bioxcel therapeutics

2019 ANNUAL REPORT

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

FORM 10-K

(Mark one)

☒ **Annual Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**
For the year ended December 31, 2019
or
☐ **Transition Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**
For the transition period from to .

Commission file number 001-38410

BioXcel Therapeutics, Inc.

(Exact name of Registrant as specified in its charter)

Delaware	**82-1386754**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
555 Long Wharf Drive	**06511**
New Haven CT	(Zip Code)
(Address of principal executive offices)	

Registrant's telephone number, including area code: **(475) 238-6837**

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of exchange on which registered
Common Stock, par value $0.001 per share	**BTAI**	**Nasdaq Capital Market**

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports) and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a 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 ☒	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 13(a) of the Exchange Act. ☒

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes ☐ No ☒

As of June 28, 2019, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's common stock held by non-affiliates of the registrant was approximately $63,469,644 (based upon the closing sale price of the registrant's common stock reported on the Nasdaq Capital Market on that date). This calculation excludes shares held by the registrant's current directors and executive officers and stockholders that the registrant has concluded are affiliates of the registrant.

There were 20,182,382 shares of our common stock outstanding at March 9, 2020.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2020 annual meeting of stockholders, which the registrant intends to file pursuant to Regulation 14A with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2019, are incorporated by reference into Part III of this Annual Report on Form 10-K.

TABLE OF CONTENTS

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Annual Report on Form 10-K are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "may," "will," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue" or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our plans relating to clinical trials for BXCL501, BXCL701 and our other product candidates;
- our plans for 505(b)(2) regulatory path approval;
- our plans to research, develop and commercialize our current and future product candidates;
- our plans to seek to enter into collaborations for the development and commercialization of certain product candidates;
- the potential benefits of any future collaboration;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our estimates regarding expenses, future revenue, capital requirements and need for additional financing;
- developments relating to our competitors and our industry;
- the impact of government laws and regulations; and
- our relationship with BioXcel Corporation.

These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part I, Item 1A. "Risk Factors," Part II, Item 7. "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this Annual Report on Form 10-K. Given these uncertainties, you should not rely on these forward-looking statements as predictions of future events. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Annual Report on Form 10-K also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. 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 reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

As used in this Annual Report on Form 10-K, unless otherwise specified or the context otherwise requires, the terms "we," "our," "us," the "Company" or "BTI" refer to BioXcel Therapeutics, Inc. and its subsidiaries, and "BioXcel" or "Parent" refer to BioXcel Corporation, the Company's parent. All brand names or trademarks appearing in this report are the property of their respective owners.

PART I

Item 1. Business

Overview

BTI is a clinical stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed for treatment of a rare form of prostate cancer, pancreatic cancer and advanced solid cancers in combination with other immuno-oncology agents.

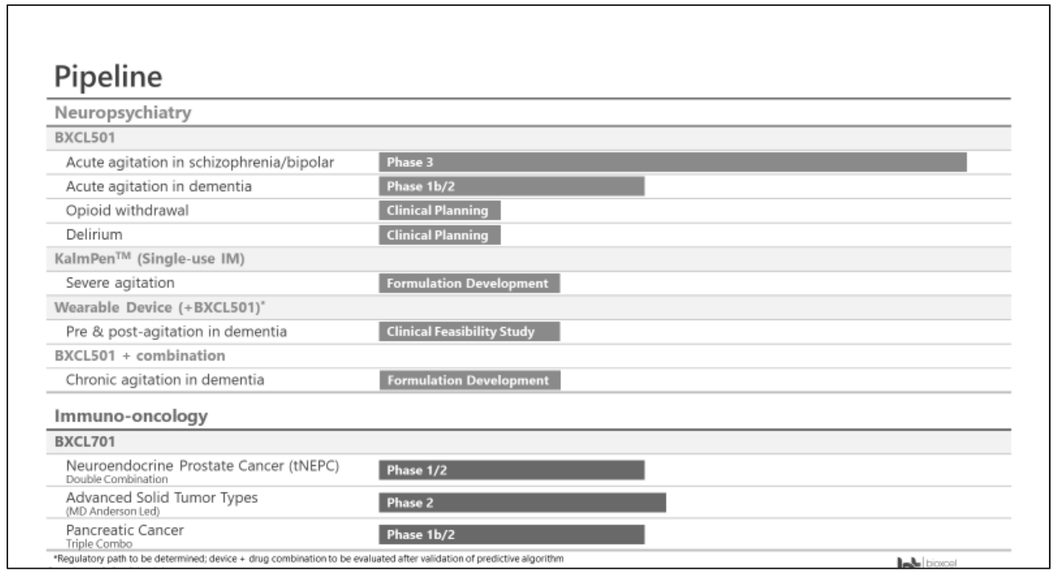
The Company's primary activities have been clinical and pre-clinical research and development of its two most advanced programs: BXCL501, a sublingual thin film formulation of dexmedetomidine, or Dex, designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer and other solid tumors.

We intend to develop first-in-class, high value therapeutics by leveraging EvolverAI, a research and development engine created and owned by our parent, BioXcel. We believe the combination of our therapeutic area expertise and our ability to generate product candidates through our exclusive collaborative relationship with our parent company, BioXcel Corporation in the areas of neuroscience and immuno-oncology gives us a significant competitive advantage. EvolverAI was developed over the last decade and integrates millions of fragmented data points using artificial intelligence, or AI and proprietary machine learning algorithms. After evaluating multiple product candidates using EvolverAI, we selected our lead programs because our analysis indicated these drugs may have utility in new therapeutic indices where there is substantial unmet medical needs and limited competition. By focusing on clinical candidates with relevant human data, we believe our approach will help us design more efficient clinical trials, thereby accelerating our product candidates' time to market. We retain global development and commercialization rights to these two programs.

We operate in a single segment. Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker in making decisions regarding resource allocation and assessing performance. To date, our chief operating decision maker has made such decisions and assessed performance at the company level as one segment.

Our Clinical Programs

The following table summarizes our lead development programs:



Our Strategy

Our goal is to become a leader in the field of neuroscience and immuno-oncology. The key elements to achieving this goal are to:

- **Advance BXCL501, a sublingual thin film formulation of Dex, a selective α_{2a} adrenergic receptor agonist, designed for acute treatment of agitation, to approval through the Section 505(b)(2) pathway.**
  - **Neurological and Psychiatric Disorders.** We believe that BXCL501, if approved, has the potential to become the standard of care for the acute treatment of agitation arising from diseases such as schizophrenia, bipolar disorder, dementia, and other indications.
  - **Additional Indications.** We recently announced plans to evaluate BXCL501 in opioid withdrawal and we may also plan to evaluate additional indications for acute treatment of agitation resulting from delirium, alcohol withdrawal and post-traumatic stress disorder, or PTSD. Dex has been shown to significantly reduce agitation in elderly patients experiencing post-surgical induced delirium who did not respond to treatment with haloperidol, a potent major tranquilizer and antipsychotic that is used to treat symptoms for schizophrenia.
  - **Agitation Franchise Expansion.** We are also investigating potential treatments for the entire spectrum of agitation from pre-agitation to severe agitation. We are exploring the use of wearable digital device technology, such as the Apple watch, with the goal of the prevention and treatment of agitation including, if approved, the administration of BXCL501 prior to the onset of agitation. Additionally, we are considering a combination approach of BXCL501 and another agent for the treatment of chronic agitation. For severe agitation, a single use intramuscular, or IM, injection called KalmPen™ is under development.

- **Complete BXCL701 Phase 2 trials to evaluate its potential for the first-line treatment of treatment-emergent neuroendocrine prostate cancer, or tNEPC, and for the second-line treatment of pancreatic cancer.**

    - **tNEPC (Orphan Segment of Prostate Cancer).** BXCL701 was previously studied in multiple clinical trials and demonstrated single agent anti-tumor activity in melanoma, an immune-sensitive tumor. The U.S. Food and Drug Administration, or FDA, authorized our investigational new drug application, or IND, allowing us to initiate a Phase 2 trial evaluating BXCL701 in combination with Pembrolizumab (KEYTRUDA®) in tNEPC, and this trial opened to accrual in February 2019 and continues enrolling patients.

    - **Pancreatic Cancer.** Preclinical data suggests that fibroblast activation protein positive, or FAP, contribute to checkpoint inhibitor resistance, and immunosuppression more generally in pancreatic cancer. We believe these data provide a strong rationale for combining BXCL701 with a checkpoint inhibitor such as avelumab (Bavencio) or nivolumab (Opdivo). Furthermore, we have observed synergy between BXCL 701 and Bempegaldesleukin (Nektar's pegylated IL2), a CD122 based agonist of IL-2, in a preclinical pancreatic model. BXCL701 has been granted orphan drug designation by the FDA for the treatment of pancreatic cancer.

    - **Basket Trial**. BXCL701 is being evaluated in an open-label phase 2 basket trial led by MD Anderson. The investigator led study is designed to evaluate the response rate of orally administered BXCL701, combined with Pembrolizumab (KEYTRUDA®) in patients with advanced solid cancers. The study will evaluate both patients who are naïve to checkpoint therapy and those who are refractory to checkpoint therapy.

    - **Potential for Expedited Review Programs.** Given that these indications represent high unmet medical needs with few treatment options, we intend to pursue breakthrough therapy designation and accelerated approval for tNEPC and pancreatic cancer.

    - **Additional Indications.** We believe BXCL701 may be active at multiple stages of the cancer immunity cycle and therefore we believe BXCL701 offers a "pipeline in a product" platform given its potential for evaluation across other cancers. BXCL701 was granted an orphan drug designation for the treatment of acute myeloid leukemia in September 2019, its third orphan drug designation in addition to pancreatic cancer and melanoma. We believe existing preclinical evidence supports the combination of BXCL701 with checkpoint inhibitors and/or agents that act on "co-stimulatory" pathways within immune effector cells. Moreover, we believe agents that stimulate antibody dependent cell mediated cytotoxicity, (ADCC) or cell-based therapies such as chimeric antigen receptor T cell (CAR T) therapy, oncolytic viruses or therapeutic vaccines all represent potential combination with BXCL701.

- **Identify biomarkers to select patients who we believe have the highest likelihood to respond to our product candidates.** Predicting optimal drug responses in patients requires the identification and validation of predictive biomarkers. We believe that our ability to identify patient subsets most likely to respond to our product candidates will increase the clinical benefit to patients and improve the probability of success of our clinical trials. The indications for our lead product candidate BXCL701 were chosen in part because they are known to overexpress dipeptidyl peptidase, or DPP 8/9, and FAP. Our planned proof-of-concept clinical trial of BXCL701 will retrospectively examine biomarkers related to its molecular and cellular targets to identify those that may correlate with clinical efficacy and increase our likelihood of success.

- **Enhance our R&D pipeline by leveraging our therapeutic area expertise with EvolverAI to identify, develop and commercialize new product candidates in neuroscience and immuno-oncology.** In addition to our leading clinical programs and our emerging and future pipeline, we intend to select our next clinical program during 2020. We have established translational and development expertise, which we believe will help us advance the present and future product candidates in these fields. We may also opportunistically in-license additional product candidates identified through our AI platform approach within our core areas of expertise.

- **Maximize the commercial potential of our product candidates.** We have worldwide development and commercialization rights to our BXCL501 and BXCL701. If BXCL501 and BXCL701 are approved in the United States, we would consider building a specialty sales force in the United States and/or collaborate with third parties to maximize the potential of our product candidates. Furthermore, we intend to commercialize BXCL501 and BXCL701, if approved, outside the United States through collaborations with third parties.

Our Novel Drug Re-Innovation Approach

Our AI-based discovery and development process is the foundation of our drug re-innovation model for identifying the next wave of medicines. Our therapeutic area experts have over 60 years of experience across the drug discovery and development value chain. We believe EvolverAI is a novel method of finding potential product candidates because it combines the comprehensiveness and efficiency of machine learning and big data analytics with the expertise and intuition of human experience in drug development. We believe the combination of our therapeutic area expertise and our ability to generate therapeutic candidates in neuroscience and immuno-oncology through our exclusive collaborative relationship in those areas with BioXcel give us a significant competitive advantage.

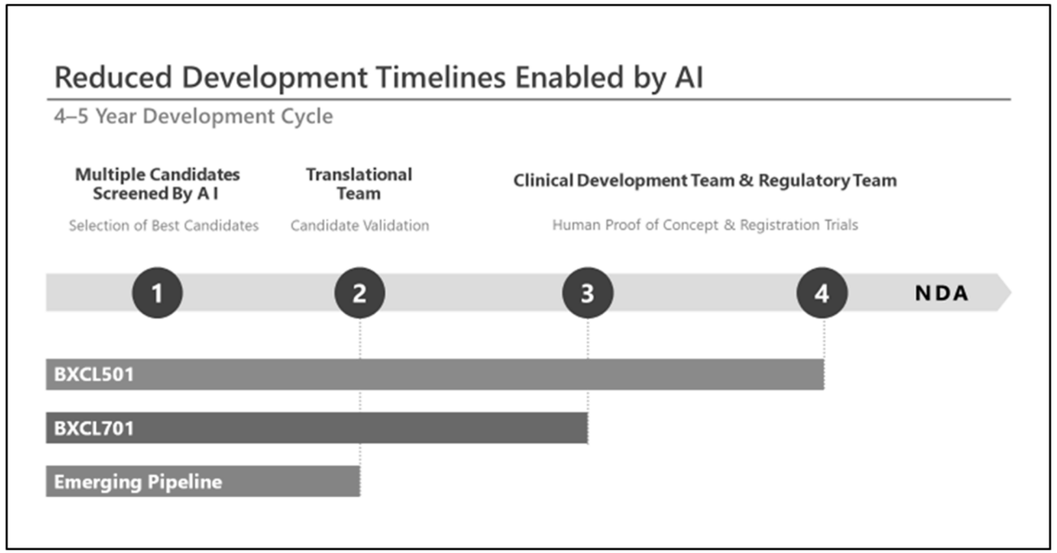
The pharmacological space spans more than 27,000 active pharmaceutical agents and only approximately 4,000 are approved and marketed drugs benefiting patients. These marketed drugs may be applied to other indications, including rare diseases, and represent an untapped potential for meeting significant unmet medical need and recoupment of research and development investments. A large number of the remaining agents are clinical candidates that are active, shelved or have failed for reasons other than toxicity and can potentially be re-engineered for different indications or patient segments. They potentially represent an unrealized investment of billions of research and development dollars by the private and public sectors, resulting in an immeasurable amount of patient suffering and sacrificing during clinical development.

Traditional drug development is plagued with low success rates (13.8%, according to an MIT study of 186,000 trials from January 2000 to October 2015), long drug development cycles (10-15 years, according to PhRMA Key Facts 2016) and exorbitant development costs ($2.6 billion per drug, according to PhRMA Key Facts). Furthermore, many serious diseases continue to go unaddressed due to limitations of the current drug discovery paradigm. The recent advent of numerous 'omics' technologies (genomics, proteomics) and rapid advances in science and medicine are generating terabytes of valuable unexploited knowledge that is widely distributed in multiple big data lakes with several orders of complexity and variety. Much of this data is not being systematically applied to the development of next generation therapeutics, thus preventing the optimization of drug development utilizing the understanding of technology, science, medicine, markets and commercial opportunities. The efficient and intuitive use of big data remains a bottleneck and a challenge to the pharmaceutical industry. Taken together, these factors underscore the need for fundamental new approaches to drug discovery and development. The market opportunity to identify new uses for existing pharmacological agents remains substantial, due to the lack of technology driven insights. Our parent, BioXcel, has created a proprietary R&D engine, EvolverAI, for drug re-innovation that provides a proprietary systems-based approach designed to unlock the hidden value in drugs. The combination of our therapeutic area expertise and our exclusive collaborative relationship with BioXcel enables us to screen, analyze, and identify the product candidates that we believe have a high likelihood of benefiting patients. The compounds in our pipeline have been identified using this proprietary platform.

EvolverAI is designed to eliminate human bias by scanning millions of data points from disparate data sources to create network maps. The nodes and connections in the network map are weighted and ranked based on the validity of supporting evidence using disease specific algorithms. They are then further analyzed using artificial intelligence and machine learning approaches supplemented by human domain-based expertise to uncover novel connections between disease parameters, molecular targets, mechanisms of actions and product candidates.

This drug re-innovation model has been exemplified by the successful development and commercialization of drugs such as Tecfidera (Biogen, Inc.), Thalomid (Celgene Corporation) and Viagra (Pfizer, Inc., or Pfizer). All of these drugs were identified by insights in biology and disease pathophysiology. The successful business models of biotech companies like Axsome-Therapeutics, Inc. and Karuna Therapeutics, Inc. are based on the re-innovation and combination of existing clinical candidates or marketed drugs to provide novel solutions for patients. Unfortunately, such discoveries have been severely limited in scope due to the lack of a genuinely integrated big data analytics based approach.

We believe that only EvolverAI allows a comprehensive and unbiased evaluation of the complete pharmacological space. We believe our drug re-innovation model and exclusive collaborative relationship with BioXcel has the potential to reduce the cost and time of drug development, help us design more efficient trials and accelerate our product candidates' time to market. This assumption is based on capitalizing product candidates with substantial clinical data and mitigated risk due to well defined safety profiles, known PK/PD properties, and an established manufacturing and regulatory path. Our approach is illustrated below:



BXCL501 Sublingual Thin Film, α_{2a} Adrenergic Receptor Agonist, for Acute Treatment of Agitation

Overview

BXCL501 is an investigational sublingual thin film formulation of Dex designed for acute treatment of agitation in neurodegenerative and psychiatric disorders. Dex has been well tolerated, having been prescribed to millions of patients as the sedative anesthetic Precedex and has been studied in over 130 clinical trials. BXCL501 is designed to be a noninvasive, easy to administer sublingual agent and to have a rapid onset of action, which we believe is critical for the acute treatment of agitation. We estimate that 9.4 million patients experience agitation in the United States with up to 18.9 million at risk. These patients suffer from various neuropsychiatric disorders including schizophrenia, bipolar disorder, dementia, delirium, and opioid withdrawal. Agitation episodes that we believe could be addressed by BXCL501 can occur multiple times a year. The current treatment options for acute treatment of agitation utilize antipsychotics and benzodiazepines, which have suboptimal safety and compliance issues. Antipsychotics have a black box warning for use in the elderly and can produce acute debilitating side effects and should only be considered for IM delivery in highly aggressive patients requiring restraint. Benzodiazepines are predominantly in pill form, which require swallowing and can produce excessive sedation. We have designed and are implementing a clinical development

program that takes advantage of the 505(b)(2) New Drug Application, or NDA, regulatory pathway and leverages the existing clinical and safety dataset of intravenous, or IV, formulation of Dex.

Agitation Overview and Market Opportunity

Psycho Agitation is a common symptom of neurological and psychiatric disorders that currently can only be addressed with invasive treatments in institutional facilities. Agitation is characterized by feelings of unease, excessive talking and/or unintentional and purposeless motions, such as wringing of the hands or pacing. People experiencing agitation may also express excitement, hostility, poor impulse control, tension, uncooperativeness and sometimes disruptive behavior, which could lead to aggression and violence. Often symptoms of agitation are observed with anxiety or aggressive behavior. In many cases, people develop agitation when treatment for their underlying disorder is not working well. Stressful situations or traumatic events can also trigger agitation. Agitation can occur suddenly or slowly and vary in length, lasting for a few minutes or for an extended period of time.

With the agitation issues associated with schizophrenia and bipolar disease coupled with a fast-growing elderly population, the difficulties and expenses of acute treatment of agitation are expected to grow significantly. Based on our market research, we estimate that in 2016 the total direct financial cost of all aspects of care for agitation in AD was approximately $40 billion. Management believes that in the near future, the total direct financial cost of all aspects of care for agitation across schizophrenia and bipolar disorder will exceed the costs associated with agitation in AD. Below are estimated statistics associated with BTI's initial indications targeting agitation in AD, schizophrenia and bipolar disease.

U.S. Market for Treating Agitation

	Schizophrenia & Bipolar Disorder	Dementia
Patients at Risk of Agitation.	7,800,000	5,700,000
Patients Experiencing Agitation.	~3,000,000 (40%)	~4,000,000 (70%)
Percent Treatable by BXCL501.	50%?	100%
Addressable Market	~1,500,000	~4,000,000
Estimated Annual Doses per Patient .	24	24#
Potential Addressable Annual Usage.	~36,000,000	~96,000,000

* - Internal company estimates
\# - Episodes increase in frequency as dementia progresses

Limitations of Current Treatments for Agitation

Despite observed suboptimal safety and side effect profile, antipsychotics are currently used off-label to treat agitation in dementia as well as delirium and are currently the standard of care for the acute treatment of agitation in schizophrenia and bipolar disease. IM delivered antipsychotics, such as haloperidol and risperidone, are used extensively in this setting but are invasive and often require patient restraint. Furthermore, these treatments include a black box warning for use in elderly patients. While sublingual tablet formulations utilizing antipsychotics have been developed, these sublingual formulations have long half-lives (21-24 hours) and significant side effects when given either acutely or chronically. Oral agents such as benzodiazepines are also used but have a slow onset of action and are consequently not effective in the acute treatment of agitation. Side effects of these agents include sedation, amnesia, confusion and a paradoxical response. They can intensify cognitive slowing, cause dependence and can contribute to increased risk of falls and fractures. In addition, long-term use of benzodiazepines has been found to be habit-forming and can cause addiction. Non-adherence with oral agents can also be problematic as patients may attempt to spit out these medications.

We believe that based on the current method of administration of oral medicine for agitation, the sublingual thin film offers compliance advantages as it will prevent patients from avoiding treatment.

There is precedent for FDA approval of a non-invasive therapy for the acute treatment of agitation. In 2012, Adasuve, an inhaled version of the antipsychotic loxapine, became the first approved non-invasive acute treatment for agitation in patients with schizophrenia and bipolar disease. The number of hospitals and pharmacies that can administer Adasuve is limited due to a risk of management program, and Adasuve also has a high incidence of side effects. Upon launch, Adasuve was priced at $145 per dose.

The sublingual route of administration is becoming an accepted alternative to oral administration of drug delivery to the central nervous system, or CNS when rapid onset or more controlled delivery is required. Currently, there are six products that are approved for thin film administration. For example, BioDelivery Sciences International, Inc., a commercial-stage specialty pharmaceutical company dedicated to patients living with chronic conditions, has developed a buccal film formulation of buprenorphine for chronic pain management and of buprenorphine and naloxone for opioid dependence. We have developed BXCL501 as a differentiated sublingual thin film dosage form of Dex, which we believe, if approved, may offer benefits such as ease of use and quick absorption for rapid therapeutic effects.

Mechanism of Action: α_{2a} Adrenergic Receptor and NE Role in Acute Agitation

BXCL501 is designed to be easily administered and to have a rapid onset of action. We believe that BXCL501, with its differentiated pharmacology and ease of administration, if approved, could potentially be a first-in-class, non-invasive acute treatment for agitation that can be rapidly administered by physicians and caregivers. Dex is approved in the United States for the sedation of initially intubated and mechanically ventilated patients during treatment in the Intensive Care Unit, or ICU. It is also used in the intensive care setting for sedation of non-intubated patients prior to and/or during surgical and other invasive procedures. Dex, launched in the United States as Precedex in 1999, is a selective α2a adrenergic receptor agonist that has a strong safety record and has been studied in over 130 clinical trials to date. It has also been launched in the European Union and multiple other countries under the trade name Dexdor as a sedative for intensive care patients. Dex gained approval by the European Medicines Agency, or EMA, for sedation of adult ICU patients (requiring a sedation level no deeper than arousal in response to verbal stimulation). It has been used to prevent or treat hyperactive delirium resulting from anesthesia in the ICU. Given these uses of the IV formulation of Dex, we believe Dex formulated in a sublingual thin film will allow for ease of administration in settings where rapid acute treatment of agitation is needed.

Phase 1 IV Dexmedetomidine Studies

Throughout 2018 and 2019 we completed four clinical trials and announced the results from several proof-of-concept Phase 1 studies of intravenous, or IV, Dex for acute treatment of agitation.

- In June 2018, we announced positive results from a phase 1b trial evaluating IV Dex in healthy middle-aged and elderly participants. This initial study enrolled 16 healthy volunteers aged 55-75. The primary endpoint of the study was the dose and drug exposure levels required to produce mild sedation, which served as a surrogate endpoint for treating agitation using the Richmond Agitation- Sedation Scale, or RASS, score.

  The goal of the healthy volunteer study, which was met, was to achieve mild sedation without any clinically meaningful cardiovascular side effects. The IV formulation of Dex achieved mild sedation or a RASS score of -1 in patients at a Dex exposure level without producing any clinically meaningful effects on blood pressure and/or heart rate. This activity was evident in eleven (11) out of twelve (12) subjects on the IV formulation of Dex and occurred within thirty minutes of starting the dose which produced the desired effect. In contrast, mild sedation was observed in only 1 out of 4 individuals on placebo. The mild sedative effects of the IV formulation of Dex persisted for ninety to one hundred and twenty minutes, a clinically relevant duration. The study also found that the IV formulation of Dex was well tolerated and the results supported further clinical evaluation of dexmedetomidine in acute treatment of agitation resulting from neuropsychiatric disorders including schizophrenia and Senile Dementia of the Alzheimer's Type, or SDAT.

- In November 2018, we announced positive results from a Phase 1b study evaluating IV Dex for acute treatment of agitation in patients suffering from schizophrenia. The trial met its primary endpoint by identifying a safe dose of IV Dex that produced a mild arousable sedation, defined by a RASS score of -1. The study enrolled a total of fourteen (14) patients. Ten (10) patients in the treatment arm received IV Dex therapy, while four (4) patients received placebo. Dose escalation was performed by infusing 0.2 to 0.6 mcg/kg/hr of the IV formulation of Dex over a period of thirty minutes. The dose range in this study was consistent with the range used in the healthy volunteer study.

  The study demonstrated that nine out of ten patients in the treatment arm achieved a RASS score of -1, while no patients in the placebo arm experienced meaningful sedation. Additionally, the drug was well tolerated without any clinically meaningful adverse effects on blood pressure and/or heart rate. As a secondary endpoint, nine out of ten patients in the treatment arm had agitation reduced to a minimum as measured by a PEC score of 7 or below ("PEC" Positive and Negative Symptom Scale - Excitatory Component is a five item scale that measures symptoms of agitation with each item rated from 1 Absent to 7 Extreme) in contrast with 0 out of 4 of the placebo patients.

- In January 2019, we announced positive results from a Phase 1 study evaluating IV Dex for acute treatment of agitation in patients suffering from SDAT. The SDAT trial met its primary endpoint by identifying a well-tolerated dose of IV Dex that produced a mild arousable sedation, defined by a RASS of -1.

  This study enrolled a total of fourteen SDAT patients. Ten patients in the treatment arm received IV Dex therapy, while four patients received placebo. In accordance with study designs used in previous participant populations, Dex treatment was begun at 0.1 mcg/kg/h and dose escalation occurred every thirty minutes by increasing the infusion rate by 0.1 mcg/kg/h to a maximum infusion of 0.5 mcg/kg/h. Such dosing allowed for the efficient determination of the optimal dose in each participant. The study demonstrated that seven out of ten patients in the treatment arm achieved arousable sedation (RASS score of -1), versus only 1 of 4 patients in the placebo arm. The drug was well tolerated without any clinically significant adverse events.

- In February 2019, we announced positive proof of concept data from a Phase 1b study of IV Dex in patients suffering from opioid withdrawal symptoms. The study provided evidence supporting further evaluation of BXCL501's selective alpha-2a adrenergic receptor mechanism application in opioid withdrawal symptoms, in addition to acute treatment of agitation in schizophrenia, bipolar disorder and dementia.

  The study enrolled total of fourteen patients with opioid dependence. Ten subjects were randomized to the treatment arm while five subjects were randomized to the placebo arm. Symptoms of opioid withdrawal were evaluated using the Clinical Opioid Withdrawal Scale, or COWS1, an 11-item scale that measures a constellation of withdrawal symptoms experienced after abstaining from opioid use. All ten subjects receiving IV Dex responded to treatment, while there were no responders in the placebo arm. Results from this study suggest that IV Dex mitigated the physiological symptoms of opioid withdrawal.

Data from the four IV Dex studies in healthy volunteers, schizophrenia patients, SDAT patients, and opioid withdrawal patients was used to determine the optimal dose of BXCL501.

BXCL501 Clinical Trials

In December 2018, the FDA accepted our IND application for BXCL501, authorizing us to initiate a first-in-human pharmacokinetic (bioavailability) and safety study in healthy volunteers.

Following the IND acceptance, on December 27, 2018, we announced that the FDA had granted us Fast Track Designation for BXCL501 for the treatment of acute agitation.

FDA's Fast Track program is designed to provide certain benefits for new drugs that treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs. The designation provides the opportunity for more frequent meetings with the FDA over the course of development and allows for the potential of rolling submission of individual sections of an NDA for review. If supported by clinical data, this designation allows for the potential of priority review.

