equity at their fair value and were treated as a standalone instrument. The fair value of the warrant was determined to be $46,118 utilizing the Black-Scholes Merton option-pricing model at the time of issuance.

8. Net Loss Per Share

The following table presents the calculation of basic and diluted net loss per share applicable to common stockholders:

	Year ended December 31,	
	2017	2016
Numerator:		
Net loss	$ (436,075)	$ (628,737)
Denominator:		
Weighted-average number of common shares outstanding – basic and diluted	8,919,665	8,919,665
Net loss per share applicable to common stockholders – basic and diluted	$ (0.05)	$ (0.07)

The following shares were excluded from the calculation of diluted net loss per share applicable to common stockholders, prior to the application of the treasury stock method, because their effect would have been anti-dilutive for the periods presented:

	Year ended December 31,	
	2017	2016
Options to purchase shares of stock	3,204,166	3,079,167
Warrants to purchase shares of stock	102,108	77,108
Total	3,306,274	3,156,275

9. Income Taxes

No provision or benefit for federal or state income taxes has been recorded, as the Company has incurred a net loss for all of the periods presented, and the Company has provided a full valuation allowance against its deferred tax assets.

At December 31, 2017 and 2016, the Company had federal net operating loss carryforwards of approximately $1,572,000 and $1,487,000, respectively, of which federal carryforwards will expire in varying amounts beginning in 2030. At December 31, 2017 and 2016, the Company had state net operating loss carryforwards of approximately $1,591,000 and $1,506,000, respectively. Utilization of net operating losses may be subject to substantial annual limitations due to the "change in ownership" provisions of the Internal Revenue Code, and similar state provisions. The annual limitations may result in the expiration of net operating losses before utilization. The Company has not yet conducted a study to determine if any such changes have occurred that could limit the Company's ability to use the net operating losses and tax credit carryforwards.

The reconciliation of the U.S. federal statutory rate to the Company's effective tax rate is as follows:

	Year Ended December 31,	
	2017	2016
Tax at statutory rates	34.00%	34.00%
State income taxes	4.68%	3.94%
Permanent differences	-0.56%	-0.32%
US tax rate change	-94.97%	0.00%
Other	0.16%	—
Change in valuation allowance	56.69%	-37.62%
Effective tax rate	0.0%	0.0%

The significant components of the Company's deferred tax asset consist of the following at December 31, 2017 and 2016:

	December 31,	
	2017	2016
Deferred tax assets:		
Net operating loss carryforwards	$ 405,882	$ 565,576
Stock-based compensation	462,315	549,844
Total deferred tax assets	868,197	1,115,420
Deferred tax asset valuation allowance	(868,197)	(1,115,420)
Net deferred tax asset	$ —	$ —

The Company has maintained a full valuation allowance against its deferred tax assets in all periods presented. A valuation allowance is required to be recorded when it is not more likely than not that some portion or all of the net deferred tax assets will be realized. Since the Company cannot determine that it is more likely than not that it will generate taxable income, and thereby realize the net deferred tax assets, a full valuation allowance has been provided. The valuation allowance (decreased)/increased ($247,223) and $238,275 for the years ended December 31, 2017 and 2016 respectively. The decrease in the 2017 valuation allowance is primarily attributable to the reduction in the U.S. corporate tax rate enacted in the fourth quarter of 2017. The increase in 2016 is primarily related to each year's taxable loss. The Company has no uncertain tax positions at December 31, 2017 and 2016 that would affect its effective tax rate. Since the Company is in a loss carryforward position, the Company is generally subject to U.S. federal and state income tax examinations by tax authorities for all years for which a loss carryforward is available.

The Tax Cuts and Jobs Act of 2017

On December 22, 2017, the Tax Cuts and Jobs Act ("TCJA") was enacted, leading to significant changes to U.S. tax law. Among other provisions, the TCJA lowered the U.S. federal corporate income tax rate from 35% to 21%, limited the deduction for net operating losses to 80% of taxable income while providing that net operating loss carryovers for years after 2017 will not expire, imposed a mandatory one-time transition tax on previously deferred foreign earnings and eliminated or reduced certain income tax deductions.

10. Commitments and Contingencies

Operating Leases

In 2018 the Company entered into a one year, non-cancelable operating lease for space in Boston, Massachusetts. The total lease obligation is $54,000, payable in 12 equal monthly installments commencing August 1, 2018.

Employment Agreement

On April 1, 2014, the Company entered into a written employment agreement (the "CEO Agreement") with the Company's CEO at an initial base annual salary of $224,000, subject to adjustment by the board of directors. His current base salary is $292,800. The CEO Agreement provided an initial 10-year fully vested option to purchase 50,000 shares of stock of the Company at an exercise price of $0.33 per share. For so long as he remains employed by the Company, the Company agrees to grant an annual option to purchase 25,000 shares of stock of the Company at an exercise price equal to the fair market value of the shares at the date of the grant to be vested pro rata in monthly installments over 12 months from the date of the grant. Bonuses, additional stock option grants or other compensation may be awarded from time to time at the sole discretion of the Company's board of directors. As of December 31, 2017, the CEO has received options to purchase up to 175,000 shares of stock of the Company. The Employment Agreement shall continue until terminated by a) mutual agreement, b) by CEO upon four weeks' written notice to the Company, c) by the Company upon four weeks written notice to CEO, d) by CEO for good reason or e) by the Company for cause. In the event of a termination for good reason or for without cause, CEO is entitled to a severance arrangement to include six months of salary continuation at his base annual salary plus six months accelerated vesting of options and extended time periods to exercise vested stock options. If the termination for good reason or without causes arises in connection with a change in control, the six months of salary continuation is extended to 12 months of salary continuation and all options will become fully vested with extended exercise periods.

The Company has a receivable from the CEO in the amount of $87,097 and $82,763 at December 31, 2017 and 2016, respectively, classified as "Due from shareholder" in the balance sheets. The receivable is accruing interest at the rate of 1.92% per annum until paid. The receivable from the CEO has a maturity date of the earlier to occur of November 2020 or the date on which the CEO experiences a separation from service from the Company.

Consulting Agreement

In July 2015, the Company entered into a consulting agreement with its Chief Medical Officer ("CMO"), pursuant to which the CMO performs services as the medical director and development of clinical plans and FDA engagements for the Company. The CMO is compensated at $13,000 per month under the agreement.

Guarantees

As permitted under Delaware law, the Company indemnifies its officers and directors for certain events or occurrences while the officer or director is, or was, serving at the Company's request in such capacity. The term of the indemnification is for the officer's or director's lifetime. The maximum potential amount of future payments the Company could be required to make is unlimited; however, the Company has directors' and officers' insurance coverage that limits its exposure and enables it to recover a portion of any future amounts paid.

The Company leases office space under a noncancelable operating lease. The Company has standard indemnification arrangements under the lease that requires it to indemnify the landlord against all costs, expenses, fines, suits, claims, demands, liabilities, and actions directly resulting from any breach, violation or nonperformance of any covenant or condition of the Company's lease.

In the ordinary course of business, the Company enters into indemnification agreements with certain suppliers and business partners where the Company has certain indemnification obligations limited to the costs, expenses, fines, suits, claims, demands, liabilities and actions directly resulting from the Company's gross negligence or willful misconduct, and in certain instances, breaches, violations or nonperformance of covenants or conditions under the agreements.

As of December 31, 2017 and 2016, the Company had not experienced any material losses related to these indemnification obligations, and no material claims with respect thereto were outstanding. The Company does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible, and no related reserves were established.

License Agreements

Emory University License Agreements

On June 8, 2010, the Company entered into two license agreements with Emory University, the first for which the Company granted to Emory 450,000 shares of its stock ("License A"), and the second for which the Company granted to Emory 500,000 shares of its stock ("License B"). In exchange, Emory granted the Company and its affiliates an exclusive worldwide sublicenseable right and license to practice under certain patent rights and technology to make, have, develop, promote, market, import, export, distribute, offer for sale, sell and otherwise use the licensed products in the field of use anywhere in the world. Unless sooner terminated as provided elsewhere in the agreement, the License A term is the later of ten years or until the expiration of the patent rights. License B was terminated in May 2013 under the normal course of business. No shares were forfeited or returned and are still owned by Emory.

The Company recorded $313,500 which represented the fair value of the shares issued as part of the total consideration to Emory for the licenses. The fair value of the shares was determined to be more reliably measurable than the fair value of the consideration received.

The Company is required to pay royalties on net sales of products and processes that are covered by the patent rights licensed under the agreement at a percentage in the low single digits, subject to reductions and offsets in certain circumstances, as well as a royalty on net sales of products that the Company sublicenses ranging from low single digit to low double digit percentages based upon stage of development. The Company is obligated to pay potential total milestone payments of $280,000 based upon achievement of certain stages of development. During the years ended December 31, 2017 and 2016, the Company did not incur any milestone fees.

Duke University License Agreement

On June 18, 2010, the Company entered into a license agreement with Duke University (the "Duke License") pursuant to which Duke granted the Company and its affiliates an exclusive worldwide sublicenseable right and license to practice under certain patent rights and technology to develop, invent, characterize, make, have made, import, export, distribute, offer for sale, sell and otherwise use the licensed patent rights and technology. Unless sooner terminated as provided elsewhere in the agreement, the Duke License term is the later of ten years or until the expiration of the patent rights.

As part of the total consideration for the Duke License, in 2010 the Company issued 700,000 shares of its stock to Duke, which the Company recorded at the fair value of the shares in the amount of $247,500. The fair value of the shares was determined to be more reliably measurable than the fair value of the consideration received.

The Company is required to pay royalties on net sales of products and processes that are covered by patent rights licensed under the agreement at a percentage in the low single digits, subject to reductions and offsets in certain circumstances, as well as a royalty on net sales of products that the Company sublicenses ranging from low single digit to mid-single digit percentages based upon stage of development. The Company is obligated to pay potential total milestone payments of $280,000 based upon achievement of certain stages of development. During the years ended December 31, 2017 and 2016, the Company did not incur any milestone fees.

Sphaera Pharma Pte. Ltd.

On March 2, 2012, the Company entered into a collaborative research and development agreement, or the Sphaera Agreement with Sphaera Pharma Pte. Ltd., or Sphaera, to collaborate on the development of the prodrug technology to be applied to protein kinase inhibitors for oncology and non-oncology indications. Under the terms of the Sphaera Agreement, each party would retain its pre-existing intellectual

property, but any intellectual property conceived or reduced to practice under and certain results arising from the Sphaera Agreement would be assigned to the Company. On October 5, 2012, the Company and Sphaera amended the Sphaera Agreement to reflect joint patent applications in the U.S. and India by us and Sphaera for a series of novel compounds. While the underlying intellectual property would be jointly owned, the Company has the exclusive right to commercialize thirteen of the twenty-four linkers detailed in the filed patent applications, collectively, the Company Compounds, including the linker attached to Imatinib that comprises the 001Pro oncology product, with the remaining nine linkers owned by Sphaera, collectively, the Sphaera Compounds. Sphaera has the right to develop the Company Compounds for oncology indications, but may not commercialize the Company Compounds unless the Company abandons the Company Compounds. The Company has notified Sphaera that they do not intend to abandon any of the Company Compounds. The Company currently does not have the right to develop the Sphaera Compounds. Additionally, if either party files an IND for a Company Compound for an oncology indication in humans, the non-filing party is prohibited from developing such Company Compound.

The prosecution of patents related to the Company Compounds, which includes the prodrug technology, are the responsibility of the Company. As consideration for its services, Sphaera has received a fixed fee of $160,000 and is entitled to milestone payments upon the achievement of specified preclinical, clinical and regulatory milestones ranging from $250,000 upon the first dosing of a patient in a Phase 1 trial to $4,000,000 for FDA approval. Sphaera is also entitled to royalty payments of a percentage of annual net sales and sublicenses ranging in the mid-single digits. The parties did not contemplate the development of IkT-001Pro as a competitor to the generic Imatinib now on the market. As such, the Company and Sphaera are re-negotiating their financial obligations to ensure furtherance of the product to market.