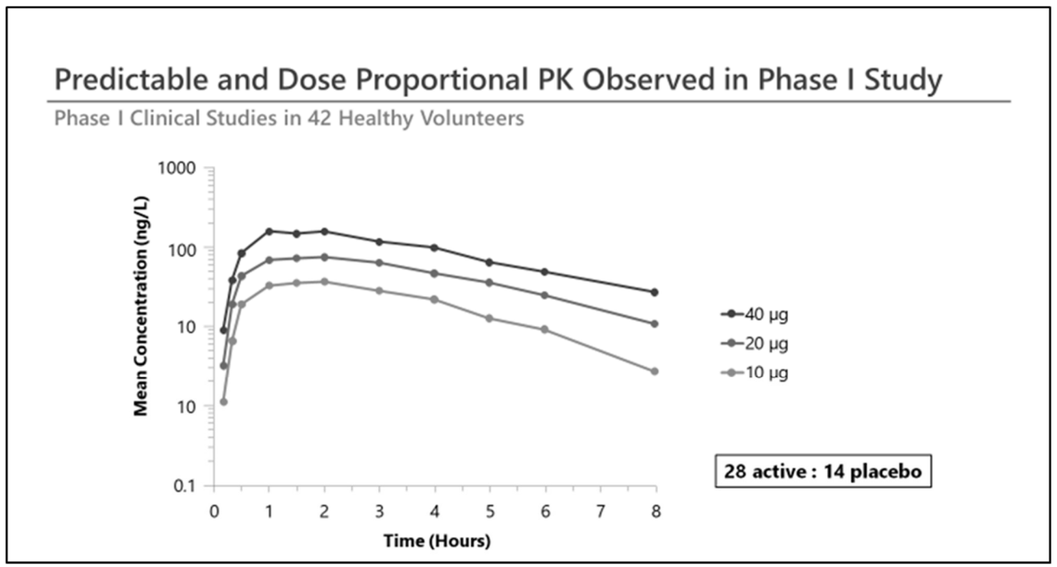
In December 2018, we dosed multiple patient cohorts in our Phase 1 pharmacokinetic (bioavailability) and safety study of BXCL501.

In May 2019, we announced positive top line data from the Phase 1 pharmacokinetic, or PK, (bioavailability) and safety study of BXCL501. Administration of BXCL501 achieved targeted exposure levels that were observed to be therapeutic in our prior IV Dex study.

The IND-opening Phase 1 study was a double-blinded placebo-controlled, single-dose, dose-escalation study of BXCL501 that enrolled 42 adult volunteers across various dosing groups. The primary endpoints of the study were PK and safety, while secondary endpoints included assessment of pharmacodynamics, or PD, and the relationship between BXCL501 concentrations and PD endpoints.

Findings from the study indicated that BXCL501 rapidly achieved exposure levels consistent with the levels observed in IV Dex study in schizophrenia patients that we announced in November 2018. Results from the Phase 1 study also showed dose-proportional PK consistent with the IV Dex study with PD activity lasting 4 to 6 hours, which we believe are clinically favorable features.

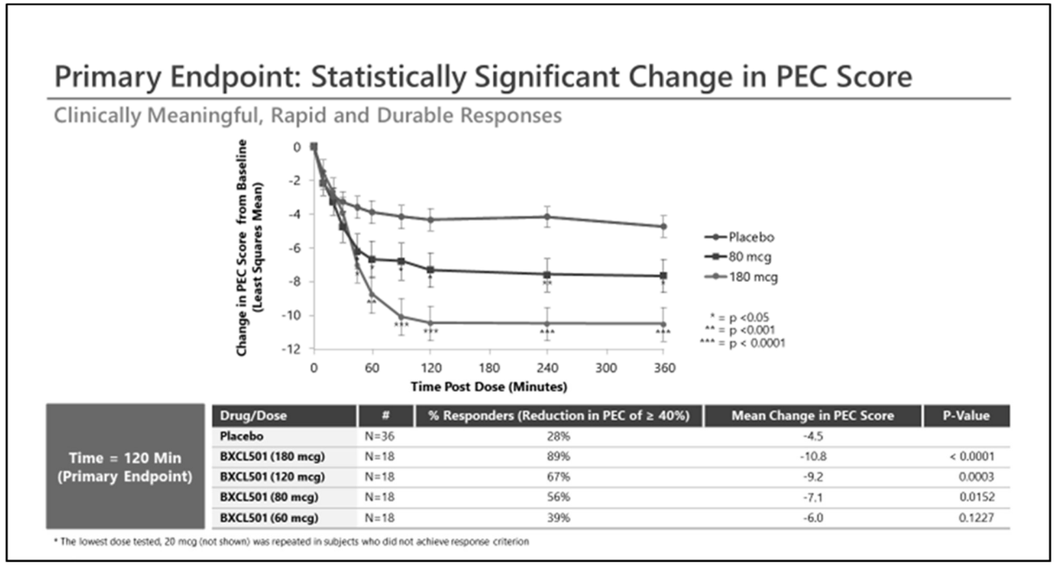
Additionally, clinical data from the study indicates BXCL501 was well tolerated. There were no serious adverse events. The most common adverse event was drowsiness, observed at rates similar to placebo. All adverse events were mild to moderate and transient. There was no clear sedative effect in comparison with placebo. Cardiovascular changes were not clinically meaningful. A maximum tolerated dose was not reached. PK data from the study are illustrated below.



In July 2019, we announced positive top-line results from our Phase 1b, randomized, double-blind, placebo-controlled, multi-center, U.S. trial, evaluating multiple doses of BXCL501 for acute treatment of agitation in 135 patients with schizophrenia. BXCL501 met the trial's primary endpoint of showing a statistically significant mean reduction in PEC score at two hours compared to placebo, with rapid and durable reductions in PEC score maintained for 4 to 6 hours.

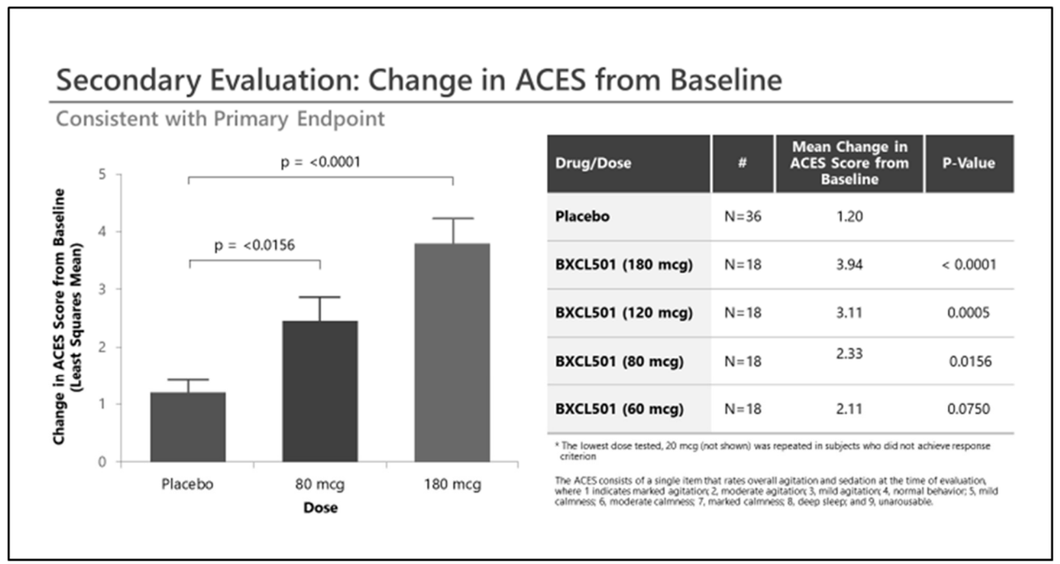
A reduction in the PEC score for agitation was observed with rapid calming without excessive sedation at two hours and at earlier time-points. The 80 mcg, 120 mcg and 180 mcg doses of BXCL501 showed reductions of PEC scores of -7.1, -9.2 and -10.8, respectively, compared to -4.5 for placebo at two hours. The results for these three doses were statistically significant in patients treated compared to placebo (80 mcg; p=0.0152), (120 mcg; p=0.0003), and (180 mcg; p<0.0001) with clinically meaningful, rapid and durable reductions in PEC score. We also observed clinically meaningful but not statistically significant reductions in PEC scores of -6.0 following 60 mcg at two hours (p=0.1227).

The secondary evaluations included assessment using the Agitation-Calmness Evaluation Scale, or ACES, which evaluated the potential calming effects of BXCL501. The ACES assessment was consistent with the analysis of the primary endpoint, and met statistical significance for calming as measured by ACES at two hours compared to placebo in the three highest doses evaluated (80mcg; p=0.0156), (120 mcg; p=0.0005) and (180 mcg; p<0.0001). BXCL501 was well-tolerated with no serious or severe adverse events across the entire dose range; the most common treatment-related adverse events were mild somnolence and dry mouth. Results from the primary and secondary endpoints are outlined below.



	Drug/Dose	#	% Responders (Reduction in PEC of ≥ 40%)	Mean Change in PEC Score	P-Value
Time = 120 Min (Primary Endpoint)	Placebo	N=36	28%	-4.5	
	BXCL501 (180 mcg)	N=18	89%	-10.8	< 0.0001
	BXCL501 (120 mcg)	N=18	67%	-9.2	0.0003
	BXCL501 (80 mcg)	N=18	56%	-7.1	0.0152
	BXCL501 (60 mcg)	N=18	39%	-6.0	0.1227

* The lowest dose tested, 20 mcg (not shown) was repeated in subjects who did not achieve response criterion



On August 20, 2019, we announced that the U.S. Department of Defense's Congressionally Directed Medical Research Program awarded a planning grant as a part of its Alcohol and Substance Abuse Disorders Research Program related to the development of BXCL501. The grant is expected to support the development of a clinical study to evaluate the use of BXCL501 for the treatment of alcohol and substance use disorders, particularly related to post-traumatic stress disorder and traumatic brain injury.

In September 2019, we announced plans to investigate the development of using wearable digital device technology such as the Apple Watch, with the goal of enhancing the prevention and treatment of agitation including, if approved, the administration of BXCL501 prior to the onset of agitation. We plan to conduct a feasibility study with potential applications in commercially available wearable digital devices to measure nervous and motor system activity relevant to both agitation and the mechanism of action of BXCL501. This study is expected be conducted in collaboration with both clinical investigators and a third-party digital solutions provider.

In December 2019, we announced our plans to initiate our pivotal Phase 3 studies following an end-of-Phase 2 meeting with the FDA. We reached a general consensus with the FDA regarding the key elements of the designs for our SERENITY (Sub-Lingual DExmedetomidine in Agitation Associated With SchizophRENIa and Bipolar Disorder STudY) program, two Phase 3 studies of BXCL501 for the acute treatment of agitation in patients with schizophrenia and bipolar disorder.

In December 2019, we also announced initiation of our SERENITY program, with topline data from both Phase 3 trials expected in mid-2020. The SERENITY studies are randomized, double-blinded, placebo-controlled, adaptive trials of up to 750 patients 18 to 75 years of age. SERENITY I will enroll patients with agitation associated with schizophrenia, with each arm receiving either BXCL501 at 120 micrograms, 180 micrograms or a placebo. SERENITY II will evaluate patients with agitation associated with bipolar disorder, also with each of three arms receiving either BXCL501 at 120 micrograms, 180 micrograms or a placebo. The primary endpoint of the trials is reducing acute agitation measured by the Positive and Negative Syndrome Scale, examining the Excited Component, or PEC change from baseline compared to placebo. A key secondary endpoint includes determining the earliest time where an effect on agitation is apparent as measured by the change from baseline in PEC total score. Topline data from both of these Phase 3 trials are expected in mid-2020.

In January 2020, we announced that the first patient had been enrolled in a Phase 1b/2 trial of BXCL501 for the acute treatment of agitation in patients with dementia. Topline data from this trial are expected in mid-2020.

In February 2020, we announced FDA clearance of our investigational new drug application, or IND, for BXCL501 for the treatment of opioid withdrawal symptoms. We are preparing to initiate a Phase 1b/2 clinical trial.

In February 2020, we also announced the initiation of an investigator sponsored clinical trial designed to evaluate the safety and efficacy of BXCL501 and measure biomarkers associated with agitation in patients with schizophrenia and their response to treatment with BXCL501. The trial is designed to identify biomarkers, such as skin conductance response, heart rate variability, and blood pressure, that can potentially be used as an initial signal for treatment and expand the BXCL501 development program to evaluate new chronic disease indications.

We are continuing to evaluate development plans for BXCL501 as a potential treatment for acute agitation in hyperactive delirium and for chronic agitation, as well as developing a single-use intramuscular injection for severe agitation.

Treatment of agitation remains a significant global healthcare challenge in patients with drug and alcohol withdrawal, delirium and post-traumatic stress disorder, as the currently available treatment options are suboptimal, invasive, difficult to administer and often pose safety issues.

Other Neuropsychiatric /Neurodegenerative Indications

Given the differentiated design features of BXCL501 and its selective mechanism of action, we believe that BXCL501 has the potential for broad applicability across several indications where agitation is a symptom of a condition or underlying disease. Schizophrenia and bipolar disorder were chosen as our lead indications. Dementia was chosen based on high unmet medical need and lack of a safe and well-tolerated standard of care for acute treatment of agitation in elderly patients suffering from acute dementia. Schizophrenia and bipolar disorder were also chosen because of the high incidence of agitation in the emergency room and psychiatric outpatient setting resulting from agitation due to residual psychosis or hypo/mania and the need for a noninvasive rapidly acting agent in this setting. There are additional neurological and psychiatric disorders as well as medical conditions where agitation is a symptom that requires treatment.

A brief description of potential indications that we could pursue in the future with BXCL501 is summarized below. We will determine the timing and prioritization of additional indications as warranted by emerging data.

- **Opioid Withdrawal Syndrome.** The burden of treating the current ongoing epidemic of heroin and opiate dependence remains a major priority for federal and state public health services and private healthcare industry. Opioid withdrawal is characterized by physiological symptoms of activation of various bodily functions clinically measured using opioid withdrawal symptom scales (e.g. COWS). Currently preferred options for treatment of acute opioid withdrawal include Medication Assisted Treatment, or MAT, which substitutes opiates with lesser abuse potential drugs such as methadone or buprenorphine. Recently lofexidine (Lucemyra ®), with a similar mechanism of action to Dex, was approved for the treatment of symptoms associated with acute opioid withdrawal. We believe Dex may similarly treat symptoms of acute opioid withdrawal similar mechanism of action by reducing withdrawal symptoms. We believe that the intrinsic properties of Dex delivered via BXCL501, if successfully developed and approved could provide advantages to patients, providers and payers over other currently available non-opioid interventions for acute opioid withdrawal for individuals suffering from opioid use disorder.

  We announced results from our Phase 1b study of IV Dex in patients suffering from opioid withdrawal symptoms in February 2019. The positive data from this Phase 1b trial provided evidence supporting the expansion of the BXCL501 development programs beyond the company's current focus for acute treatment of agitation in neuropsychiatric indications. The study provided evidence supporting further evaluation of BXCL501's selective alpha-2a adrenergic receptor mechanism in opioid withdrawal symptoms, in addition to the acute treatment of agitation in schizophrenia, bipolar disorder and dementia. Opioid addiction is

difficult to overcome largely because of the severe symptoms associated with withdrawal, an area in need of more effective non-opioid treatment options. This unmet need in conjunction with our positive IV Dex study allowed us to begin preparing for a Phase 1b/2 study to evaluate BXCL501 in patients experiencing symptoms of opioid withdrawal. On February 5, 2020, we announced FDA clearance of our IND application for BXCL501 for the treatment of opioid withdrawal symptoms. We are preparing to initiate a Phase 1b/2 clinical trial.

- **Delirium.** There are a number of studies which suggest that Dex can either prevent or mitigate agitation resulting from delirium based on information in an article published in the International Journal of Scientific Reports in 2017 by Zhang et al. We believe BXCL501, if approved, could be used in non-surgical medical situations where hyperactive delirium is an outcome. We also believe BXCL501 would potentially be of high value in elderly patients in many medical situations outside of the ICU, such as the hospital floor and nursing homes. As a result of the delirium studies mentioned in the clinical section above, there is a defined therapeutic index in elderly patients which we believe may allow us to directly initiate a proof-of-concept clinical trial, without conducting separate evaluations of the IV formulation of Dex, potentially followed by a registrational trial with BXCL501. The company is currently establishing a strategic development plan to study BXCL501 in hyperactive delirium.

- **Alcohol Withdrawal Syndrome.** Acute alcohol withdrawal remains a widespread problem in hospitalized patients. Benzodiazepines remain the primary treatment for alcohol therapy to help control hyperadrenergic output in patients resulting in withdrawal. These patients are at increased risk of experiencing respiratory depression from benzodiazepine therapy. Based on information in an article published in The American Journal of Drug and Alcohol Abuse in 2015 by Wong et al., in clinical trials, IV administration of Dex has shown potential for treating alcohol withdrawal syndrome. We believe that performing a controlled clinical trial with BXCL501 in this population would be a logical next step to develop this product candidate. In August 2019, we announced the award of a grant by the U.S. Department of Defense's Congressionally Directed Medical Research Programs to evaluate the use of BXCL501 for treatment of alcohol and substance use disorders, or ASUD, related to PTSD and traumatic brain injury. Further planning is currently underway

- **Hyperarousal in PTSD.** Hyperarousal is a primary symptom of post-traumatic stress disorder, or PTSD. It occurs when a patient becomes hyperaroused which could be as a result of a trigger or another event leading to recollection and re-experiencing or thinking about their trauma. Even though real danger may not be present, their body acts as if there is present danger, causing lasting stress after a traumatic event. The symptoms of hyperarousal include irritability, anger and angry outbursts, constant anxiety, restlessness and sleeping problems. We believe that BXCL501 has the potential to reduce symptoms which lead to agitation as well to produce a more natural sleep if taken before bedtime.

BXCL701, DPP 8/9 and FAP Inhibitor for the Treatment of tNEPC and Pancreatic Cancer

BXCL701

BXCL701 is a highly potent, oral small molecule immuno-modulator that is designed to both stimulate the innate immune system and inhibit immune evasion by inhibiting dipeptidyl peptidase, or DPP, 8/9 and fibroblast activation protein, or FAP. tNEPC and pancreatic cancer were selected as our lead indications after evaluating more than 100 different cancer types. These two indications are in the top three cancers that overexpressed or amplified DPP 8/9 and FAP. Data in the literature points to a functional role for DPP 8/9 in the biology of tNEPC. Moreover, in a number of animal models, notably pancreatic cancer, BXCL701 showed combinatorial activity with checkpoint inhibitors and other combination partners. Administration of these triple combinations resulted in complete regression with the generation of functional T-cell memory in selected animal models. Specifically, DPP8 and DPP9 have been shown recently to behave as "immuno-checkpoints," as their inhibition results in a potent pro-inflammatory, anti-tumor activity by way of the induction of pyroptosis in macrophages. Pyroptosis results in induction of a number of cytokines, notably the pro-inflammatory cytokines IL-1β and IL-18. This cytokine induction is believed to result in the downstream stimulation of multiple tumor-killing immune cells. Our preclinical data supports this hypothesis, as cancer models with a high density

of macrophages have responded robustly to BXCL701 containing combination therapy, while models with lower density of macrophages did not respond robustly to BXCL701 containing combination therapy. Taken together we believe that BXCL701 may activate an important component of the innate immune system thereby functionally inflaming the tumor microenvironment. Induction of an innate immune response within the tumor provides a host of pro-immune signals which drive subsequent stimulation and recruitment of the adaptive immune system.

Clinically, BXCL701 has been evaluated in more than 700 healthy subjects and cancer patients across multiple clinical trials, which provided evidence regarding tolerability, proof of mechanism, and single agent anti- tumor activity. In the latter case, single agent activity was seen in melanoma patients, an immuno sensitive tumor. While providing evidence regarding the safety profile of the drug, these clinical studies also identified a maximum tolerated and recommended Phase 2 dose to use in future clinical trials.

BXCL701 is in development for the treatment of tNEPC, a segment of prostate cancer patients that have progressed on second generation androgen inhibitors (Zytiga and Xtandi). Approximately one in four patients treated with Zytiga and Xtandi are expected to develop tNEPC based on current clinical literature. The combined global sales of Zytiga and Xtandi, which are only approved for prostate cancer treatment, were over $7 billion in 2018 and we believe such sales number gives a perspective of the potential market for BXCL701 in this indication. Additionally, generic alternatives for Zytiga became available in the U.S. market in 2019 and we therefore expect the number of patients with access to androgen inhibitor therapy to increase.

In pancreatic cancer, we estimate that approximately 20,000 patients annually would be eligible for treatment with BXCL701, if approved, as about 50% of pancreatic cancer patients can receive second-line therapy based on information in an article published in the Annals of Oncology in 2013 by Rahma et al.

Neuroendocrine-Prostrate Cancer Overview and Market Opportunity

Prostate cancer is the most common malignancy and is the second leading cause of cancer death in men in the United States. In 2016, there were an estimated 3 million men with prostate cancer in the United States. According to estimates from Surveillance, Epidemiology and End Results Program, SEER, more than 174,000 men are expected to be diagnosed and more than 31,000 men were expected to die from prostate cancer in 2019. While the five-year survival rate of local and regional prostate cancer is almost 100%, more aggressive forms of the disease such as metastatic prostate cancer have a five-year survival rate of approximately 30%. These aggressive forms of prostate cancer can initially be treated with androgen deprivation therapy, or ADT, however, almost all patients experience a recurrence in tumor growth which results in the patient having castrate resistant prostate cancer, or CRPC. An estimated 180,000 men in the United States are eligible for treatment with the second-generation anti-androgen drugs Zytiga and Xtandi. These drugs have widely become the standard of care and generated combined worldwide sales of over $7 billion in 2018.

Unfortunately, virtually all the patients who respond to Zytiga and Xtandi are expected to progress to even more aggressive forms of prostate cancer requiring further treatment. About one in four of the progressing patients will develop very aggressive, androgen receptor, or AR-independent tumors, or tNEPC, for which there is no effective treatment based on information in an article published in the Journal of the National Comprehensive Cancer Network in 2014 by Agarwal et. al. and an article published by Journal of Clinical Oncology in 2014 by Wang et. al. tNEPC specifically displays neuroendocrine differentiation, either pathologically with the presence of the typical neuroendocrine small cells, or molecularly by expressing neuroendocrine markers. BXCL701 is designed to target this

tumor segment because tNEPC has specific biology that we believe is addressable by the mechanism of action of BXCL701.

Large market Opportunity: tNEPC	
U.S. Prostate Cancer Patient Population	~3,000,000
Patients Eligible for Treatment with ADT	~180,000 (6%)
Patients progressing to tNEPC	~20,000

Limitations of Current Treatments for tNEPC

There is no approved therapy for tNEPC. tNEPC patients are treated off-label with cytotoxic chemotherapies, such as platinum-based regimens. These treatments have poor efficacy due to their short duration of response and substantial toxicity. While the immuno-oncology field has made several advances in the treatment of solid tumors several trials of immuno-oncology agents in patients with prostate cancer, and specifically tNEPC, have shown limited or no anti-tumor activity. We believe BXCL701, if approved, has the potential to be first-in-class therapy in tNEPC given its potential to convert immuno-resistant tumors to immuno-sensitive tumors (“cold” to “hot” tumors).

Our Solution: BXCL701, A Potential First-in-Class, Oral, Small Molecule Inhibitor of DPP 8/9 and FAP

We are not aware of any clinical stage competitors of BXCL701 in the DPP inhibitor class. The product candidate is designed to address the various ways by which DPP 8/9 and FAP play a role in the biology of tNEPC and pancreatic cancer. Specifically, BXCL701 is designed to directly affect tNEPC tumor cell survival and metastases but also modulate anti-cancer immunity against tNEPC and pancreatic cancer as described below.

- **Immuno-mediated Activity Against tNEPC.** We believe BXCL701 may modulate the immune system in multiple ways based on information in an article published by Nature Chemical Biology in 2017 by Okondo et al. and an article published in PLOS One in 2013 by Walsh et al., several of which are relevant to its potential to treat tNEPC, including:
    - stimulating the activation of multiple immune cell types
    - stimulating tumor cell killing by inducing the priming, migration and cytotoxicity of T-cells and the formation of memory T-cells
    - stimulating tumor cell killing by inducing the proliferation and activation of neutrophils
    - inhibiting the immune suppressive FAP+ CAF and Myeloid derived suppressor cell, or MDSC and delaying or preventing tNEPC development
    - synergistically increasing checkpoint inhibitor anti-tumor activity.
- **Inhibiting tNEPC Growth Factor NPY.** tNEPC is believed to be caused by neuroendocrine cells in the prostate that overexpress NPY. NPY activates the specific G protein-coupled receptor Y1-R, which then selectively stimulates growth of AR-independent, tNEPC-like cancer cells, while reducing growth in AR-dependent cells. NPY is a substrate of DPP 8/9, which cleaves it into biologically active forms. DPP 8/9 inhibition in tumor cells decreases the number of viable tumor cells by reducing NPY cleavage.

- **Inhibiting the Formation of tNEPC-type (Osteoclastic) Bone Metastasis.** Prostate cancer is characterized by the presence of bone-forming (osteoblastic) metastasis. In contrast, tNEPC is associated with bone-lysing (osteoclastic) metastasis. BXCL701 is designed to block the bone destruction by osteoclasts through the inhibition of osteoclast differentiation. In an animal model that recapitulated the formation of osteolytic metastasis of tNEPC, BXCL701 was observed to reduce osteoclast activity, bone resorption and tumor burden based on information in an article published in the British Journal of Haematology in 2009 by Pennisi et al.

BXCL 701 for the Treatment of Pancreatic Cancer

Pancreatic Cancer Overview and Market Opportunity

Pancreatic adenocarcinoma, more commonly referred as pancreatic cancer, represents one of the highest unmet needs in oncology. The American Cancer Society estimates that in 2020 there will be approximately 57,600 new diagnoses and 47,000 deaths. Pancreatic cancer has a median five-year survival rate of only about 9% for all stages combined. Recently, several new therapies have been developed consisting of new formulations of approved chemotherapies. However, these new therapies have limited efficacy with relatively short survival advantages, and well-known toxicities. It is well understood that the development of new efficacious drugs with manageable toxicity is required to achieve durable responses and increase survival in pancreatic cancer. Pancreatic cancer is known to be a highly immuno-resistant tumor. Multiple attempts to show anti-tumor activity of immunotherapies including immune checkpoints have failed due to primary resistance mechanisms. We believe BXCL701 has the potential to address the resistance to immune checkpoint inhibitors (to make “cold” tumors “hot”) and the combination with Anti-PD-1 inhibitors and other agents, particularly those such as Nektar Therapeutics’ Bempegaldesleukin activate the adaptive immune response, could generate long and profound responses and the survival increase needed to make a true breakthrough in the treatment of pancreatic cancer.

Abraxane, a new formulation of the chemotherapy agent paclitaxel in combination with gemcitabine, is considered to be the standard of care for newly diagnosed pancreatic cancer in U.S. markets, with annual sales of over $1 billion in 2018. Onivyde, a liposomal formulation of the chemotherapy agent irinotecan, was recently approved for use in second-line pancreatic cancer based on a two-month survival increase (six months vs. four months) and only 7.7% ORR, with annual sales of approximately $109 million in 2018. Our initial clinical development plan will target second-line or later pretreated patients, specifically the 50% or more that remain in good clinical condition after first-line treatment and thus may receive one or more subsequent lines of chemotherapy. Therefore, we believe that the potential number of patients treatable with the combination of BXCL701, a check point inhibitor and Nektar 214, if successfully developed and approved, would be approximately 20,000.

Pancreatic cancer is a high unmet medical need, where approved therapies have limited activity and patients have short survival. As in tNEPC, single agent immune checkpoint inhibition has not shown single agent anti-tumor activity in pancreatic cancer patients, except in the very small (1-2%) fraction that exhibit MSI-H phenotype, indicating the need to optimize their activity. Preclinical studies suggest that the combination of DPP8/9 and FAP and immune checkpoint inhibition was active. BXCL701 has been granted orphan drug designation from the FDA for the treatment of pancreatic cancer.

Large market Opportunity: Pancreatic Cancer	
U.S. Pancreatic Cancer Patient Population	~74,000
Patients Eligible for Second Line Therapy	~37,000 (50%)
Patients progressing to tNEPC.	~20,000

BXCL 701 Clinical Trials

In November 2018 we announced that the FDA accepted our IND for BXCL701. We subsequently initiated a Phase 1b /2 trial evaluating the combination of BXCL701 and pembrolizumab (Keytruda®) in tNEPC patients during the fourth quarter of 2018.