11. Simple Retirement Account for Employees (the "Simple IRA")

The Company established an individual retirement plan for employees effective January 1, 2013 under Section 408(p) of the Internal Revenue Code. The Simple IRA covers substantially all employees of the Company who received at least $5,000 in compensation from the Company during any two preceding years and are reasonably expected to receive at least $5,000 in compensation from the Company in the current year of participation. Subject to certain overall statutory limitations, the Company must match employee contributions up to 3% of employees' qualified compensation for the year. Company contributions under the Simple IRA were $6,720 for each of the years ended December 31, 2017 and 2016.

12. Subsequent Events

Notes Conversion — Mueller and Fante

During May 2018, the Company negotiated conversion terms for the Notes permitting the Holders, at their sole discretion, for a limited time period, to convert any or all of the Unpaid Balance into shares of the Company's stock at the current fair value of the shares. The conditions for the contingent conversion feature included in the Notes was never realized. In May and June 2018, the principal balance plus accrued and unpaid interest of Mueller's Note in the amount of $112,920 and of Fante's note in the amount of $54,797 was converted into 26,950 and 13,078 shares of the Company's stock, respectively, pursuant to the conversion terms.

GRA Notes Conversion

During May 2018, the Company negotiated conversion terms for the GRA Notes permitting the GRA, at its sole discretion, for a limited time period, to convert any or all of the unpaid balance on the GRA Notes into shares of the Company's stock at the current fair value of the shares. On May 31, 2018, the unpaid principal plus accrued and unpaid interest on the GRA Notes in the amount of $234,017 were converted in a total of 54,131 shares of the Company's stock pursuant to the conversion terms.

Warrant Exercise

In May 2018, the Company issued a total of 77,108 shares of its stock in connection with the exercise of two warrants at an exercise price of $0.77 per share. The net proceeds of approximately $59,000 are being used as working capital by the Company.

Share Issuances

In May 2018, an accredited investor subscribed for, and the Company issued 33,378 shares of its stock in a private unregistered transaction at a per share price of $4.19. Net proceeds were approximately $139,000. Issuance costs were not material.

During June and July 2018, an accredited investor subscribed for, and the Company issued 81,145 shares of its stock in a private unregistered transaction at a per share price of $4.19. Net proceeds were approximately $340,000. Issuance costs were not material.

The Company granted certain rights to such accredited investor with respect to the 81,145 shares of its common stock. In the event the Company issues shares or securities convertible into shares at a per share cash price of less than $4.19 in a private transaction intended to be exempt from registration, such accredited investor will have the right of participation in such transaction. In the event the Company issues to non-affiliates shares, or securities that are convertible into shares, at a per share cash price of less than $4.19, pursuant to an effective registration statement, the accredited investor will have the right to receive warrants to purchase shares of its common stock in an amount equal to the aggregate purchase price paid by such accredited investor divided by the lowest price paid by such non-affiliates, with an exercise price of 80% of such lowest price paid by such non-affiliates. In the event that the Company's planned initial public offering does not close until after March 31, 2019, the accredited investor will have the right to receive warrants to purchase up to 40,573 shares of common stock with an exercise price equal to the initial public offering price. The rights described in this paragraph will terminate upon the closing of the Company's planned initial public offering.

The Inhibikase Therapeutics, Inc. 2018 Equity Incentive Plan

In August 2018, the Board of Directors of the Company approved the Inhibikase Therapeutics, Inc. 2018 Equity Incentive Plan, or the 2018 Plan, which is authorized to take effect immediately prior to the closing of the Company's planned 2018 initial public offering.

Subject to certain adjustments, the maximum number of shares of Common Stock that may be issued under the 2018 Plan in connection with awards is 8,770,834, plus any shares that are available or become available under the 2011 Plan. All of these shares may be utilized toward the grant of any type of award, including incentive stock options. The 2018 Plan imposes a $250,000 limitation on the total grant date fair value of awards granted to any non-employee director in any single calendar year.

Board Compensation

In August 2018, the Board of Directors of the Company adopted its equity compensation plan for non-employee directors. All non-employee directors will receive 25,000 non-qualified stock options with a 12-month vesting period for each year of service. The Chairperson will receive an additional 10,000 non-qualified stock options and Committee Chairs will receive 5,000 non-qualified stock options in recognition of their service. Each such grant would be subject to the terms, conditions and any applicable limits as set for in the 2018 Plan.

In addition, non-employee directors will receive $40,000 per year for service as a board member, $30,000 per year additionally for service as non-executive Chairperson of the Board, $20,000 per year additionally for service as chair of the audit committee, $5,000 per year additionally for service as a member of the audit committee (excluding the audit committee chair), $10,000 per year additionally for service as chair of the compensation committee, $5,000 per year additionally for service as a member of the compensation committee (excluding the compensation committee chair), $5,000 per year additionally for service as chair of the corporate governance and nominating committee, $3,000 per year additionally for service as a member of the corporate governance and nominating committee (excluding the chair).

Each member of the Company's scientific advisory board earns $400 per hour for his or her service as a member of its scientific advisory board. The Company also reimburses each member of its scientific advisory board for all reasonable and necessary expenses in connection with the performance of his or her services. Members of the scientific advisory board who are also employees or directors of the Company receive no additional compensation for their service on the scientific advisory board.

The Company's compensation plan for non-employee directors will take effect as of the closing of the Company's planned 2018 initial public offering.

Consulting Agreement

In April 2018, the Company entered into a consulting agreement with its Chief Financial Officer ("CFO"). The agreement provides for $12,500 per month to be paid in cash, with an additional $12,500 per month accruing on a convertible revolving demand promissory note. As of August 17, 2018, the aggregate outstanding principal amount is $62,500 with accrued interest of $435 which can be converted into shares of the Company's common stock at 80% of the then-fair market value per share at the option of the CFO.