The goal of this single arm, Simon 2-stage open label study is to examine the safety, pharmacokinetics and anti-tumor activity combining of BXCL701 and pembrolizumab in tNEPC patients with the primary efficacy endpoint of objective response rate. In October 2019 we presented top-line data from the first safety cohort at the 2019 Annual Prostate Cancer Foundation Scientific Retreat. In this initial cohort (n = 3 patients), the combination of BXCL701 (0.4mg/day) and Keytruda® showed no serious adverse events or dose limiting toxicities. Preliminary data from the patient cohorts consisting of 400 mcg of BXCL701 plus Keytruda® and 600 mcg of BXCL701 along with Keytruda® has demonstrated on-target side effects consistent with cytokine activation at the highest once-daily dose tested and preliminary PK of BXCL701 are also within expectations. Of the 6 patients dosed in connection with this trial, the majority of patients remain on treatment, with one patient experiencing acidosis with a fatal outcome. We are currently enrolling patients in the 600 mcg cohort with a split dose and expect to complete dosing in the first quarter of 2020. Data as of January 30, 2020 from this open label trial of patient cohorts consisting of 400 mcg of BXCL701 in combination with Keytruda® and 600 mcg of BXCL701 in combination with Keytruda® showed on-target adverse events that we believe are consistent with cytokine activation at the highest once-daily dose tested. BXCL701 also showed preliminary pharmacokinetic results that were within expectations. Of the 6 patients dosed as of the data cutoff date, the majority of patients remained on treatment. One patient experienced acidosis with a fatal outcome that was determined by the investigator to be possibly related to BXCL701. We are currently enrolling patients in the 600 mcg cohort with a split dose and expect to complete dosing in the first quarter of 2020. Topline data from this trial are expected in the second half of 2020, prior to advancing to the Phase 2 efficacy portion of the trial.

In June 2019, we announced that our Clinical Trial Application was accepted by the U.K. Medicines and Healthcare products Regulatory Agency for the double combination trial of BXCL701 and Keytruda® in treatment-related neuroendocrine prostate cancer patients. We have activated two clinical sites in the U.K. and are planning to open a third clinical site, subject to approval from local U.K. authorities. This is the first step in our plan to expand our clinical trials globally.

In June 2019, we also announced that the FDA had cleared our IND application to initiate a clinical trial evaluating the triple combination of BXCL701, bempegaldesleukin (produced by Nektar Therapeutics, Inc., or Nektar) and BAVENCIO® (avelumab, Merck KGaA, Darmstadt, Germany and Pfizer) in pancreatic cancer. We expect to initiate the trial in the second half of 2020, following Nektar and Pfizer's safety run-in study of a double combination of Bempegaldesleukin and avelumab, pending the outcome of the double combination arm of the trial.

In December 2019, we announced the expansion of the clinical evaluation of BXCL701 into multiple advanced solid tumors. The MD Anderson-led Phase 2 open-label basket trial is designed to evaluate the response rate of orally administered BXCL701, combined with KEYTRUDA® in patients with advanced solid cancers. The study will evaluate both patients who are naïve to checkpoint therapy and those who are refractory to checkpoint therapy. MD Anderson has indicated that it expects to initiate this trial in the first half of 2020 with initial data expected in the second half of 2020.

We are continuing to explore additional indications for BXCL701 with synergistic combinations.

Nektar Therapeutics Collaborative Agreement

On September 21, 2018, the Company entered into a Clinical Trial Collaboration Agreement, or the Collaboration Agreement, with Nektar Therapeutics, a Delaware corporation, or Nektar. Pursuant to the Collaboration Agreement, the Company and Nektar will jointly collaborate to conduct a Phase 1/2 clinical trial evaluating a combination therapy using BXCL701, Bempegaldesleukin, a CD122-biased agonist and a checkpoint inhibitor as a potential therapy for pancreatic cancer and such other clinical trials evaluating the combined therapy as may be mutually agreed upon by the parties, each referred to as a Combined Therapy Trial. Under the Collaboration Agreement, the parties will split all out-of-pocket costs reasonably incurred from third parties in connection with the performance of a Combined Therapy Trial, including, but not limited to, third party contract research organizations, laboratories, clinical sites and institutional review boards. Each party will otherwise be responsible for its own internal costs, including internal personnel costs, incurred in connection with each Combined Therapy Trial. The Company and Nektar will use commercially reasonable efforts to manufacture and supply its compound for each Combined Therapy Trial and will bear the costs related thereto. The parties have formed a joint development committee to oversee clinical trial design, regulatory strategy, and other activities necessary to conduct and support the Combined Therapy Trials. The Company will act as sponsor of each Combined Therapy Trial. Ownership of, and global commercial rights to, BXCL701 remains solely with the Company under the Collaboration Agreement. Ownership of any patent rights and study data that does not relate exclusively to BXCL701 or Bempegaldesleukin shall be jointly owned by the parties. Each party granted to the other party a non-exclusive, worldwide, non-transferable and royalty-free research and development license to such licensing party's patent rights, technology and regulatory documentation to use its compound solely to the extent necessary to discharge its obligations under the Agreement with respect to the conduct of the Combined Therapy Trials.

Subject to termination rights for breach, bankruptcy or a material safety issue/clinical hold, the term of the Collaboration Agreement will continue in effect until completion by all centers or institutions participating in the Combined Therapy Trials, the delivery of study data to both parties and the completion of any then agreed upon protocol, statistical analysis and bioanalysis plan.

On March 4, 2019, the Company announced the addition of Merck KGaA, Darmstadt, Germany, which operates its biopharmaceutical business as EMD Serono in the USA and Canada, and Pfizer to the Nektar clinical collaboration described above to evaluate a novel triple combination therapy in pancreatic cancer. The collaboration now includes avelumab, BXCL701 and NKTR-214 as a potential combination therapy for pancreatic cancer. Avelumab is a human anti-programmed death ligand (PD-L1) co-developed and co-commercialized by Merck KGaA Darmstadt, Germany and Pfizer.

Under the collaboration, BTI will continue to be responsible for initiating and managing the clinical program, with Merck KGaA, Darmstadt, Germany and Pfizer supplying avelumab and Nektar supplying NKTR-214. BTI and Nektar will equally share all development costs.

Other Immuno-oncology Indications

In addition to tNEPC and pancreatic cancer, we plan to leverage our existing preclinical and clinical data to identify other cancer types with high unmet medical need that we believe would benefit from BXCL701's novel designed mechanism of action. We are prioritizing indications where the immuno-suppressive microenvironment is driven by the potential molecular and cellular targets of BXCL701 and where the single agent activity of approved immune checkpoint inhibitors is limited. On September 4, 2019 we announced that the FDA granted Orphan Drug Designation for BXCL701 for the treatment of acute myeloid leukemia.

In addition, we believe BXCL701, if successfully developed and approved, may provide a platform for combination with immunotherapy modalities that go beyond the currently approved immune checkpoint agents that target the PD-1/PD-L1 axis. Following our proof-of-concept trials, we plan to conduct clinical trials covering a broad range of additional combinations with other immunotherapy agents including:

- immune checkpoint inhibitors (other than PD-1/PD-L1)
- cellular therapies (CAR-T and chimeric antigen receptor natural killer cells)
- therapeutic vaccines
- ADCC driven monoclonal antibodies

Other Product Candidates

Neuroscience Program

We are targeting neuroscience disorders where there is high unmet medical need and therefore a requirement for symptom management is a priority (like agitation, seizures, dyskinesias) as well for transformative care for monogenic rare CNS disorders.

For symptomatic approaches, our neuroscience program is developing product candidates with a focus on treating symptoms for various neurological and psychiatric disorders. This entails re-innovating existing agents through formulation changes and deuteration. The utilization of EvolverAI has identified several monogenic diseases with available animal models across rare neuroscience diseases. We utilize proprietary algorithms to identify associated mechanisms with existing pharmacology to test whether these agents can improve the disease profile in the animal model either through disease modification or symptomatic manner. The agents identified must be those that we believe are Phase 2 ready with a potential for a short, cost-effective development plan (four to five years to potential NDA submission).

Immuno-oncology Program

Our immuno-oncology program is based on utilizing a comprehensive map of all known relationships that link immuno-evasion and immuno-activation pathways and targets with thousands of pharmacological agents and tumor indications. This comprehensive map has permitted us to select a potential pipeline of candidates based on our ability to alter the tumor micro-environment and the potential to address relevant unmet medical needs for various tumor types.

Finally, we continually leverage the artificial intelligence platform owned by our parent, to select and prioritize additional development opportunities to expand the current portfolio and broaden the addressable market for our lead programs through identification of new indications. This includes exploring additional combination therapy approaches to expand BXCL701's target indications beyond tNEPC and pancreatic cancer.

Competition

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. The immuno-oncology, neuroscience and rare disease segments of the industry in particular are highly competitive. While we believe that our technology, development experience and scientific knowledge provide competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies, and public and private research institutions.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical studies, conducting clinical trials, obtaining regulatory approvals and marketing approved medicines than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and in establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or

necessary for our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, the effectiveness of companion diagnostics in guiding the use of related therapeutics, if any, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize medicines that are safer, are more effective, have fewer or less severe side effects, are more convenient or are less expensive than any medicines we may develop. Our competitors also may obtain FDA or other regulatory approval for their medicines more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic medicines. There are many generic medicines currently on the market for certain of the indications that we are pursuing and additional generics are expected to become available over the coming years. If our therapeutic product candidates are approved, we expect that they will be priced at a significant premium over competitive generic medicines.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. If the product candidates of our priority programs are approved for the indications for which we are currently planning clinical trials, they will compete with the drugs discussed below and will likely compete with other drugs currently in development.

Neurological and Psychiatric Disorders

Drugs used for the acute treatment of agitation resulting from psychosis in schizophrenia and mania in bipolar disease are atypical antipsychotics administered IM and often require patient restraint. These include IM aripiprazole, olanzapine, ziprasidone and haloperidol. Oral products include the benzodiazepines, lorazepam and midazolam. Saphris (sublingual tablet asenapine) is an atypical antipsychotic that has been prescribed for use in children and teens for acute treatment of manic or mixed episodes associated with bipolar disease. Adasuve (inhaled loxapine) from Alexza is also a non-invasive treatment.

Immuno-oncology

The immuno-oncology field is characterized by the rapid evolution of technologies and products and by fierce competition based on the development of compounds, often with similar mechanisms of action. Clinical development plans are further compounded by the possibility of overlapping intellectual property. A wide variety of commercial players, large pharmaceutical companies, established and emerging biotechnology companies, and several not-for-profit entities are actively developing potentially competitive products in immuno-oncology and in our lead indications.

While we believe our product candidates, technology, knowledge, and experience provide us with competitive advantages, we face competition from established and emerging pharmaceutical and biotechnology companies. Such companies include:

- **Major pharmaceutical companies developing multiple immuno-oncology agents:** AstraZeneca PLC, Bristol-Myers Squibb Company, Celgene Corporation, Merck & Co., Inc., Novartis AG, Pfizer Inc., Roche Holding Ltd. and Sanofi SA.
- **Companies developing agents aimed at stimulating the immune response:** AdaptImmune LLC, Idera Pharmaceuticals, Inc., Immune Design Corp., NewLink Genetic Corporation, Advaxis, Inc., Argos Therapeutics, Inc., Biovest International, Inc., ImmunoCellular Therapeutics, Ltd., Immune Design, Inc., Inovio Pharmaceuticals, Inc., Intrexon Corporation and Northwest Biotherapeutics, Inc.

- **Companies developing cell-based immunotherapy approaches:** Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc. (acquired by Gilead Sciences, Inc.), Novartis AG and Pfizer Inc.

Manufacturing

BioXcel Therapeutics does not have manufacturing facilities. We currently rely on strategic manufacturing partners and expect to continue to rely on third parties for the manufacture of our product candidates for clinical research as well as for eventual, possible commercial manufacturing.

For supply of drug substance and drug product for our BXCL501 clinical program, we have secured manufacturing partners. We have completed manufacture of phase III clinical supply and registration batches of our proprietary, sublingual thin film product.

We produce clinical drug product for BXCL701 under exclusivity with the original manufacturers of the active pharmaceutical ingredient, or API, and drug product tablets, respectively.

Manufacturing partners used for both programs currently manufacture commercial products and we consider them to be suitable for commercial supply for our programs, if approved.

Commercialization

We plan to retain our worldwide commercialization rights for some of our key product candidates while for other product candidates we might consider collaboration opportunities to maximize returns.

While we currently have no sales, marketing or commercial product distribution capabilities and have no experience as a company in commercializing products, we intend to build our own commercialization organization and capabilities over time. We are considering partnerships, joint ventures, and a variety of business partnerships for the Japanese and European Markets. We currently plan to retain US rights and are beginning to lay the groundwork for commercialization efforts. When appropriate, we will decide whether to build a specialty sales force to manage commercialization for these product candidates on our own or in combination with a larger pharmaceutical partner to maximize patient coverage in the United States and to support global expansion, especially as our programs have substantial opportunity for additional follow-up indications alone or in combinations.

As product candidates advance through our pipeline, our commercial plans may change. Clinical data, the size of the development programs, the size of the target market, the size of a commercial infrastructure and manufacturing needs may all influence our United States, European Union, and rest-of-world strategies.

Intellectual Property

Our policy is to protect and enhance the proprietary technologies, inventions, and improvements that are commercially important to our business by filing patent applications in the United States and other jurisdictions related to our proprietary technology, inventions, improvements and product candidates. We also rely on trademarks, trade secrets, and know-how relating to our proprietary technologies and product candidates, continuing innovation and in-licensing technology and products. This reliance is expected to develop, strengthen, and maintain our proprietary position for novel therapeutics and novel formulations of existing therapeutics across multiple therapeutic areas. We also plan to rely on data exclusivity, market exclusivity, and patent term extensions when available.

Patent Portfolio

As of February 17, 2020, we have filed applications in the core patent family protecting BXCL501 in the United States, Taiwan and as a pending patent cooperation treaty, or PCT, application.

We have filed applications in the core patent family protecting BXCL501 in the United States, Taiwan and as a pending PCT application. Following entry into national phase, we intend to file for patent protection in major markets, including but not limited to Europe, Japan, China and Canada. We expect that patents issuing from these applications, if any, will expire no earlier than 2039. We have also filed applications in three additional patent families that are relevant to BXCL501. We have applications pending in the United States, Europe and Japan directed to methods of treating insomnia using sublingual Dex. We expect that patents issuing from these applications, if any, will expire no earlier than 2035. We also have applications filed in fifteen countries, including the United States, Europe, Japan and China, directed to methods of treating agitation. We expect that patents issuing from these applications, if any, will expire no earlier than 2037. Finally, we have a PCT application directed to intravenous administration of Dex. We expect that patents issuing from these applications, if any, will expire no earlier than 2039.

We have multiple patent families filed to protect our BXCL701 program, including our core patent family directed to methods of using BXCL701 with immune checkpoint inhibitors, which is filed in the United States and seventeen other countries. Any patents issuing from that family should expire no earlier than 2036. We have a PCT application directed to combination therapies using BXCL701 with immune checkpoint inhibitors and approaches for modifying T-cell activity. We expect any patents issuing from this family to expire no earlier than 2038. Additional PCT and ex-US applications are directed to administering BXCL701 in combinations with various other molecules and dosing regimens. We expect that patents issuing from these applications, if any, will expire no earlier than 2039. Finally, we have four provisional applications directed to various dosing regimens and combination therapies. Any patents issuing from those applications are expected to expire between 2039 and 2041 at the earliest.

We have filed patent applications to protect our proprietary drug programs in immuno-oncology, CNS and agitation. This encompasses our proprietary drug programs in immuno-oncology, CNS and agitation. These proprietary products and methods of use are covered in three separate PCT applications, four pending national phase applications and three pending United States provisional applications to date. However, we intend to file national phase patent applications in all other major counties (Europe, Canada, Japan, Australia and China) in the future. The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. Depending upon the timing, duration and specifics of FDA approval of our product candidates, a United States patent we own or license may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent restoration term of up to five years as compensation for patent term lost during product development and the drug approval regulatory review process. However, patent term restoration 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 one half the time between the effective date of an IND, and the submission date of a NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the application for extension must be made prior to expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration date, depending on the expected length of clinical trials and other factors involved in the submission of the relevant NDA.

The patent positions of companies such as ours are generally uncertain and involve complex legal and factual questions. No consistent policy regarding the scope of claims allowable in patents in the field of method of use patents or reformulation patents has emerged in the United States. The relevant patent laws and their interpretation outside of the United States are also uncertain. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our technology or product candidates and enforce the patent rights that we license, and also could affect the value of such intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell, or importing products that infringe our intellectual property will depend in part on our success in obtaining and enforcing patent claims that cover our technology, inventions, and improvements. With respect to both licensed and company owned intellectual property, we cannot guarantee that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications we may file in the future, nor can we be sure that any patents that may be granted to us in the future will be commercially useful in protecting our products, the methods of use, or the manufacture of those products. Patent and other intellectual property rights in the

pharmaceutical and biotechnology space are evolving and involve many risks and uncertainties. For example, third parties may have blocking patents that could be used to prevent us from commercializing our product candidates and practicing our proprietary technology, and the issued patents that we in license and those that may issue in the future may be challenged, invalidated, or circumvented, which could limit our ability to stop competitors from marketing related products or could limit the term of patent protection that otherwise may exist for our product candidates. In addition, the scope of the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies that are outside the scope of the rights granted under any issued patents that we own or exclusively in license. For these reasons, we may face competition with respect to our product candidates. Moreover, because of the extensive time required for development, testing, and regulatory review of a potential product, it is possible that, before any particular product candidate can be commercialized, any patent protection for such product may expire or remain in force for only a short period following commercialization, thereby reducing the commercial advantage the patent provides.

Our Relationship with BioXcel Corporation

We are currently a 45.5% owned subsidiary of BioXcel and our pipeline compounds have been identified by applying BioXcel's R&D engine, EvolverAI, for drug re-innovation.

The Company has entered into an Amended and Restated Asset Contribution Agreement, pursuant to which BioXcel agreed to provide its rights, title and interest in BXCL501, BXCL701, BXCL502 and BXCL702, collectively, the "Candidates" or the BTI Business and all of the assets and liabilities associated to the Company in consideration for (i) 9,480,000 shares of our common stock, (ii) $1 million upon completion of our initial public offering, or IPO, (iii) $500,000 upon the later of the 12 month anniversary of an initial public offering and the first dosing of a patient in the bridging bioavailability/ bioequivalence study for the BXCL501 program, (iv) $500,000 upon the later of the 12 month anniversary of an initial public offering and the first dosing of a patient in the Phase 2 proof-of-concept open label monotherapy or combination trial with Keytruda for the BXCL701 program and (v) a one-time payment of $5,000,000 within 60 days after the achievement of $50,000,000 in cumulative net sales of any product or combination of products resulting from the development and commercialization of any one of the Candidates or a product derived therefrom. With the completion of the Company's IPO in March 2018, $1 million was charged to Research and Development costs in connection with (ii) above and was paid on April 5, 2018. The Company paid $500,000 to BioXcel in connection with (iii) above in April 2019. In July 2019, the Company completed the first dosing of a patient in the combination trial of BXCL701 with Keytruda, and as a result the Company paid $500,000 to BioXcel in connection with (iv) above in July 2019.

We entered into a Separation and Shared Services Agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017 and March 6, 2020, or the Services Agreement, pursuant to which BioXcel agreed to allow us to continue to use its office space, equipment, services and leased employees based on the agreed upon terms and conditions for a payment of defined monthly and/or hourly fees. The office space and equipment portion of the Services Agreement ended effectively on April 30, 2018 when the Company moved to new office space to accommodate additional personnel that had been hired. Services to be provided by BioXcel through its subsidiary in India, were originally expected to decrease through June 30, 2019. Services provided by BioXcel through its subsidiaries in India and the United States will continue indefinitely, as agreed upon by the parties. These services are primarily for drug discovery and for chemical, manufacturing and controls cost.

Under the Services Agreement, we have an option, exercisable until December 31, 2020, to enter into a collaborative services agreement with BioXcel pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. The parties are obligated to negotiate the collaborative services agreement in good faith and to incorporate reasonable market-based terms, including consideration for BioXcel reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestones shall not exceed $10 million in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestones shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30 million in the aggregate. BioXcel shall continue to make such product identification and related services available to us for at least five years from June 30, 2017. The parties are currently

discussing extending the product identification and related services that BioXcel would provide under the collaborative services agreement, however as of the date hereof we have not reached a definitive agreement.

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, packaging, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our product candidates. A new drug must be approved by the FDA through the NDA process before it may be legally marketed in the United States.

U.S. Government Regulation of Drug Products

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 product candidates may be marketed in the United States generally involves the following:

- completion of extensive preclinical laboratory tests and preclinical animal studies, all performed in accordance with the FDA's good laboratory practice, or GLP, requirements and other applicable regulations;
- submission to the FDA of an IND which must become effective before human clinical trials may begin and must be updated annually;
- approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice, or GCP, requirements to establish the safety and efficacy of the product candidate for each proposed indication;
- submission to the FDA of an NDA after completion of all pivotal clinical trials;
- a determination by the FDA within 60 days of its receipt of an NDA file the application for review;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facilities at which the API and finished drug product are produced and tested to assess compliance with cGMP regulations 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 GCPs and the integrity of the clinical data;

- payment of user fees and securing FDA approval of the NDA to permit commercial marketing of the product for particular indications for use in the United States; and

- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess potential safety and efficacy. Prior to beginning the first clinical trial with a product candidate in the United States, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human studies. The IND also includes results of preclinical studies and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. An IND will automatically become effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to the proposed clinical trials. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before clinical trials can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical trials to commence.

Clinical trials involve the administration of the new investigational drug to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND.

Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical trials and clinical trial results to public registries.

The clinical investigation of a drug is generally divided into three phases. Although the phases are usually conducted sequentially, they may overlap or be combined. The three phases of an investigation are as follows:

- **Phase 1.** Phase 1 includes the initial introduction of an investigational new drug into humans. Phase 1 clinical trials are typically closely monitored and may be conducted in patients with the target disease or condition or in healthy volunteers. These studies are designed to evaluate the safety, dosage tolerance, metabolism and pharmacologic actions of the investigational drug in humans, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness.

- **Phase 2.** Phase 2 includes controlled clinical trials conducted to preliminarily or further evaluate the effectiveness of the investigational drug for a particular indication(s) in patients with the disease or condition under study, to determine dosage tolerance and optimal dosage, and to identify possible adverse

side effects and safety risks associated with the drug. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.

- **Phase 3.** Phase 3 clinical trials are generally controlled clinical trials conducted in an expanded patient population generally at geographically dispersed clinical trial sites. They are performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and are intended to further evaluate dosage, clinical effectiveness and safety, to establish the overall benefit-risk relationship of the investigational drug product, and to provide an adequate basis for product approval.

A registrational or pivotal study is any clinical study, which is designed to meet regulatory agency requirements for the evaluation of a product candidate's efficacy and safety such that it can be used to justify the approval of the product. Generally, registrational or pivotal studies are Phase 3 studies but may also be Phase 2 studies if the trial design provides a well-controlled and reliable assessment of clinical benefit and risk, particularly in situations where there is an unmet medical need.

Post-approval trials, sometimes referred to as Phase 4 studies, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

The FDA or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients 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. In addition, some clinical trials are overseen by an independent group of qualified experts organized by the sponsor, known as a data safety monitoring board or committee. Depending on its charter, this group may determine whether a trial may move forward at designated check points based on access to certain data from the trial.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice, and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors typically use the meetings at the end of the Phase 2 trial to discuss Phase 2 clinical results and present plans for the pivotal Phase 3 clinical trials that they believe will support approval of the new drug. Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. In addition, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

While the IND is active and before approval, progress reports summarizing the results of the clinical trials and nonclinical studies performed since the last progress report must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies suggesting a significant risk to humans exposed to the same or similar drugs, findings from animal or in vitro testing suggesting a significant risk to humans, and any clinically important increased incidence of a serious suspected adverse reaction compared to that listed in the protocol or investigator brochure.

U.S. Review and Approval Process

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational drug product information is submitted to the FDA in the form of an NDA requesting approval to market the product for one or more indications.

The application includes all relevant data available from pertinent preclinical and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product to the satisfaction of the FDA.

The submission of an NDA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances. Additionally, no user fees are assessed on NDAs for products designated as orphan drugs, unless the product also includes a non-orphan indication. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use and whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality and purity. 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 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, that 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 will typically 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 GCP requirements. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates the NDA and conducts inspections of manufacturing facilities where the drug product and/or its API will be produced, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete, and the application is not ready for approval. A Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical trial(s), and/or other significant, expensive and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the sponsor must resubmit the NDA or, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such additional information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. The FDA could also approve the NDA with a REMS plan to mitigate risks. A REMS plan is a safety strategy to manage a known or potential serious risk associated with a medicine and to enable patients to have continued access to such medicines by managing their safe use, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling, development of adequate controls and specifications, or a commitment to conduct one or more post market studies or clinical trials. Such post market testing may include Phase 4 clinical trials and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. Regulatory approval of oncology products often requires that patients in clinical trials be followed for long periods to determine the overall survival benefit of the drug. The FDA may prevent or limit further marketing of a product based on the results of post marketing studies or surveillance programs. In addition, new government

requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could impact the timeline for regulatory approval or otherwise impact ongoing development programs.

In addition, under the Pediatric Research Equity Act of 2003, or PREA, as amended and reauthorized, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A deferral may be granted for several reasons, including a finding that the drug is ready for approval for use in adults before pediatric clinical trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric clinical trials begin. The FDA must send a non-compliance letter to any sponsor that fails to submit the required assessment, keep a deferral current or fails to submit a request for approval of a pediatric formulation.

Orphan Drug Designation and Exclusivity

The Orphan Drug Act provides incentives for the development of products intended to treat rare diseases or conditions. Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation, however, does not convey any advantage in or shorten the duration of the regulatory review and approval process.

Orphan drug designation provides manufacturers with research grants, tax credits and eligibility for orphan drug exclusivity. If a product that has orphan drug designation subsequently receives the first FDA approval of the active moiety for that disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which for seven years prohibits the FDA from approving another product with the same active ingredient for the same indication, except in limited circumstances. If a drug designated as an orphan product receives marketing approval for an indication broader than the orphan drug indication for which it received the designation, it will not be entitled to orphan drug exclusivity. Orphan drug 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 drug 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 drug product has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug product has exclusivity. As a result, even if one of our product candidates receives orphan exclusivity, we may still be subject to competition. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug or if our product candidate is determined to be contained within the competitor's product for the same indication or disease.

Expedited development and review programs

The FDA has a fast track designation program that is intended to expedite or facilitate the process for reviewing new drug products that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. With regard to a fast track product, the FDA may consider for review sections of the NDA 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.

Any product submitted to the FDA for approval, including a product with a fast track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review. The FDA endeavors to review applications with priority review designations within six months of the filing date as compared to ten months for review of new molecular entity NDAs under its current PDUFA review goals.

In addition, a product may be eligible for accelerated approval. Drug products intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination that the 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, 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 that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. Drugs receiving accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails to perform required post-marketing trials or if such trials fail to verify the predicted clinical benefit. In addition, the FDA currently requires pre-approval of promotional materials as a condition for accelerated approval, which could adversely impact the timing of the commercial launch of the product.

The Food and Drug Administration Safety and Innovation Act established a category of drugs referred to as "breakthrough therapies" that may be eligible to receive breakthrough therapy designation. A sponsor may seek FDA designation of a product candidate as a "breakthrough therapy" if the product is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance. The breakthrough therapy designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met. If a product is designated as breakthrough therapy, the FDA will work to expedite the development and review of such drug.

Fast track designation, breakthrough therapy designation and priority review and do not change the standards for approval but may expedite the development or approval process. 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.

Post-Approval Requirements

After regulatory approval of a drug product is obtained, we will be required to comply with a number of post approval requirements. As a holder of an approved NDA, we would be required to report, among other things, certain adverse reactions and production problems to the FDA, to provide updated safety and efficacy information, and to comply with requirements concerning advertising and promotional labeling for any of our products. 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 also are continuing, annual program fees for any marketed products. Also, quality control and manufacturing procedures must continue to conform to cGMP after approval to ensure and preserve the long term stability of the drug product. 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. In addition, changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

The FDA may withdraw 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 revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions 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 untitled letters;
- clinical holds on 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; consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- 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 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. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Marketing Exclusivity

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 obtain approval of an NDA for a new chemical entity. A drug is a new chemical 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 abbreviated new drug application, or 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 an NDA, 505(b)(2) NDA or supplement to an approved 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, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modifications associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active pharmaceutical ingredient. 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.

Pediatric exclusivity is another type of marketing exclusivity available in the United States. Pediatric exclusivity provides for an additional six months of marketing exclusivity attached to another period of exclusivity if a sponsor conducts clinical trials in children in response to a written request from the FDA. The issuance of a written request does not require the sponsor to undertake the described clinical trials. In addition, orphan drug exclusivity, as described above, may offer a seven-year period of marketing exclusivity, except in certain circumstances.

The Hatch-Waxman Amendments: 505(b)(2) Approval Process

Section 505(b)(2) of the FDCA provides an alternate regulatory pathway to FDA approval for new or improved formulations or new uses of previously approved drug products. Specifically, Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon the FDA's findings of safety and effectiveness for an approved product that acts as the Reference Listed Drug, or RLD. If the 505(b)(2) applicant can establish that reliance on FDA's previous findings of safety and effectiveness is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require 505(b)(2) applicants to perform additional studies or measurements to support the change from the RLD. The FDA may then approve the new product candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

In seeking approval for a drug through an NDA, including a 505(b)(2) NDA, applicants are required to list with the FDA certain patents whose claims cover the applicant's product. Upon approval of an NDA, each of the patents listed in the application for the drug is then published in the Orange Book. Any applicant who files an ANDA seeking approval of a generic equivalent version of a drug listed in the Orange Book or a 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (i) no patent information on the drug product that is the subject of the application has been submitted to the FDA; (ii) such patent has expired; (iii) the date on which such patent expires; or (iv) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a paragraph IV certification. A notice of the paragraph IV certification must be provided to each owner of the patent that is the subject of the certification and to the holder of the approved NDA to which the ANDA or 505(b)(2) application refers. The applicant may also elect to submit a "section viii" statement certifying that its proposed label does not contain or carves out any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the reference NDA holder and patent owners assert a patent challenge directed to one of the Orange Book listed patents within 45 days of the receipt of the paragraph IV certification notice, the FDA is prohibited from approving the application until the earlier of 30 months from the receipt of the paragraph IV certification expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the applicant. The ANDA or 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the branded reference drug has expired.

International Regulations

In addition to regulations in the United States, we are and will be subject to a variety of foreign regulations regarding development, approval, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional review periods, and the time may be longer or shorter than that required to obtain FDA approval. The requirements governing, among other things,

the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others. If we fail to comply with applicable foreign regulatory requirements, we may be subject to fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

To obtain regulatory approval of an investigational drug under European Union regulatory systems, we must submit a marketing authorization application. The application used to file the NDA in the United States is similar to that required in Europe, with the exception of, among other things, country-specific document requirements. For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Centralized Procedure

In the European Economic Area (EEA), which is comprised of the 27 EU member states plus Iceland, Liechtenstein, and Norway, and the United Kingdom (until the end of the transition period on 31 December, 2020 provided for in the Withdrawal Agreement between the EU and the UK), the European Medicines Agency, or EMA, has responsibility for reviewing applications for the approval of human medicines under the Centralized Procedure. This procedure results in a single marketing authorization issued by the European Commission following a favorable opinion by the EEA. The centralized procedure is compulsory for human medicines that are derived from biotechnology processes, advanced therapy medicinal products (such as gene therapy, somatic cell therapy and tissue engineered products), and medicinal products that contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions, and officially designated orphan medicines. For medicines that do not fall within these categories, an applicant has the option of submitting an application for a centralized marketing authorization to the EMA, 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.

There are also two other possible routes to authorize medicinal products in several EEA countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure the decentralized procedure and the mutual recognition procedure. Under the decentralized procedure, an applicant may apply for simultaneous authorization in more than one EEA country for medicinal products that have not yet been authorized in any EEA country. Under the mutual recognition procedure, a medicine is first authorized in one EEA member state, in accordance with the national procedures of that country. Following a national authorization, the applicant may seek further marketing authorizations from other EEA countries under a procedure whereby the countries concerned agree to recognize the validity of the original national marketing authorization.

In the EEA, medicinal products designated as orphan drug products benefit from financial incentives such as reductions in marketing authorization application fees or fee waivers and 10 years of marketing exclusivity following medicinal product approval. For a medicinal product to qualify as orphan drugs (i) it must be intended for the treatment, prevention or diagnosis of a disease that is life threatening or chronically debilitating; (ii) the prevalence of the condition in the EU must not be more than five in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development; and (iii) no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorized, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

Accelerated Review

Based on results of the Phase 3 clinical trial(s) submitted in an NDA, upon the request of an applicant, the FDA may grant the NDA a priority review designation, which sets the target date for FDA action on the application at six months. Priority review is granted where preliminary estimates indicate that a product, if approved, has the potential to provide a safe and effective therapy where no satisfactory alternative therapy exists, or a significant improvement compared to marketed products is possible. If criteria are not met for priority review, the NDA is subject to the standard FDA review period of 10 months. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the Centralized Procedure in the EEA, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the Committee for Medicinal Product for Human Use or CHMP, accelerated evaluation might be granted by the CHMP in exceptional cases when a medicinal product is expected to be of a major public health interest, defined by three cumulative criteria: the seriousness of the disease (e.g. heavy disabling or life-threatening diseases) to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In this circumstance, EMA ensures that the opinion of the CHMP is given within 150 days, excluding clock stops.

There can be no assurance that we or any of our partners would be able to satisfy one or more of these requirements to conduct preclinical or clinical trials or receive any regulatory approvals.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the U.S. government enacted legislation providing a partial prescription drug benefit for Medicare beneficiaries, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. These plans will likely negotiate discounted prices for our products.

Further, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or ACA, was enacted and the Healthcare Reform Law substantially changes the way healthcare is financed in the United States by both government and private insurers. Among other cost containment measures, the ACA established:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents;

- a new Medicare Part D coverage gap discount program, in which pharmaceutical manufacturers who wish to have their drugs covered under Part D must offer discounts to eligible beneficiaries during their coverage gap period, or the "donut hole"; and

- a new formula that increases the rebates a manufacturer must pay under the Medicaid Drug Rebate Program.

Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA. For example, the Tax Cuts and Jobs Act, or the Tax Act, was enacted in 2017, which, among other things, removes penalties for not complying with the Affordable Care Act's individual mandate to carry health insurance. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the Affordable Care Act, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the Affordable Care Act are invalid as well. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. It is unclear how these decisions, subsequent appeals, if any, or other efforts to challenge, repeal or replace the Affordable Care Act will impact the law, or our business or financial condition. In addition, we expect that federal, state and local governments in the United States will continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. Future legislation could limit payments for pharmaceuticals such as the product candidates that we are developing.

Different pricing and reimbursement schemes exist in other countries. In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, new products are facing increasingly high barriers to entry. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

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. In addition, an increasing emphasis on managed care 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 secured 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 order to raise sufficient financial resources to continue to advance our product candidates, we will need to address pricing pressures and potential third-party reimbursement coverage for our product candidates. In the United States and elsewhere, sales of pharmaceutical products depend in significant part on the availability of reimbursement to the consumer from third party payors, such as government and private insurance plans. Third party payors are increasingly challenging the prices charged for medical products and services. It is and will continue to be time consuming and expensive for us or our strategic collaborators to go through the process of seeking reimbursement from Medicare and private payors. Our products may not be considered cost effective, and coverage and reimbursement may not be available or sufficient to allow us to sell our products on a competitive and profitable basis.

Other Healthcare Laws and Compliance Requirements

If we obtain regulatory approval for any of our product candidates, we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. For example, in the United States, there are federal and state anti-kickback laws that prohibit the payment or receipt of kickbacks, bribes or other remuneration intended to induce the purchase or recommendation of healthcare products and services or reward past purchases or

recommendations. Violations of these laws can lead to civil and criminal penalties, including fines, imprisonment and exclusion from participation in federal healthcare programs.

The federal Anti Kickback Statute prohibits persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce either the referral of an individual, or the furnishing, recommending, or arranging for a good or service, for which payment may be made under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. Many states have adopted laws similar to the federal Anti Kickback Statute, some of which apply to the referral of patients for healthcare items or services reimbursed by any source, not only the Medicare and Medicaid programs.

The federal False Claims Act imposes liability on any person who, among other things, knowingly presents, or causes to be presented, a false or fraudulent claim for payment by a federal healthcare program. In addition, 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. The "qui tam" provisions of the False Claims Act allow a private individual to bring civil actions on behalf of the federal government alleging that the defendant has submitted a false claim to the federal government, and to share in any monetary recovery. In addition, various states have enacted false claims laws analogous to the False Claims Act. Many of these state laws apply where a claim is submitted to any third party payer and not merely a federal healthcare program. When an entity is determined to have violated the False Claims Act, it may be required to pay up to three times the actual damages sustained by the government, plus significant civil penalties for each separate false claim.

Also, the Health Insurance Portability and Accountability Act of 1996, or HIPAA, created several new federal crimes, including healthcare fraud, and false statements relating to healthcare matters. The health care fraud statute prohibits knowingly and willfully executing a scheme to defraud any health care benefit program, including private third-party payers. The false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services.

Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation.

The Physician Payment Sunshine Act, or the Sunshine Act, which was enacted as part of the ACA, requires applicable manufacturers of drugs, devices, biologicals, or medical supplies covered under Medicare, Medicaid or the Children's Health Insurance Program, to report annually to the Secretary of the Department of Health and Human Services payments or other transfers of value made by that entity, or by a third party as directed by that entity, to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare providers starting in 2022, and teaching hospitals, or to third parties on behalf of such providers, during the course of the preceding calendar year. Failure to comply with the reporting requirements can result in significant civil monetary penalties for any payment or other transfer of value that is not reported.

Violations of any of these laws or any other governmental laws and regulations that may apply include, without limitation, significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

In addition, we may be subject to data privacy and security laws, including, without limitation, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, which established uniform standards for certain "covered entities" (healthcare providers, health plans and healthcare clearinghouses) and their business associates governing the conduct of certain electronic healthcare transactions and protecting the security and privacy of protected health information. In addition, certain state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways, thus complicating compliance efforts. For example, California recently enacted legislation, the California Consumer Privacy Act, or CCPA, which went into effect January 1, 2020. The CCPA, among other things,

creates new data privacy obligations for covered companies and provides new privacy rights to California residents, including the right to opt out of certain disclosures of their information. The CCPA also creates a private right of action with statutory damages for certain data breaches, thereby potentially increasing risks associated with a data breach. Although the law includes limited exceptions, including for "protected health information" maintained by a covered entity or business associate, it may regulate or impact our processing of personal information depending on the context.

Employees

As of December 31, 2019, we employed a total of 24 full-time employees. In addition, we have access to certain BioXcel employees and resources through various agreements we have entered into with BioXcel. We are not a party to any collective bargaining agreements. We believe that we maintain good relations with our employees and also that of the Parent.

Our Corporate Information

We were incorporated as a Delaware corporation on March 29, 2017 as a wholly-owned subsidiary of BioXcel. Our principal executive offices are located at 555 Long Wharf Drive, New Haven, CT 06511 and our telephone number is (475) 238-6837.

Available Information

Our website address is www.bioxceltherapeutics.com. The contents of, or information accessible through, our website are not part of this Annual Report on Form 10-K. We make our filings with the SEC, including our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and all amendments to those reports, available free of charge on our website as soon as reasonably practicable after we file such reports with, or furnish such reports to, the SEC. Our website and the information contained on or through that site are not incorporated into this Annual Report on Form 10-K.

Item 1A. Risk Factors

You should carefully consider the risks described below, as well as general economic and business risks and the other information in this Annual Report on Form 10-K. The occurrence of any of the events or circumstances described below or other adverse events could have a material adverse effect on our business, results of operations and financial condition and could cause the trading price of our common stock to decline. Additional risks or uncertainties not presently known to us or that we currently deem immaterial may also harm our business.

Risks Related to Financial Position and Need for Additional Capital

We have a limited operating history and have never generated any product revenues, which may make it difficult to evaluate the success of our business to date and to assess our future viability.

We were incorporated in March 2017 and our operations to date have been largely focused on staffing our company, raising capital and advancing the development of, our product candidates, including conducting clinical and preclinical studies. We have not yet demonstrated an ability to successfully obtain marketing approvals, manufacture products on a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We will need to eventually transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

Since our inception, we have incurred significant operating losses. Our net loss was $33.0 million and $19.3 million for the years ended December 31, 2019 and 2018 respectively. As of December 31, 2019, we had stockholders' equity of $26.9 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. None of our product candidates have been approved for marketing in the United States, or in any other jurisdiction, and may never receive such approval. It could be several years, if ever, before we have a commercialized product that generates significant revenues. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue the development of our product candidates;
- conduct preclinical studies and clinical trials for our current product candidates and any future product candidates that we may pursue;
- continue to build our portfolio of product candidates through the acquisition or in-license of additional product candidates or technologies;
- continue to develop, maintain, expand and protect our intellectual property portfolio;
- pursue regulatory approvals for our current and future product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any product candidate for which we may obtain marketing approval;
- hire additional clinical, regulatory, scientific and accounting personnel; and
- incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing clinical trials of our product candidates, developing commercial scale manufacturing processes, obtaining marketing approval, manufacturing, marketing and selling any current and future product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate sufficient revenue to achieve profitability.

Because of the numerous risks and uncertainties associated with product development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will obtain marketing approval to commercialize any of our product candidates. If we are required by the U.S. Food and Drug Administration, or FDA, or other regulatory authorities such as the European Medicines Agency, or EMA, to perform studies and trials in addition to those currently expected, or if there are any delays in the development, or in the completion of any planned or future preclinical studies or clinical trials of our current or future product candidates, our expenses could increase and profitability could be further delayed.

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, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

Our recurring losses from operations raise substantial doubt regarding our ability to continue as a going concern.

We currently operate with limited resources. We have incurred significant losses since our inception and have never generated revenue or profit, and it is possible we will never generate revenue or profit. For information about our liquidity and capital resources, see Part II, Item 7 "Management's Discussion and Analysis of Financial Condition and Results of Operations — Liquidity and Capital Resources." There can be no assurances that additional financing will be available to us on satisfactory terms, or at all.

Securing additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize any of our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If we are unable to obtain funding, we would be forced to delay, reduce or eliminate our research and development programs, which would adversely affect our business prospects. In addition, if we are unable to raise capital, we will also need to implement cost reductions, and any failure to effectively do so will harm our business, results of operations and future prospects. The perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations. If we are unable to continue as a going concern, investors could lose all or part of their investment in our Company.

We will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We anticipate that our expenses will increase substantially if and as we continue to develop and conduct clinical trials with respect to BXCL501, BXCL701 and our other product candidates; seek to identify and develop additional product candidates; acquire or in-license other product candidates or technologies; seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any; require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization; maintain, expand and protect our intellectual property portfolio; hire and retain additional personnel, such as clinical, quality control and scientific personnel; add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and add equipment and physical infrastructure to support our research and development programs.

We expect that our current cash and cash equivalents will be used primarily to fund our ongoing research and development efforts over the coming months. We will be required to expend significant funds in order to advance the development of BXCL501, BXCL701 and our other product candidates. In addition, while we may seek one or more collaborators for future development of our current product candidate or any future product candidates that we may develop for one or more indications, we may not be able to enter into a collaboration for any of our product candidates for such indications on suitable terms, on a timely basis or at all. In any event, the net proceeds of our IPO, our September 2019 and February 2020 offerings of common stock and our existing cash will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of our product candidates or our other preclinical programs. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Further financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

Our estimate as to how long we expect our existing cash to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials of BXCL501, BXCL701 and our other product candidates;
- our ability to enter into and the terms and timing of any collaborations, licensing agreements or other arrangements;
- the costs, timing and outcome of seeking regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- our headcount growth and associated costs as we expand our research and development as well as potentially establish a commercial infrastructure;
- revenue received from commercial sales, if any, of our current and future product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the number of future product candidates that we pursue and their development requirements;
- changes in regulatory policies or laws that may affect our operations;
- changes in physician acceptance or medical society recommendations that may affect commercial efforts;
- the costs of acquiring potential new product candidates or technology; and
- the costs of operating as a public company.

Risks Related to the Discovery and Development of Product Candidates

We have limited experience in drug discovery and drug development, and we have never had a drug approved.

Prior to the acquisition of our product candidates, we were not involved in and had no control over their preclinical and clinical development. In addition, we are relying upon the parties we have acquired our product candidates from to have conducted such research and development in accordance with the applicable protocol, legal, regulatory and scientific standards, having accurately reported the results of all clinical trials conducted prior to our acquisition of the applicable product candidate, and having correctly collected and interpreted the data from these studies and trials. To the extent any of these has not occurred, our expected development time and costs may be increased, which could adversely affect our prospects for marketing approval of, and receiving any future revenue from, these product candidates.

In the near term, we are dependent on the success of BXCL501 and BXCL701. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize BXCL501, BXCL701 and our other product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.

We currently do not have any products that have received regulatory approval and may never be able to develop marketable product candidates. We are investing a significant portion of our efforts and financial resources in the development of BXCL501, BXCL701 and our other product candidates. Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop, obtain marketing approval for and successfully commercialize product candidates in one or more disease indications.

The success of BXCL501, BXCL701 and our other product candidates will depend on several factors, including the following:

- acceptance of an IND application by the FDA authorizing us to conduct clinical trials of our product candidates in the United States;
- initiation, progress, timing, costs and results of clinical trials of our product candidates and potential product candidates;
- demonstration of safety and efficacy of our product candidates to the satisfaction of the FDA or any comparable foreign regulatory authority and sufficient for marketing approval;
- the timing and performance of our current and future collaborators;
- the nature of any required post-marketing clinical trials or other commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party raw materials suppliers and manufacturers;
- establishment of arrangements with third-party manufacturers to obtain finished drug product that is appropriately packaged for sale;
- adequate ongoing availability of raw materials and drug product for clinical development and any commercial sales;
- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payors; and
- our ability to compete with other therapies.

Many of these factors are beyond our control, including the results of clinical trials, the time required for the FDA or any comparable foreign regulatory authorities to review any regulatory submissions we may make, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive marketing approval for and successfully commercialize BXCL501, BXCL701 and our other product candidates, on our own or with any future collaborator, or experience delays as a result of any of these factors or otherwise, our business could be substantially harmed.

Interim "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose top-line or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the top-line or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once

additional data have been received and fully evaluated. Top-line or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the top-line or preliminary data we previously published. As a result, top-line and preliminary data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our preclinical studies and clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between interim data and final data could significantly harm our business prospects. Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our common stock.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure.

If the interim, top-line or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

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

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. It is not uncommon for companies in the biopharmaceutical industry to suffer significant setbacks in advanced clinical trials due to nonclinical findings made while clinical studies were underway and safety or efficacy observations made in clinical studies, including previously unreported adverse events. Our future clinical trial results may not be successful, and notwithstanding any potential promising results in earlier studies, we cannot be certain that we will not face similar setbacks. The historical failure rate for product candidates in our industry is high. 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. We have not 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.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an New Drug Application, or NDA, or other submission or to obtain regulatory approval in the United States or elsewhere; the FDA or comparable foreign regulatory authorities may disagree that our changes to branded reference drugs meet the criteria for the 505(b)(2) regulatory pathway or foreign regulatory pathways;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

We have limited experience in completing clinical trials of any of our product candidates. Consequently, we may not have the necessary capabilities, including adequate staffing, to successfully manage the execution and completion of any clinical trials we initiate in a way that leads to our obtaining marketing approval for our product candidates in a timely manner, or at all. This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate or may restrict its distribution. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

We have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patients that we are targeting for our product candidates are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

We plan to seek regulatory approval to commercialize our product candidates both in the United States, the European Union and in additional foreign countries. While the scope of regulatory approval is similar in other countries, to obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales, pricing and distribution of our product candidates, and we cannot predict success in these jurisdictions.

Clinical trials are expensive, time-consuming and difficult to design and implement, and involve an uncertain outcome

Before obtaining marketing approval from the FDA or other comparable foreign regulatory authorities for the sale of our product candidates, we must complete preclinical development and extensive clinical trials to demonstrate the safety and efficacy of our product candidates. 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. Although we are planning for certain clinical trials relating to BXCL501, BXCL701 and our other product candidates, there can be no assurance that the FDA will accept our proposed trial designs. We may experience delays in our clinical trials and we do not know whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays related to:

- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical studies;
- obtaining regulatory authorizations to commence a trial or consensus with regulatory authorities on trial designs;

- 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 trial sites;
- obtaining institutional review board, or IRB, approval at each site, or independent ethics committee, or IEC, approval at any sites outside the United States;
- dependence on the needs and timing of third party collaborators;
- changes to clinical trial protocols;
- recruiting suitable patients to participate in a trial in a timely manner and in sufficient numbers;
- clinical sites deviating from trial protocol or dropping out of a trial;
- addressing patient safety concerns that arise during the course of a trial;
- having patients complete a trial or return for post-treatment follow-up;
- imposition of a clinical hold by regulatory authorities, including as a result of unforeseen safety issues or side effects or failure of trial sites to adhere to regulatory requirements;
- the occurrence of serious adverse events in trials of the same class of agents conducted by other companies or institutions;
- subjects choosing an alternative treatment for the indications for which we are developing our product candidates, or participating in competing trials;
- adding a sufficient number of clinical trial sites;
- manufacturing sufficient quantities of a product candidate for use in clinical trials;
- lack of adequate funding to continue the clinical trial;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practice, or cGMP, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process that may be necessary or desired;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, good clinical practice, or GCP, or other regulatory requirements; or
- third-party contractors not performing data collection or analysis in a timely or accurate manner; or third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination 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 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 drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and, while we have agreements governing their committed activities, we have limited influence over their actual performance,

Further, conducting clinical trials in foreign countries, as we may do for our current and future product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

We depend on enrollment of patients in our clinical trials in order for us to continue development of our product candidates. If we are unable to enroll patients in our clinical trials, our research and development efforts could be adversely affected.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. Patient enrollment is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, the size of the patient population required for analysis of the trial's primary endpoints, the proximity of patients to study sites, our ability to recruit clinical trial investigators with the appropriate competencies and experience, our ability to obtain and maintain patient consents, the risk that patients enrolled in clinical trials will drop out of the trials before completion, and competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. Many pharmaceutical companies are conducting clinical trials in patients with the disease indications that our potential drug products target. As a result, we must compete with them for clinical sites, physicians and the limited number of patients who fulfill the stringent requirements for participation in clinical trials. Also, due to the confidential nature of clinical trials, we do not know how many of the eligible patients may be enrolled in competing studies and who are consequently not available to us for our clinical trials. Our clinical trials may be delayed or terminated due to the inability to enroll enough patients. The delay or inability to meet planned patient enrollment may result in increased costs and delay or termination of our trials, which could have a harmful effect on our ability to develop products.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

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 or other comparable foreign authorities. The clinical evaluation of BXCL501, BXCL701 and our other product candidates in patients is still in the early stages and it is possible that there may be side effects associated with their use. To date, based on information available in the package insert for Dex, patients treated with Dex have experienced drug-related side effects including hypotension, transient hypertension, bradycardia, dry mouth, acute respiratory distress syndrome, respiratory failure and agitation with hypotension, bradycardia and dry mouth considered serious adverse events. In addition, based on the investigator brochure for Talabostat, patients treated with Talabostat have experienced edema/peripheral swelling, hypotension, dizziness, hypovolemia fatigue, nausea, vomiting, pyrexia rigors and rash with edema and fatigue representing the most frequently observed serious adverse events. Results of our trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In our Phase 2 clinical trial for the treatment of emergent Neuroendocrine Prostate Cancer, one patient experienced acidosis with a fatal outcome. Although the clinical investigator could not determine that the fatality was related to treatment with BXCL701, it is possible that BXCL701 could be tied to unacceptable side effects in the future. In such an event, we, the FDA, the IRBs at the institutions in which our studies are conducted, or the DSMB could suspend or terminate our clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. For example, the FDA placed Point Therapeutics, Inc.'s IND for BXCL701 on clinical hold following an increase in observed mortality in patients receiving BXCL701 in a Phase 3 trial in patients with non-small cell lung cancer. Though we believe that this result was caused by, among other things, an imbalance in the disease severity of patients enrolled in the active arm of the clinical trial, there is no guarantee that excess mortality

will not be observed in future clinical studies. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the clinical trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such products;
- we may be required to recall a product or change the way such a product is administered to patients;
- additional restrictions may be imposed on the marketing or distribution of the particular product or the manufacturing processes for the product or any component thereof;
- regulatory authorities may require additional warnings on the label, such as a “black box” warning or contraindication;
- we may be required to implement Risk Evaluation and Mitigation Strategies, or REMS, or create a medication guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients;
- our product may become less competitive; and
- our reputation may suffer.

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

BioXcel’s approach to the discovery and development of product candidates based on EvolverAI is novel and unproven, and we do not know whether we will be able to develop any products of commercial value.

We are leveraging EvolverAI to create a pipeline of neuroscience and immuno-oncology product candidates for patients whose diseases have not been adequately addressed to date by other approaches and to design and conduct efficient clinical trials with a higher likelihood of success. While we believe that applying EvolverAI to create medicines for defined patient populations may potentially enable drug research and clinical development that is more efficient than conventional drug research and development, our approach is both novel and unproven. Because our approach is both novel and unproven, the cost and time needed to develop our product candidates is difficult to predict, and our efforts may not result in the discovery and development of commercially viable medicines. We may also be incorrect about the effects of our product candidates on the diseases of our defined patient populations, which may limit the utility of our approach or the perception of the utility of our approach. Furthermore, our estimates of our defined patient populations available for study and treatment may be lower than expected, which could adversely affect our ability to conduct clinical trials and may also adversely affect the size of any market for medicines we may successfully commercialize. Our approach may not result in time savings, higher success rates or reduced costs as we expect it to, and if not, we may not attract collaborators or develop new drugs as quickly or cost effectively as expected and therefore we may not be able to commercialize our approach as originally expected.

EvolverAI may fail to help us discover and develop additional potential product candidates.

Any drug discovery that we are conducting using EvolverAI may not be successful in identifying compounds that have commercial value or therapeutic utility. EvolverAI may initially show promise in identifying potential product candidates, yet fail to yield viable product candidates for clinical development or commercialization for a number of reasons, including:

- research programs to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product candidates. If we are unable to identify suitable additional compounds for preclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price;
- compounds found through EvolverAI may not demonstrate efficacy, safety or tolerability;
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance;
- competitors may develop alternative therapies that render our potential product candidates non-competitive or less attractive; or
- a potential product candidate may not be capable of being produced at an acceptable cost.

We have obtained Fast Track Designation for BXCL501 for the treatment of acute agitation, and we may seek Fast Track designation for other indications or for our other product candidates, but we might not receive such designations, and even if we do, such designations may not actually lead to a faster development or regulatory review or approval process.

If a product is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA Fast Track designation. We have obtained Fast Track designation for BXCL501 for the treatment of acute agitation, and we may seek Fast Track designation for other indications for BXCL701 or for one or more of our other product candidates, but we might not receive such designations from the FDA. However, even if we receive Fast Track designation, Fast Track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with Fast Track designation compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA’s priority review procedures.

If the FDA does not conclude that our product candidates satisfy the requirements for the 505(b)(2) regulatory approval pathway, or if the requirements for approval of any of our product candidates under Section 505(b)(2) are not as we expect, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and encounter significantly greater complications and risks than anticipated, and in any case may not be successful.

We intend to seek FDA approval through the 505(b)(2) regulatory pathway for certain of our product candidates, including BXCL501. The Hatch-Waxman Act added Section 505(b)(2) to the FDCA. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies that were not conducted by or for the applicant. If the FDA does not allow us to pursue the 505(b)(2) regulatory pathway for our product candidates as anticipated, we may need to conduct additional clinical trials, provide additional data and information and meet additional standards for regulatory approval. If this were to occur, the time and financial resources required to obtain FDA approval for our product candidates would likely substantially increase. Moreover, the inability to pursue the 505(b)(2) regulatory pathway could result in new competitive products reaching the market faster than our product candidates, which could materially adversely impact our competitive position and prospects. Even if we are allowed to pursue the 505(b)(2) regulatory pathway for a product candidate, we cannot assure you that we will receive the requisite or timely approvals for commercialization of such product candidate. In addition, we expect that our competitors will file citizens’ petitions with the FDA in an attempt to persuade the FDA that our product candidates, or the clinical studies that support their approval, contain deficiencies. Such actions by our competitors could delay or even prevent the FDA from approving any NDA that we submit under Section 505(b)(2).

Even if we obtain regulatory approval for BCXL501, BCXL701 or any product candidate, we will still face extensive and ongoing regulatory requirements and obligations and any product candidates, if approved, may face future development and regulatory difficulties.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising and promotional activities for such product, among other things, will be subject to extensive and ongoing requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, continued compliance with cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and GCP requirements for any clinical trials that we conduct post-approval.

Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product candidate may be marketed or to the conditions of approval, including a requirement to implement a REMS. If any of our product candidates receives marketing approval, the accompanying label may limit the approved indicated use of the product candidate, which could limit sales of the product candidate. The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. Violations of the Federal Food, Drug, and Cosmetic Act, or FDCA, relating to the promotion of prescription drugs may lead to FDA enforcement actions and investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of products;
- restrictions on product manufacturing, distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Further, the FDA's policies may change, and additional government regulations may be enacted that could impose extensive and ongoing regulatory requirements and obligations on any product candidate for which we obtain marketing approval. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the current presidential administration may impact our business and industry. Namely, the current presidential administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how these executive actions will be implemented, and the extent to which

they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses.