Inhibikase Therapeutics, Inc.

Condensed Balance Sheets

	March 31, 2018 (unaudited)	December 31, 2017 (Note 2)
Assets		
Current assets:		
Cash	$ 65,179	$ 16,665
Accounts receivable	147,247	180,780
Prepaid expenses and other current assets	625	625
Total current assets	213,051	198,070
Due from shareholder	87,478	87,097
Total assets	$ 300,529	$ 285,167
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 239,706	$ 353,521
Accrued expenses and other current liabilities	814,396	907,051
Deferred revenue	201,323	5,641
Notes payable	444,523	459,962
Total liabilities	1,699,948	1,726,175
Commitments and contingencies (see Note 8)		
Stockholders' deficit:		
Common stock, $0.001 par value; 30,000,000 shares authorized; 8,919,665 shares issued and outstanding at March 31, 2018 and December 31, 2017	8,920	8,920
Additional paid-in capital	3,024,939	2,858,366
Accumulated deficit	(4,433,278)	(4,308,294)
Total stockholders' deficit	(1,399,419)	(1,441,008)
Total liabilities and stockholders' deficit	$ 300,529	$ 285,167

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Condensed Statements of Operations
(unaudited)

	Three months ended March 31,	
	2018	**2017**
Revenue:		
Grant revenue	$ 691,197	$ 246,312
Consulting revenue	—	1,066
Total revenue	691,197	247,378
Costs and expenses:		
Research and development	585,291	256,890
Selling, general and administrative	218,975	169,783
Total costs and expenses	804,266	426,673
Loss from operations	(113,069)	(179,295)
Interest expense, net	(11,915)	(6,354)
Net loss	$ (124,984)	$ (185,649)
Net loss per share – basic and diluted	$ (0.01)	$ (0.02)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	8,919,665	8,919,665

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Condensed Statements of Cash Flows
(unaudited)

	Three months ended March 31,	
	2018	**2017**
Operating activities		
Net loss	$(124,984)	$(185,649)
Adjustments to reconcile net loss to net cash provided by operating activities:		
Stock-based compensation expense	166,574	75,185
Non-cash interest income from shareholder	(381)	(1,083)
Warrant expense	—	46,118
Changes in operating assets and liabilities:		
Accounts receivable	33,532	(79,162)
Prepaid expenses and other assets	—	625
Accounts payable	(113,815)	26,906
Accrued expenses and other liabilities	(92,655)	133,764
Deferred revenue	195,682	77,394
Net cash provided by operating activities	63,953	94,098
Financing activities		
Increase in due from shareholder	—	(14,000)
Proceeds (repayments) of note payable, net	(15,439)	3,955
Net cash used in financing activities	(15,439)	(10,045)
Net increase in cash	48,514	84,053
Cash at beginning of period	16,665	12,036
Cash at end of period	$ 65,179	$ 96,089
Supplemental disclosures of cash flow information		
Cash paid for interest	$ 2,857	$ —

See accompanying notes to financial statements.

Inhibikase Therapeutics, Inc.

Notes to Condensed Financial Statements
(unaudited)

1. Nature of Business

Inhibikase Therapeutics, Inc. (the "Company"), incorporated on June 3, 2010 as a Delaware corporation with its headquarters in Atlanta, Georgia is developing therapeutics for neurodegenerative disease inside and outside of the brain. The Company anticipates filing two INDs, for its lead program with the U.S. FDA in the first quarter of 2019.

The Company's lead programs utilize small molecule oral protein kinase inhibitors to treat PD and its gastrointestinal complications. The Company has shown that its lead clinical candidate, IkT-148009, a potent, brain penetrant c-Abl protein kinase inhibitor, halts and/or reverses neurodegeneration in the brain and gastrointestinal tract, or GI tract, in preclinical models that mimic the human disease.

Historically, symptoms of a neurodegenerative disease, like a "plaque" made up of a misfolded and/or aggregated protein(s), have been the development focus. The Company focuses instead on the proteins that become dysfunctional in a disease pathway and seeks to understand what leads to their dysfunction. In PD and other neurodegenerative diseases, the Company's pathway-focused approach has enabled a deeper understanding of the origins of the disease and an understanding of how individual proteins are linked together to define the disease process. Using this strategy, the Company has discovered novel therapeutics for c-Abl.

In addition to programs in neurodegeneration, the Company's platform drug discovery and delivery technologies have identified additional opportunities, including treatment of bacterial or viral infections using a single agent at fixed dose and a potential near-term commercial opportunity in oncology that it believes will complete clinical development in 2019, subject to FDA agreements, followed by completion of the FDA requirements for new drug approval in 2020. IkT-001Pro in oncology will seek to validate the pharmacology advantage of the Company's prodrug delivery technology in a cancer patient population. Following validation of IkT-001Pro in oncology, the Company believes the same pharmacology advantages could be applied to IkT-148009, our lead drug for neurodegenerative disease, to enhance clinical development.

Liquidity and Going Concern

The Company has recognized recurring losses. At March 31, 2018, the Company had a working capital deficit of $1,486,897, an accumulated deficit of $4,433,278, cash of $65,179 and accounts payable and accrued expenses of $1,054,102. In addition, as of March 31, 2018, the Company had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. This amount represents additional resources to pay for certain prescribed expenses incurred after March 31, 2018 pursuant to our various notices of award from the National Institute of Health.

The future success of the Company is dependent on its ability to successfully obtain additional working capital, obtain regulatory approval for and successfully launch and commercialize its product candidates and to ultimately attain profitable operations. Historically, the Company has funded its operations primarily through cash received in connections with revenue from its various grants.