If we are found to have improperly promoted off-label uses of our products or product candidates, if approved, we may become subject to significant liability. Such enforcement has become more common in the industry. The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription drug products, such as our product candidates, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we receive marketing approval for our product candidates for our proposed indications, physicians may nevertheless use our products for their patients in a manner that is inconsistent with the approved label, if the physicians personally believe in their professional medical judgment it could be used in such manner. However, if we are found to have promoted our products for any off-label uses, the federal government could levy civil, criminal and/or administrative penalties, and seek fines against us. The FDA or other regulatory authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We may be subject to extensive regulations outside the United States and may not obtain marketing approvals for products in Europe and other jurisdictions.

In addition to regulations in the United States, should we or our collaborators pursue marketing approvals for BXCL501, BXCL701 and our other product candidates internationally, we and our collaborators will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we, or our collaborators, obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country.

We expect to pursue marketing approvals for BXCL501, BXCL701 and our other product candidates in Europe and other jurisdictions outside the United States with collaborative partners. The time and process required to obtain regulatory approvals and reimbursement in Europe and other jurisdictions may be different from those in the United States regulatory and approval in one jurisdiction does not ensure approvals in any other jurisdiction; however, negative regulatory decisions in any jurisdiction may have a negative impact on the regulatory process in other jurisdictions.

Following a national referendum and enactment of legislation by the government of the United Kingdom, the United Kingdom withdrew from the European Union, or Brexit, on January 31, 2020 and entered into a transition period during which it will continue its ongoing and complex negotiations with the European Union relating to the future trading relationship between the parties. Significant political and economic uncertainty remains about whether the terms of the relationship will differ materially from the terms before withdrawal, as well as about the possibility that a so-called "no deal" separation will occur if negotiations are not completed by the end of the transition period. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

If we are found in violation of federal or state "fraud and abuse" laws, we may be required to pay a penalty and/or be suspended from participation in federal or state health care programs, which may adversely affect our business, financial condition and results of operations.

In the United States, we will be subject to various federal and state health care "fraud and abuse" laws, including anti-kickback laws, false claims laws and other laws intended to reduce fraud and abuse in federal and state health care programs, which could affect us, particularly upon successful commercialization of our products in the United States. These laws include:

- the federal Anti-Kickback Statute 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 the referral of business, including the purchase, order or prescription of a particular drug for which payment may be made under a federal health care program, such as Medicare or Medicaid. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it to have committed a violation. Under federal government regulations, some arrangements, known as safe harbors, are deemed not to violate the federal Anti-Kickback Statute. Although we seek to structure our business arrangements in compliance with all applicable requirements, these laws are broadly written, and it is often difficult to determine precisely how the law will be applied in specific circumstances. Accordingly, it is possible that our practices may be challenged under the federal Anti-Kickback Statute;

- false claims laws prohibit anyone from knowingly and willfully presenting or causing to be presented for payment to third-party payers, including government payers, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. Cases have been brought under false claims laws alleging that off-label promotion of pharmaceutical products or the provision of kickbacks has resulted in the submission of false claims to governmental health care programs. In addition, 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 laws. Further, private individuals have the ability to bring actions on behalf of the government under the federal False Claims Act;

- the Health Insurance Portability and Accountability Act of 1996, or HIPAA, prohibits persons or entities from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services. Similar to the federal Anti- Kickback Statute, a person or entity does not need to have actual knowledge of these statutes or specific intent to violate them to have committed a violation;

- federal civil monetary penalties laws, which impose civil fines for, among other things, the offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program, unless an exception applies;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;

- the federal physician sunshine requirements under the ACA, which requires certain manufacturers of drugs, devices, biologics, and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare providers starting in 2022, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members;

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and pricing information; and state laws governing the privacy and security of health information (or personal information generally) in certain circumstances, many of which differ from each other in significant ways, thus complicating compliance efforts; and

- European and other foreign law equivalents of each of the laws, including reporting requirements detailing interactions with and payments to healthcare providers.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. 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. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and imprisonment, any of which could adversely affect our ability to market our products and adversely impact our financial results.

We may be unable to maintain sufficient clinical trial liability insurance.

Our inability to retain sufficient clinical trial liability insurance at an acceptable cost to protect against potential liability claims could prevent or inhibit our ability to conduct clinical trials for product candidates we develop. We may be unable to obtain appropriate levels of such insurance. Even if we do secure clinical trial liability insurance for our programs, we may not be able to achieve sufficient levels of such insurance. Any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. We expect we will supplement our clinical trial coverage with product liability coverage in connection with the commercial launch of BXCL501, BXCL701 or other product candidates we develop in the future; however, we may be unable to obtain such increased coverage on acceptable terms or at all. If we are found liable in a clinical trial lawsuit or a product liability lawsuit in the future, we will 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.

Risks Related to Commercialization of Our Product Candidates

If our products do not gain market acceptance, our business will suffer because we might not be able to fund future operations.

A number of factors may affect the market acceptance of our products or any other products we develop or acquire, including, among others:

- the price of our products relative to other products for the same or similar treatments;
- the perception by patients, physicians and other members of the health care community of the effectiveness and safety of our products for their indicated applications and treatments;
- our ability to fund our sales and marketing efforts; and
- the effectiveness of our sales and marketing efforts.

If our products do not gain market acceptance, we may not be able to fund future operations, including developing, testing and obtaining regulatory approval for new product candidates and expanding our sales and marketing efforts for our approved products, which would cause our business to suffer.

We have been granted Orphan Drug Designation for BXCL701 for the treatment of pancreatic cancer, melanoma and acute myeloid leukemia and may seek Orphan Drug Designation for other indications or product candidates, and we may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity, and may not receive Orphan Drug Designation for other indications or for our other product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs intended for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. 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. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the United States provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances the applicable exclusivity period is ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. In September 2019, the FDA granted Orphan Drug Designation to BXCL701 for the treatment of acute myeloid leukemia. Prior to 2019, the FDA granted Orphan Drug Designation to BXCL for the treatment of pancreatic cancer and melanoma. We may seek Orphan Drug Designations for BXCL in other indications or for our other product candidates. There can be no assurances that we will be able to obtain such designations.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain or maintain orphan drug exclusivity for that product candidate. We may not be the first to obtain marketing approval of any product candidate for which we have obtained orphan drug designation for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products, and it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity expires. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient

quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties may be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care or the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process, nor does it prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation.

If we are unable to develop satisfactory sales and marketing capabilities, we may not succeed in commercializing BXCL501, BXCL701 or any other product candidate.

We have no experience in marketing and selling drug products. We have not entered into arrangements for the sale and marketing of BXCL501, BXCL701 or any other product candidate. Typically, pharmaceutical companies would employ groups of sales representatives and associated sales and marketing staff numbering in the hundreds to thousands of individuals to call on this large number of physicians and hospitals. We may seek to collaborate with a third party to market our drugs or may seek to market and sell our drugs by ourselves. If we seek to collaborate with a third party, we cannot be sure that a collaborative agreement can be reached on terms acceptable to us. If we seek to market and sell our drugs directly, we will need to hire additional personnel skilled in marketing and sales. We cannot be sure that we will be able to acquire, or establish third party relationships to provide, any or all of these marketing and sales capabilities. The establishment of a direct sales force or a contract sales force or a combination direct and contract sales force to market our products will be expensive and time-consuming and could delay any product launch. Further, we can give no assurances that we may be able to maintain a direct and/or contract sales force for any period of time or that our sales efforts will be sufficient to grow our revenues or that our sales efforts will ever lead to profits.

We operate in a highly competitive and rapidly changing industry.

Biopharmaceutical product development is highly competitive and subject to rapid and significant technological advancements. Our success is highly dependent upon our ability to in-license, acquire, develop and obtain regulatory approval for new and innovative products on a cost-effective basis and to market them successfully. In doing so, we face and will continue to face intense competition from a variety of businesses, including large, fully integrated, well-established pharmaceutical companies who already possess a large share of the market, specialty pharmaceutical and biopharmaceutical companies, academic institutions, government agencies and other private and public research institutions in the United States, the European Union and other jurisdictions.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the biopharmaceutical industry could result in even more resources being concentrated among a small number of our competitors.

Competition may further increase as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop.

Established biopharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our

competitors may succeed in obtaining patent protection, discovering, developing, receiving FDA approval for or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

The availability of our competitors' products could limit the demand and the price we are able to charge for any product candidate we commercialize, if any. The inability to compete with existing or subsequently introduced drugs would harm our business, financial condition and results of operations.

Even if we obtain regulatory approvals to commercialize BXCL501, BXCL701 or our other product candidates, our product candidates may not be accepted by physicians or the medical community in general.

There can be no assurance that BXCL501, BXCL701 and our other product candidates or any other product candidate successfully developed by us, independently or with partners, will be accepted by physicians, hospitals and other health care facilities. BXCL501, BXCL701 and any future product candidates we develop will compete with a number of products manufactured and marketed by major pharmaceutical and biotech companies. The degree of market acceptance of any drugs we develop depends on a number of factors, including:

- our demonstration of the clinical efficacy and safety of BXCL501, BXCL701 and our other product candidates;
- timing of market approval and commercial launch of BXCL501, BXCL701 and our other product candidates;
- the clinical indication(s) for which BXCL501, BXCL701 and our other product candidates are approved;
- product label and package insert requirements;
- advantages and disadvantages of our product candidates compared to existing therapies;
- continued interest in and growth of the market for anti-cancer or anti-agitation drugs;
- strength of sales, marketing, and distribution support;
- product pricing in absolute terms and relative to alternative treatments;
- future changes in health care laws, regulations, and medical policies; and
- availability of reimbursement codes and coverage in select jurisdictions, and future changes to reimbursement policies of government and third-party payors.

Significant uncertainty exists as to the coverage and reimbursement status of any product candidate for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations.

Healthcare reform measures could hinder or prevent our product candidates' commercial success.

The U.S. government and other governments have shown significant interest in pursuing healthcare reform. Any government-adopted reform measures could adversely impact the pricing of healthcare products and services in the United States or internationally and the amount of reimbursement available from governmental agencies or other third-party payors. The continuing efforts of the U.S. and foreign governments, insurance companies, managed care organizations and other payors of health care services to contain or reduce health care costs may adversely affect our ability to set prices for our products which we believe are fair, and our ability to generate revenues and achieve and maintain profitability.

New laws, regulations and judicial decisions, or new interpretations of existing laws, regulations and decisions, that relate to healthcare availability, methods of delivery or payment for products and services, or sales, marketing or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons, including policies advanced by the current executive administration in the United States, new healthcare legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the United States and some

foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

For example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the ACA has substantially changed the way healthcare is financed by both government health plans and private insurers, and significantly impacts the pharmaceutical industry. The ACA contains a number of provisions that are expected to impact our business and operations in ways that may negatively affect our potential revenues in the future. For example, the ACA imposes a non-deductible excise tax on pharmaceutical manufacturers or importers that sell branded prescription drugs to government programs which we believe will increase the cost of our products. In addition, as part of the ACA's provisions closing a funding gap that currently exists in the Medicare Part D prescription drug program, we will be required to provide a discount on branded prescription drugs equal to 50% of the government-negotiated price, for drugs provided to certain beneficiaries who fall within the donut hole. Similarly, ACA increases the level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1% and requires collection of rebates for drugs paid by Medicaid managed care organizations. The ACA also includes significant changes to the 340B drug discount program including expansion of the list of eligible covered entities that may purchase drugs under the program. At the same time, the expansion in eligibility for health insurance benefits created under ACA is expected to increase the number of patients with insurance coverage who may receive our products. While it is too early to predict all the specific effects the ACA or any future healthcare reform legislation will have on our business, they could have a material adverse effect on our business and financial condition.

Since its enactment, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to the ACA in the future. For example, the Tax Cuts and Jobs Act, or the Tax Act, was enacted, which, among other things, removes penalties for not complying with the ACA's individual mandate to carry health insurance. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. It is unclear how these decisions, subsequent appeals, if any, or other efforts to challenge, repeal or replace the ACA will impact the law, or our business or financial condition.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. These changes include the Budget Control Act of 2011, which resulted in aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2029 unless additional Congressional action is taken, as well as the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Recently, there has also been heightened government scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted legislation designed to, among other things, reform government program reimbursement methodologies. Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Furthermore, there has been increased interest by third party payors and governmental authorities in reference pricing systems and publication of discounts and list prices.

It is likely that federal and state legislatures within the United States and foreign governments will continue to consider changes to existing healthcare legislation. We cannot predict the reform initiatives that may be adopted in the future or whether initiatives that have been adopted will be repealed or modified. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect the demand for any drug products for which we may obtain regulatory approval, our ability to set a price that we believe is fair for our products, our ability to obtain adequate coverage and

reimbursement approval for a product, our ability to generate revenues and achieve or maintain profitability, and the level of taxes that we are required to pay.

Other third-party payors are increasingly challenging the prices charged for medical products and services. It will be time consuming and expensive for us to go through the process of seeking coverage and reimbursement from Medicare and private payors. Our proposed products may not be considered cost-effective, and coverage and reimbursement may not be available or sufficient to allow us to sell our proposed products on a profitable basis. Further federal and state proposals and health care reforms are likely which could limit the prices that can be charged for the product candidates that we develop and may further limit our commercial opportunities. Our results of operations could be materially adversely affected by proposed healthcare reforms, by the Medicare prescription drug coverage legislation, by the possible effect of such current or future legislation on amounts that private insurers will pay and by other health care reforms that may be enacted or adopted in the future.

In September 2007, the Food and Drug Administration Amendments Act of 2007 was enacted, giving the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in delays or increased costs during product development, clinical trials and regulatory review, increased costs to assure compliance with post-approval regulatory requirements, and potential restrictions on the sale and/or distribution of approved products.

Risks Related to Our Relationship with BioXcel Corporation

BioXcel Corporation has significant influence over the direction of our business, and the concentrated ownership of our common stock will prevent you and other stockholders from influencing significant decisions.

As of February 24, 2020, BioXcel owned approximately 45.5% of the economic interest and voting power of our outstanding common stock. Even though BioXcel controls less than a majority of the voting power of our outstanding common stock, it may influence the outcome of such corporate actions so long as it owns a significant portion of our common stock.

Approval of commercial terms between us and BioXcel does not preclude the possibility of stockholder litigation, including but not limited to derivative litigation nominally against BioXcel and against its directors and officers and also against us and our directors and officers.

The commercial terms of the Second Amended and Restated Shared Services Agreement dated March 6, 2020, or the Services Agreement, and the Amended and Restated Asset Contribution Agreement, or the Contribution Agreement, that we have entered into with BioXcel have not been negotiated on behalf of BioXcel by persons consisting solely of disinterested BioXcel directors.

No assurance can be given that any stockholder of BioXcel will not claim in a lawsuit that such terms in fact are not in the best interests of BioXcel and its stockholders, that the directors and officers of BioXcel breached their fiduciary duties in connection with such agreements and that any disclosures by BioXcel to its stockholders regarding these agreements and the relationship between BioXcel and us did not satisfy applicable requirements. In any such instance, we and our directors and officers may also be named as defendants and we would have to defend ourselves and our directors and officers. While we will seek indemnification from BioXcel under the terms of these agreements against any damages or other costs, which could be substantial, no such indemnification has yet been agreed to or may be agreed to and be in effect. Further, any such litigation would be time-consuming and would divert focus and resources from the development of our product candidates and our business, including but not limited to possibly delaying our clinical trials due to our management having to spend time and attention on such litigation.

We continue to depend on BioXcel to provide us with certain services for our business.

Certain administrative services required by us for the operation of our business have historically been provided by BioXcel, including services related to insurance and risk management, accounting and human resources. Under the

Services Agreement, BioXcel has provided us with various services and will continue to do so until we are able to build our own capabilities in the transition areas. We believe it has been efficient for BioXcel to provide these services for us to facilitate the efficient operation of our business as we transition to becoming an independent, public company. At our election, or if BioXcel does not or is unable to perform its obligations under the Services Agreement, we will be required to provide these services ourselves or to obtain substitute arrangements with other third parties. Virtually all of these administrative services have transitioned to our control. However, we may be unable to continue to provide these services because of financial or other constraints or we may be unable to implement substitute arrangements on a timely basis on terms that are favorable to us, or at all.

We exercise no control over the activities of BioXcel other than the contractual rights we have pursuant to our Services Agreement and Contribution Agreement. Because of our historical relationship with our Parent, our reputation is also tied to BioXcel. We may be subject to reputational harm, or our relationships with existing and potential clients, third-party research organizations, consultants and other business partners could be harmed if BioXcel or any of its affiliates, previously, or in the future, among other things, engages in poor business practices, restructures or files for bankruptcy, becomes subject to litigation or otherwise damages its reputation or business prospects. Any of these events might in turn adversely affect our reputation, revenues and/or business prospects, and may also adversely affect our access to EvolverAI and BioXcel's collaborative services.

We also rely, in part, on BioXcel and access to EvolverAI, a research and development engine created and owned by BioXcel, to identify, research and develop potential product candidates in neuroscience and immuno-oncology. The Company has negotiated a collaborative services agreement with BioXcel pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. On March 6, 2020, by mutual agreement, we agreed to extend this arrangement by one year to December 31, 2020. In addition, BioXcel has granted us a first right to negotiate exclusive rights to any additional product candidates in the fields of neuroscience and immuno-oncology that BioXcel may identify on its own and not in connection with BioXcel's provision of services to us under the Services Agreement. This option for first negotiation shall be valid for a period of five years from the date of our IPO. If our rights and access to BioXcel's collaborative services and to EvolverAI were to become limited, terminated, or if we were otherwise precluded from conducting research and development using EvolverAI, or if BioXcel is unable to fulfill its obligations under the agreements, such development could materially adversely affect our future operating results, financial condition and prospects. Furthermore, certain individuals conducting services on our behalf are not our employees, and except for remedies available to us under our agreements with BioXcel, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. We also cannot ensure that BioXcel retains sufficient resources of personnel or otherwise to conduct its operations. BioXcel may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting research and development activities, which could impede their ability to devote appropriate time to our research and development programs. In addition, if we fail to comply with our diligence, payment or other obligations under the agreements, any such collaboration may terminate or we may not be able to successfully negotiate agreements for future product candidates or collaborations with BioXcel.

The ownership by our executive officers and our directors of shares of BioXcel common stock and rights to purchase BioXcel common stock may create, or may create the appearance of, conflicts of interest.

The ownership by our executive officers and our directors of shares of BioXcel common stock, options to purchase shares of BioXcel common stock, or other equity awards of BioXcel may create, or may create the appearance of, conflicts of interest. Our Chief Executive Officer and director, Vimal Mehta, Ph.D., is Chief Executive Officer and Chairman of BioXcel. In addition, our director and Chief Digital Officer, Krishnan Nandabalan, Ph.D., serves as President, Chief Scientific Officer and a director of BioXcel. Because of Drs. Mehta and Nandabalan's current positions with BioXcel, they own shares of BioXcel common stock, options to purchase shares of BioXcel common stock or other equity awards of BioXcel. As of December 31, 2019, our Dr. Mehta and Dr. Nandabalan each owned approximately 42% of outstanding BioXcel voting stock. Ownership by our executive officers and directors of common stock or options to purchase common stock of BioXcel, or any other equity awards, creates, or, may create the appearance of, conflicts of interest when these individuals are faced with decisions that could have different implications for BioXcel than the decisions have for us, including decisions that relate to our Services Agreement, Contribution Agreement, as well as potential agreements relating to future product candidates and AI-related services or collaborations. In

connection with the various agreements and transactions entered into in connection with our separation from BioXcel, or the Separation, our chief executive officer has agreed to recuse himself with respect to voting on any matter coming before either BioXcel's or our board of directors related to our relationship with BioXcel, although he will still be permitted to participate in discussions and negotiations. Any perceived conflicts of interest resulting from investors questioning the independence of our management or the integrity of corporate governance procedures may materially affect our stock price.

Any disputes that arise between us and BioXcel with respect to our past and ongoing relationships could harm our business operations.

Disputes may arise between BioXcel and us in a number of areas relating to our past and ongoing relationships, including:

- intellectual property, technology and business matters, including failure to make required technology transfers and failure to comply with non-compete provisions applicable to BioXcel and us;
- labor, tax, employee benefit, indemnification and other matters arising from the Separation;
- distribution and supply obligations;
- employee retention and recruiting;
- business combinations involving us;
- sales or distributions by BioXcel of all or any portion of its ownership interest in us;
- the nature, quality and pricing of services BioXcel has agreed to provide us; and
- business opportunities that may be attractive to both BioXcel and us.

We entered into the Services Agreement with BioXcel related to the Separation of our business operations from those of BioXcel that contains certain limitations on BioXcel's ability to control various aspects of our business and operations, notwithstanding BioXcel's substantial ownership position. This agreement may be amended upon agreement between us and BioXcel.

BioXcel may experience challenges with the acquisition, development, enhancement or deployment of technology necessary for EvolverAI.

BioXcel operates in businesses that require sophisticated computer systems and software for data collection, data processing, cloud-based platforms, analytics, statistical projections and forecasting, mobile computing, social media analytics and other applications and technologies. BioXcel seeks to address its technology risks by increasing its reliance on the use of innovations by cross-industry technology leaders and adapt these for their pharmaceutical, specialty-pharma, biotech, biopharmaceutical, diagnostic, medical device and contract research and manufacturing clients. Some of the technologies supporting the industries they serve are changing rapidly and we must continue to adapt to these changes in a timely and effective manner at an acceptable cost. They also must continue to deliver data to its clients in forms that are easy to use while simultaneously providing clear answers to complex questions. There can be no guarantee that we or BioXcel will be able to develop, acquire or integrate new technologies, that these new technologies will meet our and BioXcel's needs or achieve our expected goals, or that we will be able to do so as quickly or cost-effectively as our competitors. Significant technological change could render EvolverAI obsolete. BioXcel's continued success will depend on its ability to adapt to changing technologies, manage and process ever-increasing amounts of data and information and improve the performance, features and reliability of its services in response to changing client and industry demands. BioXcel may experience difficulties that could delay or prevent the successful design, development, testing, and introduction of advanced versions of EvolverAI, limiting our ability to identify new product candidates. New services, or enhancements to existing EvolverAI services, may not adequately meet our requirements. Any of these failures could have a material adverse effect on our operating results and financial condition.

Risks Related to Our Reliance on Third Parties

We are substantially dependent on third parties for the manufacture of our clinical supplies of our product candidates, and we intend to rely on third parties to produce commercial supplies of any approved product candidate. Therefore, our development of our products could be stopped or delayed, and our commercialization of any future product could be stopped or delayed or made less profitable if third party manufacturers fail to obtain approval of the FDA or comparable regulatory authorities or fail to provide us with drug product in sufficient quantities or at acceptable prices.

The manufacture of biotechnology and pharmaceutical products is complex and requires significant expertise, capital investment, process controls and know-how. Common difficulties in biotechnology and pharmaceutical manufacturing may include: sourcing and producing raw materials, transferring technology from chemistry and development activities to production activities, validating initial production designs, scaling manufacturing techniques, improving costs and yields, establishing and maintaining quality controls and stability requirements, eliminating contaminations and operator errors, and maintaining compliance with regulatory requirements. We do not currently have nor do we plan to acquire the infrastructure or capability internally to produce an adequate supply of compounds to meet future requirements for clinical trials and commercialization of our products or to produce our products in accordance with cGMP prescribed by the FDA. Drug manufacturing facilities are subject to inspection before the FDA will issue an approval to market a new drug product, and all of the manufacturers that we intend to use must adhere to the cGMP regulations prescribed by the FDA.

We expect therefore to rely on third-party manufacturers for clinical supplies of our product candidates that we may develop. These third-party manufacturers will be required to comply with cGMPs, and other applicable laws and regulations. We will have no control over the ability of these third parties to comply with these requirements, or to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authorities do not approve the facilities of these third parties for the manufacture of our other product candidates or any products that we may successfully develop, or if it withdraws any such approval, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all. Any of these factors would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates and adversely affect our business.

We and/or our third-party manufacturers may be adversely affected by developments outside of our control, and these developments may delay or prevent further manufacturing of our products. Adverse developments may include labor disputes, resource constraints, shipment delays, inventory shortages, lot failures, unexpected sources of contamination, lawsuits related to our manufacturing techniques, equipment used during manufacturing, or composition of matter, unstable political environments, acts of terrorism, war, natural disasters, and other natural and man-made disasters. If BioXcel, we or our third-party manufacturers were to encounter any of the above difficulties, or otherwise fail to comply with contractual obligations, our ability to provide any product for clinical trial or commercial purposes would be jeopardized. This may increase the costs associated with completing our clinical trials and commercial production. Further, production disruptions may cause us to terminate ongoing clinical trials and/or commence new clinical trials at additional expense. We may also have to take inventory write-offs and incur other charges and expenses for products that fail to meet specifications or pass safety inspections. If production difficulties cannot be solved with acceptable costs, expenses, and timeframes, we may be forced to abandon our clinical development and commercialization plans, which could have a material adverse effect on our business, prospects, financial condition, and the value of our securities.

We, or third-party manufacturers on whom we rely, may be unable to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing approved products, if any.

In order to conduct clinical trials of our product candidates and commercialize any approved product candidates, we, or our manufacturers, will need to manufacture them in large quantities. We, or our manufacturers, 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 we, or any of our manufacturers, are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing, and clinical trials of that product candidate may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. If we are unable to obtain or maintain third-party manufacturing for commercial supply of our product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully.

Our failure to find third party collaborators to assist or share in the costs of product development could materially harm our business, financial condition and results of operations.

Our strategy for the development and commercialization of our proprietary product candidates may include the formation of collaborative arrangements with third parties. We are a party to several collaboration agreements (research and clinical), including agreements with Nektar Therapeutics, Inc., or Nektar, relating to Nektar's Bempegaldesleukin (NKTR-214) and Merck KGaA, Darmstadt, Germany and Pfizer Inc.'s Opdivo compound and BXCL 701. Existing and future collaborators have significant discretion in determining the efforts and resources they apply and may not perform their obligations as expected. Potential third party collaborators include biopharmaceutical, pharmaceutical and biotechnology companies, academic institutions and other entities. Third-party collaborators may assist us in:

- funding research, preclinical development, clinical trials and manufacturing;
- seeking and obtaining regulatory approvals; and
- successfully commercializing any future product candidates.

If we are not able to establish further collaboration agreements, we may be required to undertake product development and commercialization at our own expense. Such an undertaking may limit the number of product candidates that we will be able to develop, significantly increase our capital requirements and place additional strain on our internal resources. Our failure to enter into additional collaborations could materially harm our business, financial condition and results of operations.

In addition, our dependence on licensing, collaboration and other agreements with third parties may subject us to a number of risks. These agreements may not be on terms that prove favorable to us and may require us to relinquish certain rights in our product candidates. To the extent we agree to work exclusively with one collaborator in a given area, our opportunities to collaborate with other entities could be curtailed. Lengthy negotiations with potential new collaborators may lead to delays in the research, development or commercialization of product candidates. The decision by our collaborators to pursue alternative technologies or the failure of our collaborators to develop or commercialize successfully any product candidate to which they have obtained rights from us could materially harm our business, financial condition and results of operations.

We rely on third parties to conduct our preclinical and clinical trials. If these third parties do not successfully perform their contractual legal and regulatory duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party medical institutions, clinical investigators, contract laboratories and other third party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical and clinical trials, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCPs, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the member states of the European Economic Area, or EEA, and comparable foreign regulatory authorities for all of our products in clinical development.

Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon

inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. In addition, 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 on-going clinical, nonclinical and preclinical programs. 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 clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our 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, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If the third parties conducting our GLP preclinical studies or our clinical trials do not perform their contractual duties or obligations, experience work stoppages, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the clinical data they obtain is compromised due to their failure to adhere to our clinical trial protocols or to GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties. Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Risks Related to Our Business and Industry

We will need to increase the size of our organization and the scope of our outside vendor relationships, and we may experience difficulties in managing growth.

As of December 31, 2019, we employed a total of 24 full-time employees. In addition, we have access to certain of BioXcel's employees and resources through the various agreements we have entered into with BioXcel. Our current internal departments include finance, research and development and administration. We have been expanding our management team to include an operational ramp up of additional technical staff required to achieve our business objectives. We will need to continue to expand our managerial, operational, technical and scientific, financial and other resources in order to manage our operations and clinical trials, establish independent manufacturing, continue our research and development activities, and commercialize our product candidate. Our management and scientific personnel, systems and facilities currently in place may not be adequate to support our future growth.

Our need to effectively manage our operations, growth and various projects requires that we:

- manage our clinical trials effectively, including our planned clinical trials of BXCL501, BXCL701 and our other product candidates;
- manage our internal development efforts effectively while carrying out our contractual obligations to licensors, contractors and other third parties;
- continue to improve our operational, financial and management controls and reporting systems and procedures; and
- attract and retain sufficient numbers of talented employees.

We may utilize the services of third party vendors to perform tasks including pre-clinical and clinical trial management, statistics and analysis, regulatory affairs, medical advisory, market research, formulation development, chemistry, manufacturing and control activities, other drug development functions, legal, auditing, financial advisory, and investor relations. Our growth strategy may also entail expanding our group of contractors or consultants to implement these and other tasks going forward. Because we rely on numerous consultants, to outsource many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. However, 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 for our product candidate 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, or at all. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may be unable to successfully implement the tasks necessary to further develop and commercialize our product candidate and, accordingly, may not achieve our research, development and commercialization goals.