The Company is subject to a variety of risks similar to other early stage life science companies including, but not limited to the successful development, regulatory approval and market acceptance of the Company's product candidates, development by its competitors of new technological innovations, protection of proprietary technology, and raising additional working capital. Working capital is defined as current assets less current liabilities. The Company has incurred significant research and development expenses and general and administrative expenses related to its product candidate programs. The Company anticipates costs and expenses to increase in the future as the Company continues to develop its product candidates.

The Company may seek to fund its operations through public equity or private equity or debt financings, as well as other sources. However, the Company may be unable to raise additional working capital, or if it is able to raise additional working capital it may be unable to do so on commercially favorable terms. The Company's failure to raise capital or enter into such other arrangements if and when needed would have a negative impact on the Company's business, results of operations and financial condition and the Company's ability to continue to develop its product candidates.

As of March 31, 2018, the Company had active government grant awards totaling $2,838,798, of which $1,354,754 was still available in accounts held by the U.S. Treasury. However, as certain elements of the Company's operating plan are outside of the Company's control, including the receipt of anticipated grants and funding from a future capital raise, they cannot be considered probable. If the Company does not receive additional working capital from future anticipated grants and future anticipated capital raises, its existing resources are projected to be sufficient to fund its operations through May 31, 2019.

These conditions raise substantial doubt regarding the Company's ability to continue as a going concern for a period of one year after the date the financial statements are issued. Management's plans to alleviate the conditions that raise substantial doubt include delaying certain research projects and capital expenditures and eliminating certain future operating expenses in order to fund operations at reduced levels for the Company to continue as a going concern for a period of 12 months from the date the financial statements are issued. Management has concluded the likelihood that its plan to obtain sufficient funding from one or more of these sources or adequately reduce expenditures will be successful, while reasonably possible, is less than probable. Accordingly, the Company has concluded that substantial doubt exists about the Company's ability to continue as a going concern for a period of at least 12 months from the date of issuance of these financial statements.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainties described above.

2. Summary of Significant Accounting Policies

Basis of Presentation of Interim Financial Statements

The accompanying unaudited condensed financial statements were prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC") and, in the opinion of management, include all normal and recurring adjustments necessary to present fairly the results of the interim periods shown. The December 31, 2017 balance sheet was derived from December 31, 2017 audited Financial Statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepted accounting principles ("US GAAP") have been condensed or omitted pursuant to such SEC rules and regulations. Management believes that the disclosures made are adequate to make the information presented not misleading. The results for the interim periods are not necessarily indicative of results to be expected for the fiscal year ending December 31, 2018. The condensed financial statements contained herein should be read in conjunction with the Company's annual audited financial statements and notes thereto for the year ended December 31, 2017 included in the Company's Registration Statement filed on SEC Form S-1.

These condensed financial statements have been prepared on the assumption that the Company will be able to realize its assets and discharge its liabilities in the normal course of business. This assumption is presently uncertain and contingent upon the Company's ability to raise additional working capital. The financial statements do not include any adjustments relating to recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should the Company be unable to continue as a going concern.

The condensed financial statements have been prepared in conformity with GAAP. Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and are generally adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its financial position or results of operations upon adoption.

We qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act. The JOBS Act permits an emerging growth company such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

Accounting Standards Issued, Not Yet Adopted

In August 2016, the FASB issued ASU 2016-15,*Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments* ("ASC 2016-15"), which provides guidance on the classification of certain specific cash flow issues including debt prepayment or extinguishment costs, settlement of certain debt instruments, contingent consideration payments made after a business combination, proceeds from the settlement of certain insurance claims and distributions received from equity method investees. The standard requires the use of a retrospective approach to all periods presented, but may be applied prospectively if retrospective application would be impracticable. The guidance is effective for public entities for fiscal years beginning after December 15, 2017, and interim periods within those years, and after December 31, 2018 and interim periods beginning after December 31, 2019 for all other entities. Early adoption is permitted. The impact of its pending adoption of ASU 2016-15 is not expected to be material to the Company's financial statements.

In February 2016, the FASB issued ASU No. 2016-02,*Leases* ("ASU 2016-02"), which applies to all leases. Under ASU 2016-02, a right-of-use asset and lease obligation will be recorded for all leases, whether operating or financing leases, while the statement of operations will reflect lease expense for operating leases and amortization and interest expense for financing leases. ASU 2016-02 is effective for public entities for fiscal years beginning after December 15, 2018 and interim periods within those years, and after December 31, 2019 and interim periods beginning after December 31, 2020 for all other entities. Early adoption is permitted. Entities are required to use a modified retrospective approach of adoption for leases that exist or are entered into after the beginning of the earliest comparative period in the financial statements. Full retrospective application is prohibited. The adoption of the new standard is not expected to have a material impact on its financial statements.

In June 2014, the FASB issued amended guidance, ASU No. 2014-09,*Revenue from Contracts with Customers* ("ASU 2014-09"), which is applicable to revenue recognition that will be effective for public entities for fiscal years beginning after December 31, 2017 and interim periods within those years, and after December 31, 2018 and interim periods beginning after December 31, 2019 for all other entities as a result of the deferral of the effective date adopted by the FASB in July 2015. The new guidance must be adopted using either a full retrospective approach for all periods presented or a modified retrospective approach. For public entities, early adoption prior to the original adoption date (annual reporting periods beginning after December 15, 2016) of ASU 2014-09 is not permitted. The new guidance applies a more principles-based approach to revenue recognition. The Company will adopt the new standard, effective January 1, 2019, under the modified retrospective method. The adoption of the new standard is not expected to have a material impact on its financial statements.

3. Supplemental Balance Sheet Information

Accrued expenses consist of the following:

	March 31, 2018	December 31, 2017
	(unaudited)	
Accrued consulting	$ 13,200	$ 13,200
Accrued legal	350,038	350,038
Accrued research and development	233,422	326,988
Accrued interest	126,534	118,060
Accrued other	91,202	98,765
Total accrued expenses	$814,396	$ 907,051

4. Stock-Based Compensation

2011 Equity Incentive Plan

The Company's 2011 Equity Incentive Plan (the "2011 Plan") was established for granting stock incentive awards to directors, officers, employees and consultants to the Company.

Stock Options

During the quarters ended March 31, 2018 and 2017, the Company granted no options and no options were exercised or forfeited.

Stock-Based Compensation Expense

The following table summarizes the stock-based compensation expense for stock options granted to employees and non-employees:

	Quarters ended March 31,	
	2018	2017
	(unaudited)	(unaudited)
Research and development	$ 68,548	$31,405
Selling, general and administrative	98,026	43,780
Total stock-based compensation expense	$166,574	$75,185

5. Warrants

In connection with the GRA Notes, during January 2017, the Company issued to the GRA a warrant to purchase up to 25,000 shares of the Company's stock. The warrant is exercisable at any time prior to January 2027 at an exercise price of $2.02 per share. This warrant was issued in consideration of a 2016 suspension of certain monthly payments of principal and interest under the GRA Notes. Interest continued to accrued on the unpaid balance during this period at the stated rate of five percent. The warrants are classified within stockholders' equity at their fair value and were treated as a standalone instrument. The fair value of the warrant was determined to be $46,118 utilizing the Black-Scholes Merton option-pricing model at the time of issuance.

6. Net Loss Per Share

The following table presents the calculation of basic and diluted net loss per share applicable to common stockholders:

	Quarters ended March 31,	
	2018	2017
	(unaudited)	
Numerator:		
Net loss	$ (124,984)	$ (185,649)
Denominator:		
Weighted-average number of common shares outstanding – basic and diluted	8,919,665	8,919,665
Net loss per share applicable to common stockholders – basic and diluted	$ (0.01)	$ (0.02)

The following shares were excluded from the calculation of diluted net loss per share applicable to common stockholders, prior to the application of the treasury stock method, because their effect would have been anti-dilutive for the periods presented:

	Quarters ended March 31,	
	2018	2017
	(unaudited)	
Options to purchase shares of stock	3,204,166	3,079,167
Warrants to purchase shares of stock	102,108	77,108
Total	3,306,274	3,156,275

7. Income Taxes

During the three months ended March 31, 2018 and 2017, there was no provision for income taxes as the Company incurred losses during both periods. Deferred tax assets and liabilities reflect the net tax effect of temporary differences between the carrying amount of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The Company records a valuation allowance against its deferred tax assets as the Company believes it is more likely than not the deferred tax assets will not be realized. The valuation allowance against deferred tax assets was approximately $0.9 million and 1.2 million as of March 31, 2018 and December 31, 2017, respectively.

8. Commitments and Contingencies

Due from Shareholder

The Company has a receivable from the CEO in the amount of $87,478 and $87,097 at March 31, 2018 and 2017, respectively, classified as “Due from shareholder” in the balance sheets. The receivable is accruing interest at the rate of 1.92% per annum until paid. The receivable from the CEO has a maturity date of the earlier to occur of November 2020 or the date on which the CEO experiences a separation from service from the Company.

Consulting Agreement

In July 2015, the Company entered into a consulting agreement with its Chief Medical Officer (“CMO”), pursuant to which the CMO performs services as the medical director and development of clinical plans and FDA engagements for the Company. The CMO is compensated at $13,000 per month under the agreement.

9. Simple Retirement Account for Employees (the "Simple IRA")

The Company established an individual retirement plan for employees effective in 2013 under Section 408(p) of the Internal Revenue Code. The Simple IRA covers substantially all employees of the Company who received at least $5,000 in compensation from the Company during any two preceding years and are reasonably expected to receive at least $5,000 in compensation from the Company in the current year of participation. Subject to certain overall statutory limitations, the Company must match employee contributions up to 3% of employees' qualified compensation for the year. Company contributions under the Simple IRA were $1,680 and $560 for the quarters ended March 31, 2018 and 2017, respectively.

10. Subsequent Events

Notes Conversion — Mueller and Fante

During May 2018, the Company negotiated conversion terms for the Notes permitting the Holders, at their sole discretion, for a limited time period, to convert any or all of the Unpaid Balance into shares of the Company's stock at the current fair value of the shares. The conditions for the contingent conversion feature included in the Notes was never realized. In May and June 2018, the principal balance plus accrued and unpaid interest of Mueller's Note in the amount of $112,920 and of Fante's note in the amount of $54,797 was converted into 26,950 and 13,078 shares of the Company's stock, respectively, pursuant to the conversion terms.

GRA Notes Conversion

During May 2018, the Company negotiated conversion terms for the GRA Notes permitting the GRA, at its sole discretion, for a limited time period, to convert any or all of the unpaid balance on the GRA Notes into shares of the Company's stock at the current fair value of the shares. On May 31, 2018, the unpaid principal plus accrued and unpaid interest on the GRA Notes in the amount of $234,017 were converted in a total of 54,131 shares of the Company's stock pursuant to the conversion terms.

Warrant Exercise

In May 2018, the Company issued a total of 77,108 shares of its stock in connection with the exercise of two warrants at an exercise price of $0.77 per share. The net proceeds of approximately $59,000 are being used as working capital by the Company.

Share Issuances

In May 2018, an accredited investor subscribed for, and the Company issued 33,378 shares of its stock in a private unregistered transaction at a per share price of $4.19. Net proceeds were approximately $139,000. Issuance costs were not material.

During June and July 2018, an accredited investor subscribed for, and the Company issued 81,145 shares of its stock in a private unregistered transaction at a per share price of $4.19. Net proceeds were approximately $340,000. Issuance costs were not material.

The Company granted certain rights to such accredited investor with respect to the 81,145 shares of its common stock. In the event the Company issues shares or securities convertible into shares at a per share cash price of less than $4.19 in a private transaction intended to be exempt from registration, such accredited investor will have the right of participation in such transaction. In the event the Company issues to non-affiliates shares, or securities that are convertible into shares, at a per share cash price of less than $4.19, pursuant to an effective registration statement, the accredited investor will have the right to receive warrants to purchase shares of its common stock in an amount equal to the aggregate purchase price paid by such accredited investor divided by the lowest price paid by such non-affiliates, with an exercise price of 80% of such lowest price paid by such non-affiliates. In the event that the Company's planned initial public offering does not close until after March 31, 2019, the accredited investor will have the right to receive warrants to purchase up to 40,573 shares of common stock with an exercise price equal to the initial public offering price. The rights described in this paragraph will terminate upon the closing of the Company's planned initial public offering.