We depend on our senior management team, and the loss of one or more of our executive officers or key employees or an inability to attract and retain highly skilled employees could adversely affect our business.

Our success depends largely upon the continued services of our key executive officers, Vimal Mehta, our Chief Executive Officer, President, Secretary and Director and Frank Yocca, our Chief Scientific Officer. We do not maintain "key person" insurance for any of these executive officers or any of our other key employees. We also rely on our leadership team in the areas of research and development, marketing, services and general and administrative functions. From time to time, there may be changes in our executive management and leadership teams resulting from the hiring or departure of executives or other key employees, which could disrupt our business. The replacement of one or more of our executive officers or other key employees would likely involve significant time and costs and may significantly delay or prevent the achievement of our business objectives.

To continue to execute our growth strategy, we also must attract and retain highly skilled personnel. We might not be successful in maintaining our unique culture and continuing to attract and retain qualified personnel. We have from time to time in the past experienced, and we expect to continue to experience in the future, difficulty in hiring and retaining highly skilled personnel with appropriate qualifications. The pool of qualified personnel with experience working with the pharma market is limited overall. In addition, many of the companies with which we compete for experienced personnel have greater resources than we have.

In addition, in making employment decisions, particularly in the internet, biotechnology and high-technology industries, job candidates often consider the value of the stock options or other equity instruments they are to receive in connection with their employment. Volatility in the price of our stock might, therefore, adversely affect our ability to attract or retain highly skilled personnel. Furthermore, the requirement to expense stock options and other equity instruments might discourage us from granting the size or type of stock option or equity awards that job candidates require to join our company. If we fail to attract new personnel or fail to retain and motivate our current personnel, our business and future growth prospects could be severely harmed.

We may acquire other companies or technologies, which could divert our management's attention, result in dilution to our stockholders and otherwise disrupt our operations and adversely affect our operating results.

We may in the future seek to acquire or invest in businesses, applications and services or technologies that we believe could complement or expand our services, enhance our technical capabilities or otherwise offer growth opportunities. The pursuit of potential acquisitions may divert the attention of management and cause us to incur various expenses in identifying, investigating and pursuing suitable acquisitions, whether or not they are consummated.

In addition, we do not have any experience in acquiring other businesses. If we acquire additional businesses, we may not be able to integrate the acquired personnel, operations and technologies successfully, or effectively manage the

combined business following the acquisition. We also may not achieve the anticipated benefits from the acquired business due to a number of factors, including:

- inability to integrate or benefit from acquired technologies or services in a profitable manner;
- unanticipated costs or liabilities associated with the acquisition;
- difficulty integrating the accounting systems, operations and personnel of the acquired business;
- difficulties and additional expenses associated with supporting legacy products and hosting infrastructure of the acquired business;
- difficulty converting the customers of the acquired business onto our platform and contract terms, including disparities in the revenue, licensing, support or professional services model of the acquired company;
- diversion of management's attention from other business concerns;
- adverse effects to our existing business relationships with business partners and customers as a result of the acquisition;
- the potential loss of key employees;
- use of resources that are needed in other parts of our business; and
- use of substantial portions of our available cash to consummate the acquisition.

In addition, a significant portion of the purchase price of companies we acquire may be allocated to acquired goodwill and other intangible assets, which must be assessed for impairment at least annually. In the future, if our acquisitions do not yield expected returns, we may be required to take charges to our operating results based on this impairment assessment process, which could adversely affect our results of operations.

Acquisitions could also result in dilutive issuances of equity securities or the incurrence of debt, which could adversely affect our operating results. In addition, if an acquired business fails to meet our expectations, our operating results, business and financial position may suffer.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with any regulations applicable to us, to provide accurate information to regulatory authorities, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations, or to report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risk.

Business interruptions could adversely affect future operations, revenues, and financial conditions, and may increase our costs and expenses.

Our operations, and those of our directors, advisors, contractors, consultants, CROs, and collaborators, could be adversely affected by earthquakes, floods, hurricanes, typhoons, extreme weather conditions, fires, water shortages, power failures, business systems failures, medical epidemics and other natural and man-made disaster or business interruptions. Our phones, electronic devices and computer systems and those of our directors, advisors, contractors, consultants, CROs, and collaborators are vulnerable to damages, theft and accidental loss, negligence, unauthorized access, terrorism, war, electronic and telecommunications failures, and other natural and man-made disasters. Several of our employees conduct business outside of our headquarters and leased or owned facilities. These locations may be subject to additional security and other risk factors due to the limited control of our employees. If such an event as

described above were to occur in the future, it may cause interruptions in our operations, delay research and development programs, clinical trials, regulatory activities, manufacturing and quality assurance activities, sales and marketing activities, hiring, training of employees and persons within associated third parties, and other business activities. For example, the loss of clinical trial data from completed 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 will rely on third parties to manufacture BXCL501 and BXCL701 and conduct clinical trials, and similar events as those described in the prior paragraph relating to their business systems, equipment and facilities 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 applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidate could be delayed or altogether terminated.

Risks associated with data privacy issues, including evolving laws, regulations and associated compliance efforts, may adversely impact our business and financial results.

Legislation in various countries around the world with regard to cybersecurity, privacy and data protection is rapidly expanding and creating a complex compliance environment. We are subject to many privacy and data protection laws and regulations in the U.S. and around the world, some of which place restrictions on our ability to process personal data across our business. In particular, the General Data Protection Regulation, or GDPR, which became effective in May 2018, has caused more stringent data protection requirements in the European Union. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and implement policies as part of its mandated privacy governance framework. It also requires data controllers to be transparent and disclose to data subjects how their personal information is to be used; imposes limitations on retention of personal data; introduces mandatory data breach notification requirements; and sets higher standards for data controllers to demonstrate that they have obtained valid consent for certain data processing activities. To the extent we collect data from individuals in the European Union, we will be subject to the supervision of local data protection authorities in those E.U. jurisdictions where we are established or otherwise subject to the GDPR. Certain breaches of the GDPR requirements could result in substantial fines, which can be up to four percent of worldwide revenue or 20 million Euros, whichever is greater. In addition to the foregoing, a breach of the GDPR could result in regulatory investigations, reputational damage, orders to cease/change our use of data, enforcement notices, as well potential civil claims including class action type litigation where individuals suffered harm.

Our failure to successfully acquire, develop and market additional product candidates or approved drug products could impair our ability to grow.

As part of our growth strategy, we may evaluate, acquire, license, develop and/or market additional product candidates and technologies. These investments will not constitute a significant portion of our business. However, our internal research capabilities are limited and we may be dependent upon pharmaceutical and biotechnology companies, academic scientists and other researchers to sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify, select and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. We have limited resources to identify and execute the acquisition or in-licensing of third party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire the rights to additional product candidates on terms that we find acceptable, or at all.

In addition, future acquisitions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;

- disruption of our business and diversion of our management's and technical personnel's time and attention to develop acquired products or technologies;
- incurrence of substantial debt or dilutive issuances of securities to pay for acquisitions;
- higher than expected acquisition and integration costs;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

Any product candidate that we acquire may require additional development efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot provide assurance that any products that we develop or approved products that we acquire will be manufactured profitably or achieve market acceptance.

Our ability to use net operating losses and research and development credits to offset future taxable income may be subject to certain limitations.

As of December 31, 2019, we had federal and state net operating loss carryforwards, or NOLs, of approximately $16.4 million. Our NOLs arising before January 1, 2018 are subject to expiration and will begin to expire in 2037. As of December 31, 2019, we also had federal and state research and development and other tax credit carryforwards, or credits of approximately $1.3 million available to reduce future tax liabilities. The federal and state credits expire at various dates through 2037. These NOLs and credits could expire unused and be unavailable to offset future taxable income or income tax liabilities. In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change NOLs or credits to offset future taxable income or income tax liabilities. For these purposes, an ownership change generally occurs where the aggregate change in stock ownership of one or more stockholders or groups of stockholders owning at least 5% of a corporation's stock exceeds 50 percentage points over a three-year period. Future changes in our stock ownership, including as a result of February 2020 offering of common stock, many of which are outside of our control, could result in an ownership change. Our NOLs or credits may also be impaired under state law. Accordingly, even if we attain profitability, we may not be able to utilize a material portion of our NOLs or credits. Furthermore, under the Tax Cuts and Jobs Act of 2017, although the treatment of NOLs arising on or before December 31, 2017 has generally not changed, NOLs arising on or after January 1, 2018 and beyond may only be used to offset 80% of taxable income. This change may require us to pay federal income taxes in future years despite generating a loss for federal income tax purposes in prior years.

Risks Related to Our Intellectual Property

It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection. If our patent position does not adequately protect our product candidates, others could compete against us more directly, which would harm our business, possibly materially.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our current and future product candidates, the processes used to manufacture them and the methods for using them, as well as successfully defending these patents against third-party challenges. We are the owner of record of patents and patent applications pending in the United States and in certain foreign jurisdictions. We own Patent Cooperation Treaty, or PCT, patent applications relating to our platform technologies covering methods of use and applications of the platform technologies. As of February 20, 2020, we have three allowed patents, all ex-U.S., which are relevant to our BXCL701 program. We cannot be certain that any future patents will issue with claims that cover our product candidates. Our ability to stop third parties from making, using, selling, offering to sell or importing our product

candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

The patent positions of biotechnology and pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States or in foreign jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently or may in the future own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in interference, opposition, reexamination, review, reissue, post grant review or invalidity proceedings before U.S. or non-U.S. patent offices.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make compounds that are similar to our product candidates, but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- our pending patent applications may not result in issued patents;
- the claims of our issued patents or patent applications when issued may not cover our products or product candidates;
- any patents that we obtain may not provide us with any competitive advantages;
- any granted patents may be held invalid or unenforceable as a result of legal challenges by third parties; and
- the patents of others may have an adverse effect on our business.

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

Periodic maintenance, renewal and annuity fees and various other government fees on any issued patent and pending patent application must be paid to the U.S. Patent and Trademark Office ("USPTO") and foreign patent agencies in several stages or annually over the lifetime of our owned and in-licensed patents and patent applications. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, potential competitors might be able to enter the market with similar or identical products or technology. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it would have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

We may be required to enter into intellectual property license agreements that are important to our business. These license agreements may impose various diligence, milestone payment, royalty and other obligations on us. For example, we may enter into exclusive license agreements with various universities and research institutions, we may be required to use commercially reasonable efforts to engage in various development and commercialization activities with respect to licensed products, and may need to satisfy specified milestone and royalty payment obligations. If we fail to comply with any obligations under our agreements with any of these licensors, we may be subject to termination of the license agreement in whole or in part; increased financial obligations to our licensors or loss of exclusivity in a particular field or territory, in which case our ability to develop or commercialize products covered by the license agreement will be impaired.

In addition, disputes may arise regarding intellectual property subject to a license 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;
- our diligence obligations under the license agreement and what activities satisfy those obligations;
- if a third-party expresses interest in an area under a license that we are not pursuing, under the terms of certain of our license agreements, we may be required to sublicense rights in that area to a third party, and that sublicense could harm our business; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our product candidates, which could harm our business significantly.

We may be subject to claims challenging the inventorship or ownership 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 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. Litigation may be necessary to defend against these and other claims challenging inventorship or our 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. 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, financial condition, results of operations and prospects.

An NDA submitted under Section 505(b)(2) subjects us to the risk that we may be subject to a patent infringement lawsuit that would delay or prevent the review or approval of our product candidate.

Our product candidates will be submitted to the FDA for approval under Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act, or FDCA. Section 505(b)(2) permits the submission of an NDA where at least some of the information required for approval comes from studies that were not conducted by, or for, the applicant and on which the

applicant has not obtained a right of reference. The 505(b)(2) application would enable us to reference published literature and/or the FDA's previous findings of safety and effectiveness for a branded reference drug with the same active ingredient. For NDAs submitted under Section 505(b)(2) of the FDCA, the patent certification and related provisions of the Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, apply. In accordance with the Hatch-Waxman Act, such NDAs may be required to include certifications, known as paragraph IV certifications, that certify that any patents listed in the Patent and Exclusivity Information Addendum of the FDA's publication, Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book, with respect to any product referenced in the 505(b)(2) application, are invalid, unenforceable or will not be infringed by the manufacture, use or sale of the product that is the subject of the 505(b)(2) NDA.

Under the Hatch-Waxman Act, the holder of patents that the 505(b)(2) application references may file a patent infringement lawsuit after receiving notice of the paragraph IV certification. Filing of a patent infringement lawsuit against the filer of the 505(b)(2) applicant within 45 days of the patent owner's receipt of notice triggers a one-time, automatic, 30 month stay of the FDA's ability to approve the 505(b)(2) NDA, unless patent litigation is resolved in the favor of the paragraph IV filer or the patent expires before that time. Accordingly, we may invest a significant amount of time and expense in the development of one or more product candidates only to be subject to significant delay and patent litigation before such product candidates may be commercialized, if at all. In addition, a 505(b)(2) application will not be approved until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, or NCE, listed in the Orange Book for the branded reference drug product has expired. The FDA may also require us to perform one or more additional clinical studies or measurements to support the change from the branded reference drug, which could be time consuming and could substantially delay our achievement of regulatory approvals for such product candidates. The FDA may also reject our future 505(b)(2) submissions and require us to file such submissions under Section 505(b)(1) of the FDCA, which would require us to provide extensive data to establish safety and effectiveness of the drug product for the proposed use and could cause delay and be considerably more expensive and time consuming. These factors, among others, may limit our ability to successfully commercialize our product candidates.

We may incur substantial costs as a result of litigation or other proceedings relating to patents and other intellectual property rights.

If we choose to commence a proceeding or litigation to prevent another party from infringing our patents, that party will have the right to ask the examiner or court to rule that our patents are invalid or should not be enforced against them. There is a risk that the examiner or court will decide that our patents are not valid and that we do not have the right to stop the other party from using the related inventions. There is also the risk that, even if the validity of our patents is upheld, the examiner or court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to such patents. In addition, the U.S. Supreme Court has recently modified some tests used by the U.S. Patent and Trademark Office, or USPTO, in granting patents over the past 20 years, which may decrease the likelihood that we will be able to obtain patents and increase the likelihood of challenge to any patents we obtain or license. Any proceedings or litigation to enforce our intellectual property rights or defend ourselves against claims of infringement of third-party intellectual property rights could be costly and divert the attention of managerial and scientific personnel, regardless of whether such litigation is ultimately resolved in our favor. We may not have sufficient resources to bring these actions to a successful conclusion. Moreover, if we are unable to successfully defend against claims that we have infringed the intellectual property rights of others, we may be prevented from using certain intellectual property and may be liable for damages, which in turn could materially adversely affect our business, financial condition or results of operations.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing our product candidates.

Our success will depend in part on our ability to operate without infringing, misappropriating or otherwise violating the proprietary rights of third parties. There is considerable patent and other intellectual property litigation in the pharmaceutical and biotechnology industries. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our products, or the manufacture or use of our product candidates, including interference proceedings, post grant review, inter partes review, and derivation proceedings before

the USPTO and similar proceedings in foreign jurisdictions. The legal threshold for initiating litigation or contested proceedings is low, so that even lawsuits or proceedings with a low probability of success might be initiated and require significant resources to defend. The costs of these lawsuits could affect our results of operations and divert the attention of managerial and scientific personnel. Some of these third parties may be better capitalized and have more resources than us. There is a risk that a court would decide that we are infringing the third party's patents and would order us to stop the activities covered by the patents. In that event, we may not have a viable way around the patent and may need to halt commercialization of the relevant product candidate. In addition, there is a risk that a court will order us to pay the other party damages for having violated the other party's patents. In addition, we may be obligated to indemnify our licensors and collaborators against certain intellectual property infringement claims brought by third parties, which could require us to expend additional resources. The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform.

If we are sued for patent infringement, we would need to demonstrate that our products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, which may not be available, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may incur substantial monetary damages, encounter significant delays in bringing our product candidates to market and be precluded from manufacturing or selling our product candidates.

We cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent the technology, because:

- some patent applications in the United States may be maintained in secrecy until the patents are issued;
- patent applications in the United States are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed US patent applications on inventions similar to ours that claims priority to any applications filed prior to the priority dates of our applications, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar inventions prior to our own inventions, resulting in a loss of our U.S. patent position with respect to such inventions. Other countries have similar laws that permit secrecy of patent applications and may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

If we are not able to adequately prevent disclosure of trade secrets and other proprietary information, the value of our technology and products could be significantly diminished.

We also rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Furthermore, any license agreements we enter into in the future may require us to notify, and in some cases license back to the licensor, certain additional proprietary information or intellectual property that we developed using the rights licensed to us under these agreements. Any such licenses back to the licensor could allow our licensors to use that proprietary information or intellectual property in a manner that could harm our business. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its transparency initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed alleged trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors 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 our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we could lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

Our intellectual property may not be sufficient to protect our products from competition, which may negatively affect our business as well as limit our partnership or acquisition appeal.

We may be subject to competition despite the existence of intellectual property we license or own. We can give no assurances that our intellectual property claims will be sufficient to prevent third parties from designing around patents we own or license and developing and commercializing competitive products. The existence of competitive products that avoid our intellectual property could materially adversely affect our operating results and financial condition. Furthermore, limitations, or perceived limitations, in our intellectual property may limit the interest of third parties to partner, collaborate or otherwise transact with us, if third parties perceive a higher than acceptable risk to commercialization of our products or future products.

Our drug re-innovation approach involves the filing of patent applications covering new methods of use and/or new formulations of previously known, studied and/or marketed drugs. Although the protection afforded by our patent applications may be significant with respect to BXCL501 and BXCL701, when looking at our patents' ability to block competition, the protection offered by our patents may be, to some extent, more limited than the protection provided by patents claiming the composition of matter of entirely new chemical structures previously unknown. If a competitor were able to successfully design around any method of use and formulation patents we may have in the future, our business and competitive advantage could be significantly affected.

We may elect to sue a third party, or otherwise make a claim, alleging infringement or other violation of patents, trademarks, trade dress, copyrights, trade secrets, domain names or other intellectual property rights that we either own

or license from BioXcel. If we do not prevail in enforcing our intellectual property rights in this type of litigation, we may be subject to:

- paying monetary damages related to the legal expenses of the third party;
- facing additional competition that may have a significant adverse effect on our product pricing, market share, business operations, financial condition, and the commercial viability of our products; and
- restructuring our company or delaying or terminating select business opportunities, including, but not limited to, research and development, clinical trial, and commercialization activities, due to a potential deterioration of our financial condition or market competitiveness.

A third party may also challenge the validity, enforceability or scope of the intellectual property rights that we license or own; and, the result of these challenges may narrow the scope or claims of or invalidate patents that are integral to our product candidates in the future. There can be no assurance that we will be able to successfully defend patents we own in an action against third parties due to the unpredictability of litigation and the high costs associated with intellectual property litigation, amongst other factors.

Intellectual property rights and enforcement may be less extensive in jurisdictions outside of the United States; thus, we may not be able to protect our intellectual property and third parties may be able to market competitive products that may use some or all of our intellectual property.

Changes to patent law, including the Leahy-Smith America Invents Act, AIA or Leahy-Smith Act, of 2011 and the Patent Reform Act of 2009 and other future article of legislation, may substantially change the regulations and procedures surrounding patent applications, issuance of patents, and prosecution of patents. We can give no assurances that our patents and those of our licensor, BioXcel, can be defended or will protect us against future intellectual property challenges, particularly as they pertain to changes in patent law and future patent law interpretations.

In addition, enforcing and maintaining our intellectual property protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by the U.S. Patent and Trademark Office, courts and foreign government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Risks Related to Owning our Common Stock

The price of our common stock may fluctuate substantially.

You should consider an investment in our common stock to be risky, and you should invest in our common stock only if you can withstand a significant loss and wide fluctuations in the market value of your investment. Some factors that may cause the market price of our common stock to fluctuate, in addition to the other risks mentioned in this "Risk Factors" section, are:

- sale of our common stock by our stockholders, executives, and directors;
- volatility and limitations in trading volumes of our shares of common stock;
- our ability to obtain financings to conduct and complete research and development activities including, but not limited to, our clinical trials, and other business activities;
- possible delays in the expected recognition of revenue due to lengthy and sometimes unpredictable sales timelines;
- the timing and success of introductions of new applications and services by us or our competitors or any other change in the competitive dynamics of our industry, including consolidation among competitors, customers or strategic partners;
- network outages or security breaches;
- our ability to attract new customers;
- customer renewal rates and the timing and terms of customer renewals;

- our ability to secure resources and the necessary personnel to conduct clinical trials on our desired schedule;
- commencement, enrollment or results of our clinical trials for our product candidates or any future clinical trials we may conduct;
- changes in the development status of our product candidates;
- any delays or adverse developments or perceived adverse developments with respect to the FDA's review of our planned preclinical and clinical trials;
- any delay in our submission for studies or product approvals or adverse regulatory decisions, including failure to receive regulatory approval for our product candidates;
- unanticipated safety concerns related to the use of our product candidates;
- failures to meet external expectations or management guidance;
- changes in our capital structure or dividend policy, future issuances of securities, sales of large blocks of common stock by our stockholders;
- our cash position;
- announcements and events surrounding financing efforts, including debt and equity securities;
- our inability to enter into new markets or develop new products;
- reputational issues;
- competition from existing technologies and products or new technologies and products that may emerge;
- announcements of acquisitions, partnerships, collaborations, joint ventures, new products, capital commitments, or other events by us or our competitors;
- changes in general economic, political and market conditions in or any of the regions in which we conduct our business;
- changes in industry conditions or perceptions;
- changes in valuations of similar companies or groups of companies;
- analyst research reports, recommendation and changes in recommendations, price targets, and withdrawals of coverage;
- departures and additions of key personnel;
- disputes and litigations related to intellectual properties, proprietary rights, and contractual obligations;
- changes in applicable laws, rules, regulations, or accounting practices and other dynamics; and
- other events or factors, many of which may be out of our control.

In addition, if the market for stocks in our industry or industries related to our industry, or the stock market in general, experiences a loss of investor confidence, the trading price of our common stock could decline for reasons unrelated to our business, financial condition and results of operations. If any of the foregoing occurs, it could cause our stock price to fall and may expose us to lawsuits that, even if unsuccessful, could be costly to defend and a distraction to management.

If securities or industry analysts do not publish research or reports, or publish unfavorable research or reports about our business, our stock price and trading volume may 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, our business, our markets and our competitors. We do not control these analysts. If securities analysts do not cover our common stock, the lack of research coverage may adversely affect the market price of our common stock. Furthermore, if one or more of the analysts who do cover us downgrade our stock or if those analysts issue other unfavorable commentary about us or our business, our stock price would likely decline. If one or more of these analysts cease coverage of us or fails to regularly publish reports on us, we could lose visibility in the market and interest in our stock could decrease, which in turn could cause our stock price or trading volume to decline and may also impair our ability to expand our business with existing customers and attract new customers.

Because certain of our stockholders control a significant number of shares of our common stock, they may have effective control over actions requiring stockholder approval.

As of February 24, 2020, our directors, executive officers and principal stockholders, and their respective affiliates, beneficially owned approximately 48.1% of our outstanding shares of common stock. As a result, these stockholders, acting together, would have significant control over the outcome of matters submitted to our stockholders for approval, including the election of directors and any merger, consolidation or sale of all or substantially all of our assets. In addition, these stockholders, acting together, would have significant control over the management and affairs of our company. Accordingly, this concentration of ownership might harm the market price of our common stock by:

- delaying, deferring or preventing a change in corporate control;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

Future sales and issuances of our common stock could result in additional dilution of the percentage ownership of our stockholders and could cause our share price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations, including increased marketing, hiring new personnel, commercializing our products, and continuing activities as an operating public company. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

We do not intend to pay cash dividends on our shares of common stock so any returns will be limited to the value of our shares.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the increase, if any, of our share price.

If we were deemed to be an investment company under the Investment Company Act of 1940, as amended, or the 1940 Act, applicable restrictions could make it impractical for us to continue our business as contemplated and could have a material adverse effect on our business, financial condition and results of operations.

Under Sections 3(a)(1)(A) and (C) of the 1940 Act, a company generally will be deemed to be an "investment company" for purposes of the 1940 Act if (1) it is, or holds itself out as being, engaged primarily, or proposes to engage primarily, in the business of investing, reinvesting or trading in securities or (2) it engages, or proposes to engage, in the business of investing, reinvesting, owning, holding or trading in securities and it owns or proposes to acquire investment securities having a value exceeding 40% of the value of its total assets (exclusive of U.S. government securities and cash items) on an unconsolidated basis. We do not believe that we are an "investment company," as such term is defined in either of those sections of the 1940 Act.

Notwithstanding Sections 3(a)(1)(A) and (C) of the 1940 Act, we are a research and development company and comply with the safe harbor requirements of Rule 3a-8 of the 1940 Act. We intend to conduct our operations so that we will not be deemed an investment company. However, if we were to be deemed an investment company, restrictions imposed by the 1940 Act, including limitations on our capital structure and our ability to transact with affiliates, could make it impractical for us to continue our business as contemplated and could have a material adverse effect on our business, financial condition and results of operations.

We are an "emerging growth company" and "smaller reporting company" and are able to avail ourselves of reduced disclosure requirements applicable to emerging growth companies and small reporting companies, which could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, Section 107 of the JOBS Act also provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are not electing to delay such adoption of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. We cannot predict if investors will find our common stock less attractive because we may 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. We may take advantage of these reporting exemptions until we are no longer an "emerging growth company." We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of our initial public offering; (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 (the "SEC").

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common stock held by non-affiliates is more than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosure, are exempt from the auditor attestation requirements of Section 404, and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide selected financial data, supplemental financial information or risk factors.

We have elected to take advantage of certain of the reduced reporting obligations. 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 reduced or more volatile.

We may be at risk of securities class action litigation.

We may be at risk of securities class action litigation. In the past, biotechnology and pharmaceutical companies have experienced significant stock price volatility, particularly when associated with binary events such as clinical trials and product approvals. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business and results in a decline in the market price of our common stock.

Our certificate of incorporation and our bylaws, and Delaware law may have anti-takeover effects that could discourage, delay or prevent a change in control, which may cause our stock price to decline.

Our amended and restated certificate of incorporation and our amended and restated bylaws and Delaware law could make it more difficult for a third party to acquire us, even if closing such a transaction would be beneficial to our stockholders. We are authorized to issue up to 10,000,000 shares of preferred stock. This preferred stock may be issued

in one or more series, the terms of which may be determined at the time of issuance by our board of directors without further action by stockholders. The terms of any series of preferred stock may include voting rights (including the right to vote as a series on particular matters), preferences as to dividend, liquidation, conversion and redemption rights and sinking fund provisions. No preferred stock is currently outstanding. The issuance of any preferred stock could materially adversely affect the rights of the holders of our common stock, and therefore, reduce the value of our common stock and the Notes. In particular, specific rights granted to future holders of preferred stock could be used to restrict our ability to merge with, or sell our assets to, a third party and thereby preserve control by the present management.

Provisions of our amended and restated certificate of incorporation and our amended and restated bylaws and Delaware law also could have the effect of discouraging potential acquisition proposals or making a tender offer or delaying or preventing a change in control, including changes a stockholder might consider favorable. Such provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. In particular, the certificate of incorporation and bylaws and Delaware law, as applicable, among other things:

- provide the board of directors with the ability to alter the bylaws without stockholder approval;
- place limitations on the removal of directors;
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings; and
- provide that vacancies on the board of directors may be filled by a majority of directors in office, although less than a quorum.

Financial reporting obligations of being a public company in the United States are expensive and time-consuming, and our management is required to devote substantial time to compliance matters.

As a publicly traded company we have incurred significant additional legal, accounting and other expenses that we did not incur as a privately held subsidiary of BioXcel. The obligations of being a public company in the United States requires significant expenditures and will place significant demands on our management and other personnel, including costs resulting from public company reporting obligations under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and the rules and regulations regarding corporate governance practices, including those under the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, and the listing requirements of the stock exchange on which our securities are listed. These rules require the establishment and maintenance of effective disclosure and financial controls and procedures, internal control over financial reporting and changes in corporate governance practices, among many other complex rules that are often difficult to implement, monitor and maintain compliance with. Moreover, despite recent reforms made possible by the JOBS Act, the reporting requirements, rules, and regulations will make some activities more time-consuming and costly, particularly after we are no longer an "emerging growth company." In addition, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage that we had through BioXcel. Our management and other personnel will need to devote a substantial amount of time to ensure that we comply with all of these requirements and to keep pace with new regulations, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems.

If we fail to comply with the rules under the Sarbanes-Oxley Act of 2002 related to accounting controls and procedures in the future, or, if we discover material weaknesses and other deficiencies in our internal control and accounting procedures, our stock price could decline significantly and raising capital could be more difficult.

Section 404 of the Sarbanes-Oxley Act requires annual management assessments of the effectiveness of our internal control over financial reporting. If we fail to comply with the rules under the Sarbanes-Oxley Act related to disclosure controls and procedures in the future, or, if we discover material weaknesses and other deficiencies in our internal control and accounting procedures, our stock price could decline significantly and raising capital could be more difficult. If material weaknesses or significant deficiencies are discovered or if we otherwise fail to achieve and maintain the adequacy of our internal control, we may not be able to ensure that we can conclude on an ongoing basis that we have effective internal controls over financial reporting in accordance with Section 404 of the Sarbanes-Oxley Act. Moreover, effective internal controls are necessary for us to produce reliable financial reports and are important to helping prevent

financial fraud. If we cannot provide reliable financial reports or prevent fraud, our business and operating results could be harmed, investors could lose confidence in our reported financial information, and the trading price of our common stock could drop significantly.

Comprehensive tax reform bills could adversely affect our business and financial condition.

The U.S. government recently enacted comprehensive federal income tax legislation that includes significant changes to the taxation of business entities. These changes include, among others, a permanent reduction to the corporate income tax rate. Notwithstanding the reduction in the corporate income tax rate, the overall impact of this tax reform is uncertain, and our business and financial condition could be adversely affected. We urge our stockholders to consult with their legal and tax advisors with respect to any such legislation and the potential tax consequences of investing in our common stock.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our corporate headquarters are located at 555 Long Wharf Drive in New Haven, Connecticut. The Company occupies 11,040 square feet of space. The lease for this space is for a seven-year period with a renewal option for one additional five-year term. We believe that our existing facilities are suitable and adequate to meet our current needs. We intend to add new facilities or expand existing facilities as we add employees, and we believe that suitable additional or substitute space will be available as needed to accommodate any such expansion of our operations.

Item 3. Legal Proceedings

We are not a party to any material legal proceedings, and we are not aware of any claims or actions pending or threatened against us. In the future, we might from time to time become involved in litigation relating to claims arising from our ordinary course of business, the resolution of which we do not anticipate would have a material adverse impact on our financial position, results of operations or cash flows.

Item 4. Mine Safety Disclosures

Not applicable.

Part II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock is traded on the Nasdaq Capital Market under the symbol "BTAI."

Stockholders

As of March 2, 2020, there were 11 stockholders of record of our common stock. The actual number of holders of our common stock is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees.

Dividend Policy

We have never paid or declared any cash dividends on our common stock, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. We intend to retain all available funds and any future earnings to fund the development and expansion of our business. Any future determination to pay dividends will be at the discretion of our board of directors and will depend upon a number of factors, including our results of operations, financial condition, future prospects, contractual restrictions, restrictions imposed by applicable law and other factors that our board of directors deems relevant.

Sales of Unregistered Sales of Securities

During 2019, we issued 54,938 shares of our common stock upon the exercise of stock options previously granted under our equity incentive plan. The net proceeds to the Company from these option exercises was approximately $22,500. This securities issuance was in reliance on the exemption contained in Section 4(a)(2) of the Securities Act, as a transaction by issuers not involving a public offering.

Equity Compensation Plans

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 11. of Part III of this Annual Report.

Issuer Purchases of Equity Securities

In connection with the February 2020 registered offering of our common stock (the "primary offering"), and as disclosed in connection with the primary offering, we granted the underwriters in the primary offering the option to purchase up to 300,000 additional shares of our common stock, the net proceeds from which we would use to purchase the same number of shares of our common stock from BioXcel at the per share price paid by the underwriters in the primary offering. The underwriters exercised their option in full and, in February 2020, we purchased 300,000 shares of our common stock from BioXcel at a per share price of $30.08.

Item 6. Selected Financial Data

The following table presents our selected financial data and certain other financial data. The balance sheet data as of December 31, 2019 and 2018, and the results of operations data for the years then ended were derived from our audited financial statements included elsewhere in this Annual Report on Form 10-K. The balance sheet data as of December 31, 2017 and the results of operations data for the year then-ended have been derived from our audited consolidated financial statements not included in this Annual Report on Form 10-K. The financial data and other financial data presented below should be read in conjunction with our financial statements and the related notes thereto included elsewhere in this Annual Report on Form 10-K and with Part II, Item 7, "Management's Discussion and Analysis of

Financial Condition and Results of Operations" of this Annual Report on Form 10-K. The selected financial data in this section are not intended to replace our financial statements and the related notes. Our historical reposts are not indicative of our future results.

For the years ended December 31,	2019	2018
	(in thousands, except per share amounts)	
Results of operations data		
Revenues	$ —	$ —
Operating costs and expenses		
Research and development	25,797	14,558
General and administrative	7,804	5,404
Total operating expenses	33,601	19,962
Loss from operations	(33,601)	(19,962)
Other income		
Dividend and interest income, net	633	692
Net loss	$ (32,968)	$ (19,270)
Net loss per share attributable to common stockholders/ Parent basic and diluted	$ (2.02)	$ (1.32)
Weighted average shares outstanding - basic and diluted	16,289,175	14,571,553

As of December 31,	2019	2018
	(in thousands)	
Balance sheet data		
Cash and cash equivalents	$ 32,426	$ 42,565
Working capital	25,639	38,511
Total assets	36,392	43,549
Total stockholders' equity	26,895	38,889

Item 7. 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 "Selected Financial Data" and our financial statements and the related notes appearing elsewhere in this report. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those discussed below. Factors that could cause or contribute to such differences include, but are not limited to, those identified below, and those discussed in the section titled "Risk Factors" included elsewhere in this Annual Report on Form 10-K. All amounts in this report are in U.S. dollars, unless otherwise noted.

Overview

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed for treatment of a rare form of prostate cancer, pancreatic cancer and advanced solid cancers in combination with other immuno-oncology agents.

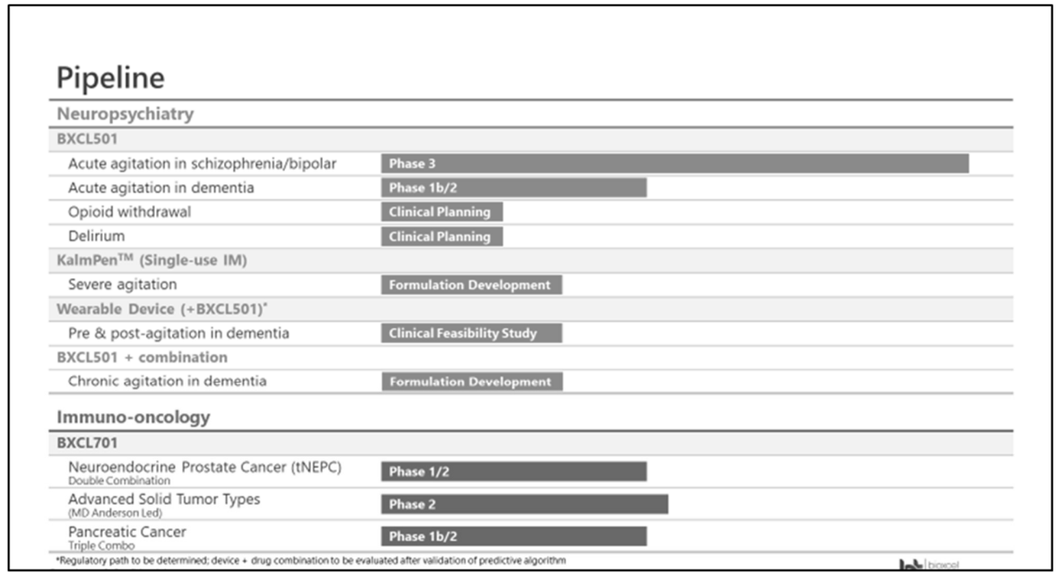
The Company's primary activities have been clinical and pre-clinical research and development of two its two most advanced programs: BXCL501, a sublingual thin film formulation of dexmedetomidine, or Dex, designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer and other solid tumors.

We intend to develop first-in-class, high value therapeutics by leveraging EvolverAI, a research and development engine created and owned by our parent, BioXcel. We believe the combination of our therapeutic area expertise and our ability to generate product candidates through our exclusive collaborative relationship with BioXcel in the areas of neuroscience and immuno-oncology gives us a significant competitive advantage. EvolverAI was developed over the last decade and integrates millions of fragmented data points using artificial intelligence, or AI and proprietary machine learning algorithms. After evaluating multiple product candidates using EvolverAI, we selected our lead programs because our analysis indicated these drugs may have utility in new therapeutic indices where there is substantial unmet medical needs and limited competition. By focusing on clinical candidates with relevant human data, we believe our approach will help us design more efficient clinical trials, thereby accelerating our product candidates' time to market. We retain global development and commercialization rights to these two programs.

We operate in a single segment. Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker in making decisions regarding resource allocation and assessing performance. To date, our chief operating decision maker has made such decisions and assessed performance at the company level, as one segment.

Our Clinical Programs

The following table summarizes our lead development programs:



Our Strategy

Our goal is to become a leader in the field of neuroscience and immuno-oncology. The key elements to achieving this goal are to:

- **Advance BXCL501, a sublingual thin film formulation of Dex, a selective α_{2a} adrenergic receptor agonist, designed for acute treatment of agitation, to approval through the Section 505(b)(2) pathway.**
    - **Neurological and Psychiatric Disorders.** We believe that BXCL501, if approved, has the potential to become the standard of care for the acute treatment of agitation arising from diseases such as schizophrenia, bipolar disorder, dementia, and other indications.
    - **Additional Indications.** We recently announced plans to evaluate BXCL501 in opioid withdrawal and we may also plan to evaluate additional indications for acute treatment of agitation resulting from delirium, alcohol withdrawal and post-traumatic stress disorder, or PTSD. Dex has been shown to significantly reduce agitation in elderly patients experiencing post-surgical delirium who did not respond to treatment with haloperidol, a potent major tranquilizer and antipsychotic that is used to treat symptoms for schizophrenia.
    - **Agitation Franchise Expansion.** We are also investigating potential treatments for the entire spectrum of agitation from pre-agitation to severe agitation. We are exploring the use of wearable digital device technology, such as the Apple watch, with the goal of prevention and treatment of agitation including, if approved, the administration of BXCL501 prior to the onset of agitation. Additionally, we are considering a combination approach of BXCL501 and another agent for the treatment of chronic agitation. For severe agitation, a single use intramuscular, or IM, injection called KalmPen™ is under development.

- **Complete BXCL701 Phase 2 trials to evaluate its potential for the first-line treatment of treatment-emergent neuroendocrine prostate cancer, or tNEPC, and for the second-line treatment of pancreatic cancer.**
    - **tNEPC (Orphan Segment of Prostate Cancer).** BXCL701 was previously studied in multiple clinical trials and demonstrated single agent anti-tumor activity in melanoma, an immune-sensitive tumor. The U.S. Food and Drug Administration, or FDA, authorized our investigational new drug application, or IND, allowing us to initiate a Phase 2 trial evaluating BXCL701 in combination with Pembrolizumab (KEYTRUDA®) in tNEPC, and this trial opened to accrual in February 2019 and continues enrolling patients.
    - **Pancreatic Cancer.** Preclinical data suggests that fibroblast activation protein positive, or FAP, contribute to checkpoint inhibitor resistance, and immunosuppression more generally in pancreatic cancer. We believe these data provide a strong rationale for combining BXCL701 with a checkpoint inhibitor such as avelumab (Bavencio) or nivolumab (Opdivo). Furthermore, we have observed synergy between BXCL701 and Bempegaldesleukin (Nektar's pegylated IL2), a CD122 based agonist of IL-2, in a preclinical pancreatic model. BXCL701 has been granted orphan drug designation by the FDA for the treatment of pancreatic cancer.
    - **Basket Trial**. BXCL701 is being evaluated in an open-label phase 2 basket trial led by MD Anderson. The investigator led study is designed to evaluate the response rate of orally administered BXCL701, combined with Pembrolizumab (KEYTRUDA®) in patients with advanced solid cancers. The study will evaluate both patients who are naïve to checkpoint therapy and those who are refractory to checkpoint therapy.
    - **Potential for Expedited Review Programs.** Given that these indications represent high unmet medical needs with few treatment options, we intend to pursue breakthrough therapy designation and accelerated approval for tNEPC and pancreatic cancer.
    - **Additional Indications.** We believe BXCL701 may be active at multiple stages of the cancer immunity cycle and therefore we believe BXCL701 offers a "pipeline in a product" platform given its potential for evaluation across other cancers. BXCL701 was granted an orphan drug designation for the treatment of acute myeloid leukemia in September 2019, its third orphan drug designation in addition to pancreatic cancer and melanoma. We believe existing preclinical evidence supports the combination of BXCL701 with checkpoint inhibitors and/or agents that act on "co-stimulatory" pathways within immune effector cells. Moreover, we believe agents that stimulate antibody dependent cell mediated cytotoxicity, (ADCC) or cell-based therapies such as chimeric antigen receptor T cell (CAR T) therapy, oncolytic viruses or therapeutic vaccines all represent potential combination with BXCL701.
- **Identify biomarkers to select patients who we believe have the highest likelihood to respond to our product candidates.** Predicting optimal drug responses in patients requires the identification and validation of predictive biomarkers. We believe that our ability to identify patient subsets most likely to respond to our product candidates will increase the clinical benefit to patients and improve the probability of success of our clinical trials. The indications for our lead product candidate BXCL701 were chosen in part because they are known to overexpress dipeptidyl peptidase, or DPP 8/9, and FAP. Our planned proof-of-concept clinical trial of BXCL701 will retrospectively examine biomarkers related to its molecular and cellular targets to identify those that may correlate with clinical efficacy and increase our likelihood of success.
- **Enhance our R&D pipeline by leveraging our therapeutic area expertise with EvolverAI to identify, develop and commercialize new product candidates in neuroscience and immuno-oncology.** In addition to our leading clinical programs and our emerging and future pipeline, we intend to select our next clinical program during 2020. We have established translational and development expertise, which we believe will help us advance the present and future product candidates in these fields. We may also

opportunistically in-license additional product candidates identified through our AI platform approach within our core areas of expertise.

- **Maximize the commercial potential of our product candidates.** We have worldwide development and commercialization rights to our BXCL501 and BXCL701. If BXCL501 and BXCL701 are approved in the United States, we would consider building a specialty sales force in the United States and/or collaborate with third parties to maximize the potential of our product candidates. Furthermore, we intend to commercialize BXCL501 and BXCL701, if approved, outside the United States through collaborations with third parties.

Our Novel Drug Re-Innovation Approach

Our AI-based discovery and development process is the foundation of our drug re-innovation model for identifying the next wave of medicines. Our therapeutic area experts have over 60 years of experience across the drug discovery and development value chain. We believe EvolverAI is a novel method of finding potential product candidates because it combines the comprehensiveness and efficiency of machine learning and big data analytics with the expertise and intuition of human experience in drug development. We believe the combination of our therapeutic area expertise and our ability to generate therapeutic candidates in neuroscience and immuno-oncology through our exclusive collaborative relationship in those areas with BioXcel give us a significant competitive advantage.

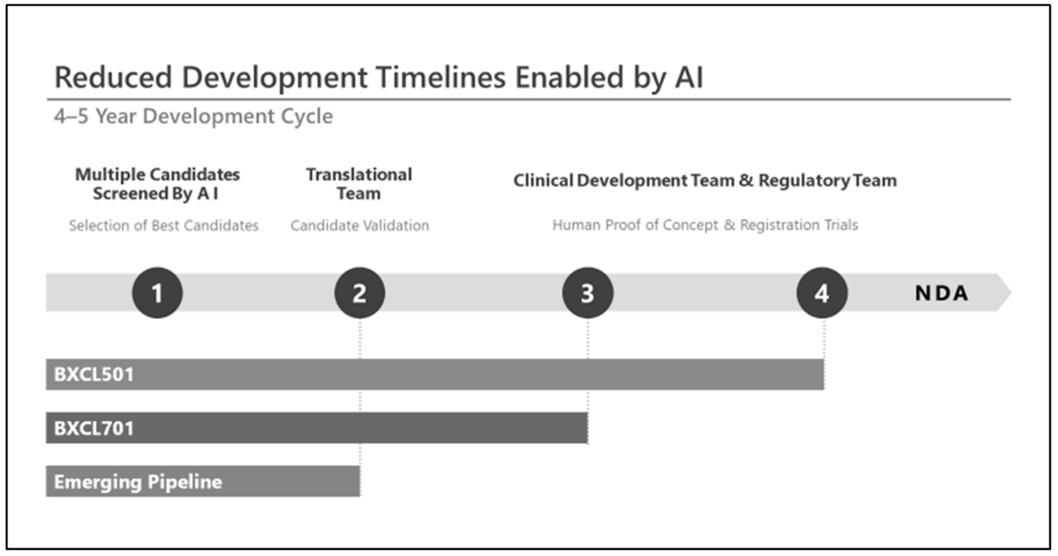
The pharmacological space spans more than 27,000 active pharmaceutical agents and only approximately 4,000 are approved and marketed drugs benefiting patients. These marketed drugs may be applied to other indications, including rare diseases, and represent an untapped potential for meeting significant unmet medical need and recoupment of research and development investments. A large number of the remaining agents are clinical candidates that are active, shelved or have failed for reasons other than toxicity and can potentially be re-engineered for different indications or patient segments. They potentially represent an unrealized investment of billions of research and development dollars by the private and public sectors, resulting in an immeasurable amount of patient suffering and sacrificing during clinical development.

Traditional drug development is plagued with low success rates (13.8%, according to an MIT study of 186,000 trials from January 2000 to October 2015), long drug development cycles (10-15 years, according to PhRMA Key Facts 2016) and exorbitant development costs ($2.6 billion per drug, according to PhRMA Key Facts). Furthermore, many serious diseases continue to go unaddressed due to limitations of the current drug discovery paradigm. The recent advent of numerous ‘omics’ technologies (genomics, proteomics) and rapid advances in science and medicine are generating terabytes of valuable unexploited knowledge that is widely distributed in multiple big data lakes with several orders of complexity and variety. Much of this data is not being systematically applied to the development of next generation therapeutics, thus preventing the optimization of drug development utilizing the understanding of technology, science, medicine, markets and commercial opportunities. The efficient and intuitive use of big data remains a bottleneck and a challenge to the pharmaceutical industry. Taken together, these factors underscore the need for fundamental new approaches to drug discovery and development. The market opportunity to identify new uses for existing pharmacological agents remains substantial, due to the lack of technology driven insights. Our parent, BioXcel, has created a proprietary R&D engine, EvolverAI, for drug re-innovation that provides a proprietary systems-based approach designed to unlock the hidden value in drugs. The combination of our therapeutic area expertise and our exclusive collaborative relationship with BioXcel enables us to screen, analyze, and identify the product candidates that we believe have a high likelihood of benefiting patients. The compounds in our pipeline have been identified using this proprietary platform.

EvolverAI is designed to eliminate human bias by scanning millions of data points from disparate data sources to create network maps. The nodes and connections in the network map are weighted and ranked based on the validity of supporting evidence using disease specific algorithms. They are then further analyzed using artificial intelligence and machine learning approaches supplemented by human domain-based expertise to uncover novel connections between disease parameters, molecular targets, mechanisms of actions and product candidates.

This drug re-innovation model has been exemplified by the successful development and commercialization of drugs such as Tecfidera (Biogen, Inc.), Thalomid (Celgene Corporation) and Viagra (Pfizer, Inc.). All of these drugs were identified by insights in biology and disease pathophysiology. The successful business models of biotech companies like Axsome-Therapeutics, Inc. and Karuna Therapeutics, Inc. are based on the re-innovation and combination of existing clinical candidates or marketed drugs to provide novel solutions for patients. Unfortunately, such discoveries have been severely limited in scope due to the lack of a genuinely integrated big data analytics based approach.

We believe that only EvolverAI allows a comprehensive and unbiased evaluation of the complete pharmacological space. We believe our drug re-innovation model and exclusive collaborative relationship with BioXcel has the potential to reduce the cost and time of drug development, help us design more efficient trials and accelerate our product candidates’ time to market. This assumption is based on capitalizing product candidates with substantial clinical data and mitigated risk due to well defined safety profiles, known PK/PD properties, and an established manufacturing and regulatory path. Our approach is illustrated below:



Basis of Presentation

The Company's financial statements are prepared in accordance with Generally Accepted Accounting Principles in the United States of America ("GAAP"). All amounts are presented in thousands.

Components of Our Results of Operations

Revenues

We have not recognized any revenue since inception.

Operating Costs and Expenses

Research and Development

Research and development expenses consist primarily of costs incurred for the research and development of our clinical and pre-clinical product candidates, which includes payments to BioXcel, our Parent.

- employee-related expenses, including salaries, benefits and stock-based compensation expense and travel expenses for employees engaged in research and development functions;
- expenses incurred under agreements with contract research organizations, or CROs, and sites that conduct our non-clinical studies and clinical trials;
- costs of outside consultants engaged in research and development activities, including their fees, stock-based compensation and travel expenses;
- the cost of acquiring, developing and manufacturing pre-clinical and clinical trial materials and lab supplies; and
- depreciation and other expenses.

We expense research and development costs to operations as incurred.

Our research and development costs by program for the year ended December 31, 2019 and 2018 are as follows:

	2019	2018
BXCL 501	$ 15,859	$ 6,051
BXCL 701	6,947	5,984
BXCL 502	331	75
BXCL 702	350	105
Other research and development programs	708	573
Research and development support services	1,602	1,770
Total research and development expenses	$ 25,797	$ 14,558

General and Administrative

General and administrative expenses consist primarily of personnel costs, including salaries, benefits, stock-based compensation and travel expenses for our executive, finance, corporate development and other administrative functions. General and administrative expenses also include legal expenses to pursue patent protection of our intellectual property, professional fees for audit and tax and insurance charges.

We expect that our general and administrative expenses will increase as we expand our clinical programs. We expect increased administrative costs resulting from our clinical trials and the potential commercialization of our product candidates. We believe that these increases will likely include increased costs for director and officer liability insurance, hiring additional personnel to support future market research and future product commercialization efforts and increased fees for outside consultants, attorneys and accountants. We also expect to incur increased costs to comply with corporate governance, internal controls, investor relations and disclosures and similar requirements applicable to public companies.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is set forth in Note 4 to the financial statements included in this Annual Report on Form 10-K.

Results of Operations

For a discussion of our results of operations for the year ended December 31, 2017, including a year-to-year comparison between 2018 and 2017, refer to Part II, Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the year ended December 31, 2018.

Comparison of the Years Ended December 31, 2019 and 2018

The following table summarizes our results of operations for the years ended December 31, 2019 and 2018:

	Year Ended December 31,		
	2019	2018	Change
		(in thousands)	
Operating costs and expenses			
Research and development	$ 25,797	$ 14,558	$ 11,239
General and administrative	7,804	5,404	2,400
Total operating expenses	33,601	19,962	13,639
Loss from operations	(33,601)	(19,962)	(13,639)
Other expense			
Dividend and interest income, net	633	692	(59)
Net loss	$ (32,968)	$ (19,270)	$ (13,698)

Research and Development Expense

Research and development expenses for the years ended December 31, 2019 and 2018 were $25,797 and $14,558, respectively. The increase of $11,239 is attributable to the costs described in the table below:

	Year Ended December 31,		
	2019	2018	Change
Salaries, bonus & related costs	$ 5,648	$ 3,086	$ 2,562
Non-cash stock-based compensation	1,791	1,843	(52)
Professional research & project related costs	4,456	2,240	2,216
Drug acquisition costs	1,000	1,000	—
Clinical trials expense	7,904	3,851	4,053
Chemical, manufacturing and controls cost	3,429	1,693	1,736
All other	1,569	845	724
Total research and development expenses	$ 25,797	$ 14,558	$ 11,239

Salaries, bonus and related costs increased due to higher bonus accruals, increases in headcount, payroll taxes, recruiting fees and travel related costs.

Non-cash stock-based compensation decreased due to the adoption of FASB ASU 2018-07 as of January 1, 2019 which allowed non-employee options to be expensed using the adoption date fair value. The adoption date value of the stock price was significantly lower than prior re-measurement dates. In addition, several large option grants became fully

vested during the first quarter of 2018 and there was no corresponding charge during the first quarter of 2019. These lower charges were offset in part by increases in expense relating to new hires beginning in the second quarter of 2018.

Drug acquisition costs are related to certain payments triggered pursuant to our Amended and Restated Asset Contribution Agreement with our Parent as discussed in Note 5 to the financial statements included elsewhere in this Annual Report on Form 10-K.

The increase in professional research & project related costs, clinical trials expenses and chemical, manufacturing and controls cost reflect the acceleration of research and development activities.

All other increased due to higher technical service charges from BioXcel, and increased depreciation and amortization charges related to our new leased office space.

General and Administrative Expense

General and administrative expenses for the years ended December 31, 2019 and 2018 were $7,804 and $5,404, respectively. The increase of $2,400 is attributable to the costs described in the table below:

	Year Ended December 31,		
	2019	2018	Change
Salaries, bonus & related costs	$ 2,334	$ 1,409	$ 925
Non-cash stock-based compensation	1,351	1,239	112
Professional fees	2,523	1,745	778
Insurance	902	681	221
All other	694	330	364
Total general and administrative expenses	$ 7,804	$ 5,404	$ 2,400

Salaries, bonus and related costs increased due to increases in headcount, higher bonus accruals, payroll taxes, recruiting fees and travel related costs.

Non-cash stock-based compensation increased slightly in 2019.

Professional fees increased due to expanding operations and operating as a public company. Higher legal, audit, investor relations, licensing and information technology costs were incurred during 2019.

Insurance costs increased primarily due to increased costs for Director and Officer liability insurance.

All other expenses increased due to additional franchise taxes, office space costs and regulatory filing fees.

Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during the periods presented.

Liquidity and Capital Resources

As of December 31, 2019, we had cash and cash equivalents of $32,426, working capital of $25,639 and stockholders' equity of $26,895. Net cash used in operating activities was $27,280 and $13,509 for the years ended December 31, 2019 and 2018. We incurred losses of approximately $32,968 and $19,270 for the years ended December 31, 2019 and 2018. We have not yet generated any revenues and we have not yet achieved profitability. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need to generate significant product revenues to achieve profitability. We believe that our existing cash and cash

equivalents as of December 31, 2019, and a review of projected project timing, will enable us to fund our operating expenses and capital expenditure requirements for at least one year from the date of this Annual Report on Form 10-K.

Management's plans to obtain additional resources for the Company include obtaining capital from the sale of its equity securities, entering into strategic partnership arrangements and short-term borrowings from banks, stockholders or other related parties, if needed. However, management cannot provide any assurance that the Company will be successful in accomplishing any of its plans.

Sources of Liquidity

We have focused our efforts on raising capital and building the products in our pipeline. Since our inception, and through our initial public offering of our common stock, or IPO, all our operations have been financed by our Parent, BioXcel, or the sales of our common stock in a series of private placements, three public offerings and an Open Market Sale Agreement. We have not yet established an ongoing source of revenue sufficient to cover our operating costs and will need to do so in future periods.

In January and February 2018, the Company issued 283,452 shares of common stock with an issuance price of $6.88 per share for gross and net proceeds of $1,950.

In March 2018, we completed our IPO and we issued and sold 5,454,545 shares of common stock at a public offering price of $11.00 per share. Gross proceeds totaled $60,000, and net proceeds totaled $54,102.

In May 2019, we entered into an Open Market Sale Agreement, or the Sale Agreement, with Jefferies LLC, or Jefferies, pursuant to which we could offer and sell shares of our common stock having an initial offering price no greater than $20.0 million, from time to time, through an "at the market offering" program under which Jefferies would act as sales agent. From May 2019 to September 2019, we sold a total of 66,193 shares for gross proceeds of $737 and net proceeds of $387. We terminated the Sale Agreement on September 22, 2019.

In September 2019, we sold in a registered offering 2,303,030 shares of our common stock at a public offering price of $8.25 per share, less underwriting discounts and commissions for which we received gross and net proceeds of approximately $19,000 and $17,423, respectively.

In February 2020, we sold in a registered offering 2,000,000 shares of our common stock at a public offering price of $32.00 per share for gross proceeds of $64,000 and net proceeds of approximately $60,000.

Cash Flows

	Year Ended December 31,	
(in thousands)	2019	2018
Cash provided by (used in) in thousands:		
Operating activities	$ (27,280)	$ (13,509)
Investing activities	(870)	(340)
Financing activities	18,011	55,527

Operating Activities

For the year ended December 31, 2019, net cash used in operating activities was approximately $27,280 which consisted of a net loss of $32,968 partially offset by $3,142 in stock-based compensation and $156 of depreciation and amortization. Increases in accounts payable and accrued expenses of $3,580 were offset in part by increases in prepaid expenses (primarily for insurance premiums) and other assets of $1,190.

For the year ended December 31, 2018, net cash used in operating activities was approximately $13,509 which consisted of a net loss of $19,270 partially offset by $3,082 in stock-based compensation and $17 of depreciation.

Increases in accounts payable and accrued expenses of $3,201 were offset in part by increases in prepaid expenses (primarily for insurance premiums) and other assets of $539.