The Inhibikase Therapeutics, Inc. 2018 Equity Incentive Plan

In August 2018, the Board of Directors of the Company approved the Inhibikase Therapeutics, Inc. 2018 Equity Incentive Plan, or the 2018 Plan, which is authorized to take effect immediately prior to the effectiveness of the Company's planned 2018 initial public offering.

Subject to certain adjustments, the maximum number of shares of Common Stock that may be issued under the 2018 Plan in connection with awards is 8,770,834, plus any shares that are available or become available under the 2011 Plan. All of these shares may be utilized toward the grant of any type of award, including incentive stock options. The 2018 Plan imposes a $250,000 limitation on the total grant date fair value of awards granted to any non-employee director in any single calendar year.

Board Compensation

In August 2018, the Board of Directors of the Company adopted its equity compensation plan for non-employee directors. All non-employee directors will receive 25,000 non-qualified stock options with a 12-month vesting period for each year of service. The Chairperson will receive an additional 10,000 non-qualified stock options and Committee Chairs will receive 5,000 non-qualified stock options in recognition of their service. Each such grant would be subject to the terms, conditions and any applicable limits as set for in the 2018 Plan.

In addition, non-employee directors will receive $40,000 per year for service as a board member, $30,000 per year additionally for service as non-executive Chairperson of the Board, $20,000 per year additionally for service as chair of the audit committee, $5,000 per year additionally for service as a member of the audit committee (excluding the audit committee chair), $10,000 per year additionally for service as chair of the compensation committee, $5,000 per year additionally for service as a member of the compensation committee (excluding the compensation committee chair), $5,000 per year additionally for service as chair of the corporate governance and nominating committee, $3,000 per year additionally for service as a member of the corporate governance and nominating committee (excluding the chair).

Each member of the Company's scientific advisory board earns $400 per hour for his or her service as a member of its scientific advisory board. The Company also reimburses each member of its scientific advisory board for all reasonable and necessary expenses in connection with the performance of his or her services. Members of the scientific advisory board who are also employees or directors of the Company receive no additional compensation for their service on the scientific advisory board.

The Company's compensation plan for non-employee directors will take effect as of the closing of the Company's planned 2018 initial public offering.

Consulting Agreement

In April 2018, the Company entered into a consulting agreement with its Chief Financial Officer ("CFO"). The agreement provides for $12,500 per month to be paid in cash, with an additional $12,500 per month accruing on a convertible revolving demand promissory note. As of August 17, 2018, the aggregate outstanding principal amount is $62,500 with accrued interest of $435 which can be converted into shares of the Company's common stock at 80% of the then-fair market value per share at the option of the CFO.

Through and including [•], 2018 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

Shares

Inhibikase Therapeutics, Inc.

Common Stock

[•], 2018

PART II

INFORMATION NOT REQUIRED IN THE PROSPECTUS

Item 13. ***Other Expenses of Issuance and Distribution***

The following table sets forth the expenses to be incurred in connection with the offering described in this Registration Statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimates except the SEC's registration fee, the Financial Industry Regulatory Authority, Inc.'s filing fee and the NASDAQ listing fee.

	Amount to be Paid
SEC Registration Fee	$
FINRA filing fee	
NASDAQ listing fee	
Printing expenses	
Legal fees and expenses	
Accounting fees and expenses	
Transfer agent and registrar fees	
Miscellaneous expenses	
Total	$

Item 14. ***Indemnification of Directors and Officers***

Section 145 of the Delaware General Corporation Law empowers a corporation to indemnify its directors and officers and to purchase insurance with respect to liability arising out of their capacity or status as directors and officers, provided that the person acted in good faith and in a manner the person reasonably believed to be in our best interests, and, with respect to any criminal action, had no reasonable cause to believe the person's actions were unlawful. The Delaware General Corporation Law further provides that the indemnification permitted thereunder shall not be deemed exclusive of any other rights to which the directors and officers may be entitled under the corporation's bylaws, any agreement, a vote of stockholders or otherwise. The certificate of incorporation of the registrant to be in effect upon the completion of this offering provides for the indemnification of the registrant's directors and officers to the fullest extent permitted under the Delaware General Corporation Law. In addition, the bylaws of the registrant to be in effect upon the completion of this offering require the registrant to fully indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding (whether civil, criminal, administrative or investigative) by reason of the fact that such person is or was a director or officer of the registrant, or is or was a director or officer of the registrant serving at the registrant's request as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorney's fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such action, suit or proceeding, to the fullest extent permitted by applicable law.

Section 102(b)(7) of the Delaware General Corporation Law permits a corporation to provide in its certificate of incorporation that a director of the corporation shall not be personally liable to the corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except (i) for any breach of the director's duty of loyalty to the corporation or its stockholders; (ii) for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law; (iii) for payments of unlawful dividends or unlawful stock repurchases or redemptions; or (iv) for any transaction from which the director derived an improper personal benefit. The registrant's certificate of incorporation to be in effect upon the completion of this offering provides that the registrant's directors shall not be personally liable to it or its stockholders for monetary damages for breach of fiduciary duty as a director and that if the Delaware General Corporation Law is amended to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of the registrant's directors shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

As permitted by the Delaware General Corporation Law, the registrant has entered into separate indemnification agreements with each of the registrant's directors and certain of the registrant's officers which require the registrant, among other things, to indemnify them against certain liabilities which may arise by reason of their status as directors, officers or certain other employees.

The registrant expects to obtain and maintain insurance policies under which its directors and officers are insured, within the limits and subject to the limitations of those policies, against certain expenses in connection with the defense of, and certain liabilities which might be imposed as a result of, actions, suits or proceedings to which they are parties by reason of being or having been directors or officers. The coverage provided by these policies may apply whether or not the registrant would have the power to indemnify such person against such liability under the provisions of the Delaware General Corporation Law.

These indemnification provisions and the indemnification agreements entered into between the registrant and the registrant's officers and directors may be sufficiently broad to permit indemnification of the registrant's officers and directors for liabilities (including reimbursement of expenses incurred) arising under the Securities Act of 1933, as amended.

The underwriting agreement between the registrant and the underwriters to be filed as Exhibit 1.1 to this registration statement provides for the indemnification by the underwriters of the registrant's directors and officers and certain controlling persons against specified liabilities, including liabilities under the Securities Act with respect to information provided by the underwriters specifically for inclusion in the registration statement.

Item 15. ***Recent Sales of Unregistered Securities***

The following list sets forth information regarding all unregistered securities sold by us in the past three years.

a) In June and July 2018, we issued and sold 81,145 shares of our common stock to an accredited investor at $4.19 per share, for aggregate proceeds of $339,998.

b) In June 2018, we issued 13,078 shares of our common stock to an accredited investor upon conversion of $54,797 of principal and accrued interest under an outstanding convertible promissory note.

c) In May 2018, we issued 77,108 shares of our common stock to an accredited investor in connection with the exercise of two warrants at an exercise price of $0.77 per share for aggregate proceeds of $59,373.

d) In May 2018, we issued 54,131 shares of our common stock to an accredited investor in exchange for the cancellation of $234,017 of principal and accrued interest under outstanding subordinated promissory notes.

e) In May 2018, we issued and sold 33,378 shares of our common stock to an accredited investor at $4.19 per share, for aggregate proceeds of $139,854.

f) In May 2018, we issued 26,950 shares of our common stock to an accredited investor upon conversion of $112,921 of principal and accrued interest under an outstanding convertible promissory note.

g) In January 2017, we issued a warrant to purchase up to 25,000 shares of our common stock at an exercise price of $2.02 to a lender in consideration for suspension of certain monthly payments of principal and interest.

h) We have granted stock options to purchase an aggregate of 725,000 shares of our common stock, with an exercise price of $2.02 per share, to directors pursuant to the 2011 Plan. Since August 17, 2015, no options have been exercised.

The offers, sales and issuances of the securities described in Items 15(a) through 15(g) were exempt from registration under the Securities Act under Section 4(a)(2) of the Securities Act as transactions by an issuer not involving a public offering. The recipients of securities in each of these transactions acquired the

securities for investment only and not with a view to or for sale in connection with any distribution thereof. Each of the recipients of securities in these transactions was an accredited person and had adequate access, through employment, business or other relationships, to information about the Company.

The offers, sales and issuances of the securities described in Item 15(h) were exempt from registration under the Securities Act under either (1) Rule 701 in that the transactions were under compensatory benefit plans and contracts relating to compensation as provided under Rule 701 or (2) Section 4(a)(2) of the Securities Act as transactions by an issuer not involving any public offering. The recipients of such securities were the registrant's employees, consultants or directors and received the securities under the 2011 Plan. The recipients of securities in each of these transactions represented their intention to acquire the securities for investment only and not with view to or for sale in connection with any distribution thereof.

Item 16. ***Exhibit and Financial Statement Schedules***

(a) Exhibits.

The exhibit index attached hereto is incorporated herein by reference.

(b) Financial Statement Schedules.

Schedules have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. ***Undertakings***

The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreements, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act of 1933 shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, as amended, the registrant has duly caused this registration statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Atlanta, State of Georgia, on the [•] day of [•], 2018.

INHIBIKASE THERAPEUTICS, INC.

By: _____

Milton H. Werner, Ph.D.
President and Chief Executive Officer

KNOW ALL MEN BY THESE PRESENTS, that each officer and director of Inhibikase Therapeutics, Inc. whose signature appears below constitutes and appoints Milton H. Werner, Ph.D. and Joseph Frattaroli and each of them, his true and lawful attorney-in-fact and agent, with full power of substitution and revocation, for him and in his name, place and stead, in any and all capacities, to execute any or all amendments including any post-effective amendments and supplements to this Registration Statement, and any additional Registration Statement filed pursuant to Rule 462(b), and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorney-in-fact and agent full power and authority to do and perform each and every act and thing requisite and necessary to be done, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorney-in-fact and agent, or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

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Pursuant to the requirements of the Securities Act of 1933, as amended, this registration statement on Form S-1 has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
Milton H. Werner, Ph.D.	President, Chief Executive Officer and Director (Principal Executive Officer)	[•], 2018
Joseph Frattaroli	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	[•], 2018
Peter Mueller, Ph.D.	Chairperson of our board of directors	[•], 2018
Lisa Evrén	Director	[•], 2018
Richard F. Fante	Director	[•], 2018
Hilary Malone, M.D.	Director	[•], 2018

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
1.1*	Form of Underwriting Agreement, including Form of Lock-Up Agreement
3.1*	Form of Amended and Restated Certificate of Incorporation of Inhibikase Therapeutics, Inc.
3.2*	Form of Amended and Restated Bylaws of Inhibikase Therapeutics, Inc.
4.1*	Specimen common stock Certificate of the Registrant
5.1*	Form of Opinion of Pepper Hamilton LLP
10.1*	Material Contracts, Employment Agreements, Incentive Plans and D&O Indemnification Agreements
23.1*	Consent of CohnReznick LLP
23.2*	Consent of Pepper Hamilton LLP (included in Exhibit 5.1)
24.1*	Power of Attorney (included on the signature page of this Registration Statement)

* To be filed by amendment.

^ Previously filed.

\+ Indicated management contract or compensatory plan.

\# Portions of this exhibit have been omitted pursuant to a request for confidential treatment and this exhibit has been filed separately with the SEC.