Investing Activities

Net cash used in investing activities was $870 for the year ended December 31, 2019, compared to $340 for the year ended December 31, 2018. Expenditures for 2019 were primarily related to construction costs and furniture associated with the occupancy of the Company's 12th floor office at 555 Long Wharf Drive in New Haven, CT. Expenditures in 2018 were primarily for design costs associated with the 12th floor occupancy. We also purchased computers and related equipment for technical research and for additional headcount during 2019 and 2018.

Financing Activities

Net cash provided by financing activities was $18,011 for the year ended December 31, 2019. Our September 2019 Offering provided funds of $17,423, net of $1,577 of closing costs. In addition, our ATM program provided funds of $387, net of $350 of closing costs. The two equity issuances accounted for the majority of our financing activities.

Net cash provided by financing activities was approximately $55,527 for the year ended December 31, 2018 which was mainly attributable to the proceeds from issuance of common stock in our IPO and private placements of common stock, offset in part by repayment of loans to our Parent.

Operating Capital and Capital Expenditure Requirements

We expect to continue to incur significant and increasing operating losses at least for the next several years as we expand our clinical trials of BXCL501 and BXCL701, seek marketing approval for our product candidates and pursue development of our other product candidates. We do not expect to generate revenue unless and until we successfully complete development and obtain regulatory approval for our product candidates. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our planned clinical trials and our expenditures on other research and development activities.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. We anticipate that our expenses will increase substantially as we:

- continue our clinical development of BXCL501 and BXCL701;
- conduct additional research and development with our product candidates;
- seek to identify, acquire, develop and commercialize additional product candidates;
- integrate acquired technologies into a comprehensive regulatory and product development strategy;
- maintain, expand and protect our intellectual property portfolio;
- hire scientific, clinical, quality control and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our drug development efforts;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any product candidates for which we may obtain regulatory approval; and
- continue to operate as a public company.

We expect that we will need to obtain substantial additional funding in order to complete our clinical trials. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that

include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of BXCL501, BXCL701 or other product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to BXCL501, BXCL701 or other product candidates that we otherwise would seek to develop or commercialize ourselves.

Critical Accounting Policies

The preparation of our financial statements in conformity with GAAP requires management to exercise its judgment. We exercise considerable judgment with respect to establishing sound accounting policies and in making estimates and assumptions that affect the reported amounts of our assets and liabilities, our recognition of revenues and expenses, and disclosure of commitments and contingencies at the date of the financial statements.

On an ongoing basis, we evaluate our estimates and judgments. We base our estimates and judgments on a variety of factors including our historical experience, knowledge of our business and industry, current and expected economic conditions, the attributes of our products, the regulatory environment, and in certain cases, the results of outside appraisals. We periodically re-evaluate our estimates and assumptions with respect to these judgments and modify our approach when circumstances indicate that modifications are necessary.

While we believe that the factors we evaluate provide us with a meaningful basis for establishing and applying sound accounting policies, we cannot guarantee that the results will always be accurate. Since the determination of these estimates requires the exercise of judgment, actual results could differ from such estimates.

A description of significant accounting policies that require us to make estimates and assumptions in the preparation of our financial statements is as follows:

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States of America requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosures of contingent liabilities at the dates of the financial statements and the reported amounts of revenues and expenses during the reporting periods. Actual results could differ from these estimates.

Research and Development

Research and development expenses are expensed as incurred. Patent costs and patent acquisition costs are expensed as incurred and included in general and administrative expenses.

Stock-based Compensation

Charges from our Parent, BioXcel Corporation.

BioXcel has granted stock options to its employees under its own equity incentive plan, or the BioXcel Plan. Stock-based compensation expense from awards granted under the BioXcel Plan is allocated to us over the required service period over which those stock option awards vest and is based upon the percentage of time the award recipient spent working on our activities compared to BioXcel activities, which is the same basis used for allocation of salary costs.

The BioXcel stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of these BioXcel stock option awards was determined using the

Black Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of these awards, as they are not publicly traded.

Our estimation of fair value of the awards considered recent transactions entered into by BioXcel, relevant industry and comparable public company data. Since BioXcel is a non-public entity, the majority of the inputs used to estimate the fair value of the common stock option awards are considered level 3 due to their unobservable nature. Each option award is subject to specified vesting schedules and requirements (a mix of time-based and corporate event-based, including financing events). Compensation expense is charged to us by BioXcel over the required service period to earn the award which is expected to be up to four years, subject to the achievement of time and event-based vesting requirements.

BioXcel Therapeutics, Inc. 2017 Equity Incentive Plan

Our board of directors adopted the 2017 Equity Incentive Plan, or the Plan, on August 22, 2017. The Plan will expire on August 22, 2027. The purpose of the Plan is to attract and retain key personnel and to provide a means for directors, officers, managers, employees, consultants and advisors to acquire and maintain an interest in our company, which interest may be measured by reference to the value of its common stock.

The Company accounts for stock-based compensation in accordance with ASC 718, "*Compensation—Stock Compensation,*" which requires the measurement and recognition of compensation expense based on estimated fair market values for all share-based awards made to employees and directors, including stock options. The Company's stock-based compensation plan was adopted and became effective in August 2017. Prior to the Company adopting its stock-based compensation plan the Parent granted stock options to its employees. As a result, related stock-based compensation expense has been allocated to the Company over the required service period over which these BioXcel stock option awards vest in the same manner salary costs of employees have been allocated to the BTI Business in the carve-out process.

Both BioXcel and the Company's stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of stock option awards was determined using the Black-Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of these awards, as they were not publicly traded. Stock awards granted by the Company subsequent to the IPO are valued using market prices at the date of grant.

The Company adopted FASB ASU 2018-07 as of January 1, 2019 which allowed non-employee options to be expensed using the adoption date fair value.

The Company adopted FASB ASU 2016-09 as of January 1, 2018 and has elected to account for forfeitures as they occur, by reversing compensation cost when the award is forfeited.

The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different.

Expenses Accrued Under Contractual Arrangements with Third Parties; Accrued Clinical Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and contract research organizations that conduct and manage clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing expenses, we estimate the time period over which services will be performed and the level of effort to be expended in each period, which is based on an established protocol specific to each clinical trial. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual accordingly. 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 relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period.

JOBS Act

On April 5, 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies.

We have chosen to opt out of the extended transition periods available to emerging growth companies under the JOBS Act for complying with new or revised accounting standards. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition periods for complying with new or revised accounting standards is irrevocable.

Contractual Obligations and Commitments

The Company entered into an agreement to lease approximately 11,040 square feet of space on the 12th floor of the building located at 555 Long Wharf Drive, New Haven, Connecticut that commenced February 22, 2019 (the "Commencement Date"). The premises were occupied in March 2019.

The term of the 12th floor lease continues from the Commencement Date through the last day of the calendar month immediately following the seventh anniversary of the Commencement Date.

The following table summarizes our contractual obligations at December 31, 2019 and the effect such obligations are expected to have on our liquidity and cash flow in future periods and is solely related to the 12th floor lease:

	Payments due by Period				
	Total	Less Than 1 year	1-3 years	3-5 years	More Than 5 years
Operating lease commitments	$ 1,354	$ 208	$ 415	$ 455	$ 276

For additional details, see "Note 12 to Financial Statements – Leases."

Off-Balance Sheet Arrangements

We did not have during the periods presented, nor do we currently have, any off-balance sheet arrangements as defined under SEC rules.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

Our balance sheet as of December 31, 2019 includes cash and cash equivalents of $32,426. We do not participate in any foreign currency hedging activities and we do not have any other derivative financial instruments. We did not recognize any significant exchange rate losses during the years ended December 31, 2019 and 2018, respectively.

We do not believe that our cash and cash equivalents have significant risk of default or illiquidity. While we believe our cash and cash equivalents does not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash at one or more financial institutions that are in excess of federally insured limits.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during the periods presented.

Item 8. Financial Statements and Supplementary Data

The financial statements required to be filed pursuant to this Item 8 are appended to this Annual Report on Form 10-K and incorporated herein by reference.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2019. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on the evaluation of our disclosure controls and procedures as of December 31, 2019, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures are effective at the reasonable assurance level.

Management's Annual Report on Internal Controls Over Financial Reporting

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our management conducted an assessment of the effectiveness of our internal control over financial reporting based on the criteria set forth in "Internal Control–Integrated Framework (2013)" issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on this assessment, management concluded that, as of December 31, 2019, our internal control over financial reporting was effective.

Attestation Report of the Registered Public Accounting Firm

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm on internal control over financial reporting due to an exemption established by the JOBS Act for "emerging growth companies."

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the three months ended December 31, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to our annual meeting of stockholders to be held in 2020 (the "2020 Annual Meeting of Stockholders"), which we intend to file with the SEC within 120 days of the year ended December 31, 2019.

Item 11. Executive Compensation

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2020 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the year ended December 31, 2019.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2020 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the year ended December 31, 2019.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2020 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the year ended December 31, 2019.

Item 14. Principal Accounting Fees and Services

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2020 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the year ended December 31, 2019.

PART IV

Item 15. Exhibits, Financial Statement Schedules

(a) The following documents are filed as part of this report:

(1) Financial Statements:

(2) Financial Statement Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits.

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
3.1	Amended and Restated Certificate of Incorporation.	8-K	001-38410	3.1	3/13/2018	
3.2	Amended and Restated Bylaws	8-K	001-38410	3.2	3/13/2018	
4.1	Description of the Registrant's Securities Registered Under Section 12 of the Exchange Act					*
4.2	Specimen Stock Certificate evidencing the shares of common stock	S-1/A	333-222990	4.2	2/26/2018	
10.1	Stock Purchase Agreement, dated February 18, 2020, between BioXcel Corporation and BioXcel Therapeutics, Inc.	8-K	001-38410	10.1	2/21/2020	
10.2^	Second Amended and Restated Separation and Shared Services Agreement, dated March 6, 2020, by and between BioXcel Corporation and BioXcel Therapeutics, Inc.					*
10.3#	Amended and Restated Asset Contribution Agreement, effective November 7, 2017, by and between BioXcel Corporation and BioXcel Therapeutics, Inc.	S-1/A	333-222990	10.2	2/12/2018	
10.4	Collaborative Research Agreement, dated August 27, 2017, by and between BioXcel Therapeutics, Inc. and Nektar Therapeutics	S-1/A	333-222990	10.13	2/26/2018	

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
10.5#	Clinical Trial Collaboration Agreement, dated September 21, 2018, by and between BioXcel Therapeutics, Inc. and Nektar Therapeutics	10-Q	001-38410	10.1	11/09/2018	
10.6	Lease Agreement, dated as of August 20, 2018, by and between Fusco Harbour Associates, LLC, as Landlord, and BioXcel Therapeutics, Inc., as Tenant	8-K	001-38410	10.1	8/23/2018	
10.7†	2017 Equity Incentive Plan	S-1/A	333-222990	10.3	2/12/2018	
10.8†	Form of Incentive Stock Option Agreement under the 2017 Equity Incentive Plan	S-1/A	333-222990	10.4	2/12/2018	
10.9†	Form of Non-Statutory Stock option Agreement under the 2017 Equity Incentive Plan	S-1/A	333-222990	10.5	2/12/2018	
10.10†	Form of Indemnification Agreement with directors and executive officers	S-1/A	333-222990	10.6	2/12/2018	
10.11†	Employment Agreement, dated March 7, 2018 by and between BioXcel Therapeutics, Inc. and Vimal Mehta	8-K	001-38410	10.1	3/13/2018	
10.12†	Employment Agreement, dated February 12, 2018, by and between BioXcel Therapeutics, Inc. and Frank Yocca	S-1/A	333-222990	10.11	2/12/2018	
10.13†	Employment Agreement, effective October 2, 2017, by and between BioXcel Therapeutics, Inc. and Richard Steinhart	S-1/A	333-222990	10.12	2/12/2018	
10.14†	Employment Agreement, dated June 1, 2018, by and between BioXcel Therapeutics, Inc. and Dr. Vincent O'Neill, M.D.	8-K	001-38410	10.1	6/07/2018	
21.1	Subsidiaries of BioXcel Therapeutics, Inc.			21.1		*
23.1	Consent of BDO USA, LLP			23.1		*
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.			31.1		*

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.			31.2		*
32.1	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.			32.1		**
32.2	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.			32.2		**
101.INS	XBRL Instance Document					*
101.SCH	XBRL Taxonomy Extension Schema Document					*
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document					*
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document					*
101.LAB	XBRL Taxonomy Extension Label Linkbase Document					*
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document					*

† Indicates a management contract or any compensatory plan, contract or arrangement.
^ Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.
# Confidential treatment has been granted for portions omitted from this exhibit and those portions have been separately filed with the Securities and Exchange Commission.
* Filed herewith.
** Furnished herewith.

Item 16. Form 10-K Summary

Not applicable

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

BioXcel Therapeutics, Inc.

Dated: March 9, 2020

By:
/s/ Vimal Mehta
Vimal Mehta
Chief Executive Officer
(Principal Executive Officer)

Dated: March 9, 2020

By:
/s/ Richard Steinhart
Richard Steinhart, Chief Financial Officer
(Principal Financial Officer)

Signature	Title	Date
/s/ VIMAL MEHTA Vimal Mehta, Ph.D.	Chief Executive Officer, President, Secretary and Director *(Principal Executive Officer)*	March 9, 2020
/s/ RICHARD STEINHART Richard Steinhart	Chief Financial Officer *(Principal Financial Officer and Principal Accounting Officer)*	March 9, 2020
/s/ PETER MUELLER Peter Mueller, Ph.D.	Chairman of the Board of Directors	March 9, 2020
/s/ KRISHNAN NANDABALAN Krishnan Nandabalan, Ph.D.	Director	March 9, 2020
/s/ SANDEEP LAUMAS Sandeep Laumas, M.D.	Director	March 9, 2020
/s/ MICHAL VOTRUBA Michal Votruba	Director	March 9, 2020

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Stockholders and Board of Directors of BioXcel Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of BioXcel Therapeutics, Inc. (the "Company") as of December 31, 2019 and 2018, and the related statements of operations, changes in stockholders' equity (deficit), and cash flows for each of the two years in the period ended December 31, 2019, 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, 2019 and 2018, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2019, in conformity with accounting principles generally accepted in the United States of America.

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/ BDO USA, LLP

We have served as the Company's auditor since 2017.

Stamford, Connecticut

March 9, 2020

BIOXCEL THERAPEUTICS, INC.

BALANCE SHEETS

(amounts in thousands, except share and per share data)

	December 31, 2019	December 31, 2018
ASSETS		
Current assets		
Cash and cash equivalents	$ 32,426	$ 42,565
Prepaid expenses and other current assets	1,681	491
Due from Parent	—	115
Total current assets	34,107	43,171
Property and equipment, net	1,041	327
Operating lease right-of-use asset	1,193	—
Other assets	51	51
Total assets	**$ 36,392**	**$ 43,549**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 4,953	$ 1,604
Accrued expenses	3,120	3,056
Due to Parent	64	—
Other current liabilities	331	—
Total current liabilities	8,468	4,660
Operating lease liability	1,029	—
Total liabilities	**9,497**	**4,660**
Stockholders' equity		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value, 50,000,000 shares authorized; 18,087,382 and 15,663,221 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	18	16
Additional paid-in-capital	83,565	62,593
Accumulated deficit	(56,688)	(23,720)
Total stockholders' equity	26,895	38,889
Total liabilities and stockholders' equity	**$ 36,392**	**$ 43,549**

The accompanying notes are an integral part of these financial statements.

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

(amounts in thousands, except share and per share data)

	2019	2018
Revenues	$ —	$ —
Operating costs and expenses		
Research and development	25,797	14,558
General and administrative	7,804	5,404
Total operating expenses	33,601	19,962
Loss from operations	(33,601)	(19,962)
Other income		
Dividend and interest income, net	633	692
Net loss	$ (32,968)	$ (19,270)
Net loss per share attributable to common stockholders/ Parent basic and diluted	$ (2.02)	$ (1.32)
Weighted average shares outstanding - basic and diluted	16,289,175	14,571,553

The accompanying notes are an integral part of these financial statements.

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY (DEFICIT)

(amounts in thousands, except share and per share data)

	Common Stock		Additional Paid in	Accumulated	
	Shares	Amount	Capital	Deficit	Total
Balance as of January 1, 2018	9,907,548	$ 10	$ 3,458	$ (4,450)	$ (982)
Issuance of common stock	283,452	1	1,949	—	1,950
Issuance of common stock, upon completion of Initial Public Offering, net of issuance costs of $5,898	5,454,545	5	54,097	—	54,102
Stock-based compensation	—	—	3,082	—	3,082
Exercise of stock options	17,676	—	7	—	7
Net loss	—	—	—	(19,270)	(19,270)
Balance as of December 31, 2018	15,663,221	$ 16	$ 62,593	$ (23,720)	$ 38,889
Issuance of common stock, net of issuance costs of $1,991	2,369,223	$ 2	$ 17,808	$ —	$ 17,810
Stock-based compensation		—	3,142	—	3,142
Exercise of stock options	54,938	—	22	—	22
Net loss		—	—	(32,968)	(32,968)
Balance as of December 31, 2019	18,087,382	$ 18	$ 83,565	$ (56,688)	$ 26,895

The accompanying notes are an integral part of these financial statements.

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amounts in thousands, except share and per share data)

	Year ended December 31, 2019	Year ended December 31, 2018
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (32,968)	$ (19,270)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	156	17
Stock-based compensation expense	3,142	3,082
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,190)	(539)
Accounts payable, accrued expenses and other liabilities	3,580	3,201
Net cash used in operating activities	(27,280)	(13,509)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of equipment	(870)	(340)
Net cash used in investing activities	(870)	(340)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	17,810	56,513
Exercise of options	22	7
Payable to Parent for services	—	(67)
Due to Parent	179	(555)
Note Payable — Parent	—	(371)
Net cash provided by financing activities	18,011	55,527
Net (decrease) increase in cash and cash equivalents	(10,139)	41,678
Cash and cash equivalents, beginning of the period	42,565	887
Cash and cash equivalents, end of the period	$ 32,426	$ 42,565
Supplemental cash flow information:		
Interest paid	$ 62	$ 1
Supplemental disclosure of non-cash Operating, Investing and Financing Activities:		
Deferred issuance costs reclassified to additional paid-in-capital upon completion of initial public offering	$ —	$ 461
Right-of-use asset obtained in exchange for new operating lease liability	$ 1,308	—

The accompanying notes are an integral part of these financial statements.

BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS

(amounts in thousands, except share and per share data)

Note 1. Organization and Principal Activities

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed for treatment of a rare form of prostate cancer, pancreatic cancer and advanced solid cancers in combination with other immuno-oncology agents. The Company's primary activities have been clinical and pre-clinical research and development for BXCL501 and BXCL701.

As used in these financial statements, unless otherwise specified or the context otherwise requires, the terms the "Company" or "BTI" refer to BioXcel Therapeutics, Inc., and "BioXcel" or "Parent" refer to BioXcel Corporation, the Company's parent.

The Company is a majority-owned subsidiary of BioXcel Corporation, also referred to as BioXcel or Parent, and was incorporated under the laws of the State of Delaware on March 29, 2017. The Company's principal office is in New Haven, Connecticut.

Note 2. Initial Public Offering

On March 7, 2018, the Company's registration statement on Form S-1 relating to its initial public offering of its common stock, or the IPO, was declared effective by the Securities and Exchange Commission, or the SEC. The IPO closed on March 12, 2018, and the Company issued and sold 5,454,545 shares of common stock at a public offering price of $11.00 per share. Gross proceeds totaled $60,000 and net proceeds totaled $54,102 after deducting underwriting discounts and commissions of $4,200 and other offering expenses of approximately $1,698.

In connection with and immediately prior to the closing of its IPO, the Company effectuated a 237 to one stock split. Accordingly, all share and per share amounts for all periods presented in the accompanying financial statements have been adjusted retroactively, where applicable, to reflect the stock split.

Also, in connection with the completion of its IPO, the Company amended its articles of incorporation to authorize the issuance of up to 50,000,000 shares of common stock with a par value of $.001 each and 10,000,000 shares of preferred stock with a par value of $.001 each.

Note 3. Basis of Presentation

The Company's financial statements are prepared in accordance with Generally Accepted Accounting Principles in the United States of America ("GAAP").

Note 4. Summary of Significant Accounting Policies

Use of Estimates

The Company's financial statements are prepared in accordance with GAAP. The preparation of the Company's financial statements requires it to make estimates and assumptions that impact the reported amounts of assets, liabilities and expenses in its financial statements and the accompanying notes. The most significant estimates in the financial

statements relate to the fair value of equity awards and valuation allowance related to the Company's deferred tax assets. Although these estimates are based on the Company's knowledge of current events and actions it may undertake in the future, actual results may ultimately materially differ from these estimates.

Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. As of December 31, 2019 and 2018, cash equivalents were comprised of money market funds. Cash and cash equivalents held at financial institutions may at times exceed federally insured amounts. We believe we mitigate such risk by investing in or through major financial institutions.

Deferred Offering Costs

The Company capitalized certain legal, professional accounting and other third-party fees that were directly associated with in-process equity financings as deferred offering costs until the equity financing was consummated. After consummation of an equity financing, these costs are recorded in shareholders' equity (deficit) as a reduction of proceeds generated as a result of the offering. As of December 31, 2017, the Company recorded deferred offering costs relating to its IPO of $461. The Company's IPO was completed in March 2018, and these costs, as well as additional IPO costs including commissions of $4,200 and an additional $1,237 of other expenses incurred in 2018, were recorded as a reduction to shareholders' equity.

Property and Equipment

Equipment consists of computers and related equipment and furniture that are stated at cost and depreciated using the straight-line method over estimated useful life of 5 years. Leasehold improvements are amortized over the shorter of the life of the lease or asset.

The Company follows the guidance provided by FASB ASC Topic 360-10, *Property, Plant, and Equipment*. Long-lived assets are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to undiscounted future net cash flows expected to be generated. Impairment charges are recognized at the amount by which the carrying amount of an asset exceeds the fair value of the asset. Assets to be disposed of are reported at the lower of the carrying amount or the fair value less costs to sell.

Since its inception, the Company has not recognized any impairment or disposition of long-lived assets.

Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with ASC 718, "*Compensation—Stock Compensation,*" which requires the measurement and recognition of compensation expense based on estimated fair market values for all share-based awards made to employees and directors, including stock options. The Company's stock-based compensation plan was adopted and became effective in August 2017.

Both BioXcel and the Company's stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of stock option awards was determined using the Black-Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of these awards, as they were not publicly traded. Stock awards granted by the Company subsequent to the IPO are valued using market prices at the date of grant.

The Company adopted FASB ASU 2018-07 as of January 1, 2019 which allowed non-employee options to be expensed using the adoption date fair value.

ASC 718 requires companies to estimate the fair value of share-based awards on the date of grant using an option-pricing model. The Black-Scholes option-pricing model was used as its method of determining fair value. This model is affected by the Company's stock price as well as assumptions regarding a number of subjective variables. These subjective variables include, but are not limited to, the expected stock price volatility over the term of the awards, and actual and projected employee stock option exercise behaviors. The value of the award is recognized as an expense in the statement of operations over the requisite service period. The periodic expense is then determined based on the valuation of the options.

The Company adopted FASB ASU 2016-09 as of January 1, 2018 and has elected to account for forfeitures as they occur, by reversing compensation cost when the award is forfeited.

Research and Development Costs

Research and development expenses include wages, benefits, facilities, supplies, external services, clinical study and manufacturing costs and other expenses that are directly related to the Company's research and development activities. At the end of the reporting period, the Company compares payments made to third party service providers to the estimated progress toward completion of the research or development objectives. Such estimates are subject to change as additional information becomes available. Depending on the timing of payments to the service providers and the progress that the Company estimates has been made as a result of the service provided, the Company may record net prepaid or accrued expense relating to these costs. The Company expenses research and development costs as incurred.

Patent Costs

Costs related to filing and pursuing patent applications are recorded as general and administrative expense and expensed as incurred since recoverability of such expenditures is uncertain.

Fair Value Measurements

ASC 820 "*Fair Value Measurements*" defines fair value, establishes a framework for measuring fair value in GAAP and expands disclosures about fair value measurements. ASC 820 defines fair value as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. ASC 820 establishes a fair value hierarchy that distinguishes between (1) market participant assumptions developed based on market data obtained from independent sources, or observable inputs, and (2) an entity's own assumptions about market participant assumptions developed based on the best information available in the circumstances, or unobservable inputs. The fair value hierarchy consists of three broad levels, which gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1) and the lowest priority to unobservable inputs (Level 3). The three levels of the fair value hierarchy under ASC 820 are described below:

Level 1—Quoted prices (unadjusted) in active markets that are accessible at the measurement date for assets or liabilities. The fair value hierarchy gives the highest priority to Level 1 inputs.

Level 2—Directly or indirectly observable inputs as of the reporting date through correlation with market data, including quoted prices for similar assets and liabilities in active markets and quoted prices in markets that are not active. Level 2 also includes assets and liabilities that are valued using models or other pricing methodologies that do not require significant judgment since the input assumptions used in the models, such as interest rates and volatility factors, are corroborated by readily observable data from actively quoted markets for substantially the full term of the financial instrument.

Level 3—Unobservable inputs that are supported by little or no market activity and reflect the use of significant management judgment. These values are generally determined using pricing models for which the assumptions utilize management's estimates of market participant assumptions.

In determining fair value, the Company utilizes valuation techniques that maximize the use of observable inputs and minimize the use of unobservable inputs to the extent possible, as well as considering counterparty credit risk in its assessment of fair value.

The carrying amounts of cash and accounts payable approximate fair value due to the short-term nature of these instruments.

Net Loss per Share

The Company computes basic net loss per share by dividing net loss per share available to common stockholders by the weighted average number of common shares outstanding for the period and excludes the effects of any potentially dilutive securities. Diluted earnings per share, if presented, would include the dilution that would occur upon the exercise or conversion of all potentially dilutive securities into common stock using the "treasury stock" and/or "if converted" methods as applicable. The Company did not have any potentially dilutive securities outstanding in any period presented in the accompanying financial statements. There were 3,009,386 and 2,588,729 shares of options that were excluded from the calculation of the loss per share for the years ended December 31, 2019 and 2018, respectively. Inclusion of potential common shares would be anti-dilutive for all periods presented and have been excluded from the calculations.

Recent Accounting Pronouncements

In February 2016, the FASB issued *ASU 2016-02 Lease Accounting Topic 842*. This ASU requires the Company to record all leases longer than one year on its balance sheet. Under the new guidance, when the Company records leases on its balance sheet, it will record a liability with a value equal to the present value of payments it will make over the life of the lease and an asset representing the underlying leased asset. The new accounting guidance requires the Company to determine if its leases are operating or financing leases, similar to current accounting guidance. The Company will record expense for operating type leases on a straight-line basis as an operating expense and it will record expense for finance type leases as interest expense. The new lease standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted. The Company adopted the new standard in January 2019 and recorded a Right of Use asset and related liability in the amount of $1,308 on commencement of a new office lease.

In December 2019, the FASB issued *ASU No. 2019-12, Income Taxes (Topic 740)* which amends the existing guidance relating to the accounting for income taxes. This ASU is intended to simplify the accounting for income taxes by removing certain exceptions to the general principles of accounting for income taxes and to improve the consistent application of GAAP for other areas of accounting for income taxes by clarifying and amending existing guidance. The ASU is effective for fiscal years beginning after December 15, 2020. The Company does not expect that the adoption of this new guidance will have a material impact on the Company's Financial Statements.

Note 5. Transactions with BioXcel

The Company has entered into the Amended and Restated Asset Contribution Agreement, pursuant to which BioXcel agreed to contribute BioXcel's rights, title and interest in BXCL501, BXCL701, BXCL502 and BXCL702, and all of the assets and liabilities associated in consideration for (i) 9,480,000 shares of our common stock, (ii) $1,000 upon completion of an initial public offering, (iii) $500 upon the later of the 12 month anniversary of an initial public offering and the first dosing of a patient in the bridging bioavailability/ bioequivalence study for the BXCL501 program, (iv) $500 upon the later of the 12 month anniversary of an initial public offering and the first dosing of a patient in the Phase 2 proof of concept open label monotherapy or combination trial with Keytruda for the BXCL701 program and (v) a one-time payment of $5,000 within 60 days after the achievement of $50,000 in cumulative net sales of any product or combination of products resulting from the development and commercialization of any one of the Candidates or a product derived therefrom. With the completion of the Company's IPO in March 2018, $1,000 was charged to Research and Development costs in connection with (ii) above and was paid on April 5, 2018. The Company paid $500 to BioXcel in connection with (iii) above in April 2019. In July 2019, the Company completed the first dosing of a patient in the combination trial of BXCL701 with Keytruda, and as a result the Company paid $500 to BioXcel in connection with (iv) above in July 2019.

The Company entered into a Separation and Shared Services Agreement with BioXcel that took effect on June 30, 2017, as amended and restated on November 7, 2017 and March 6, 2020, or the Services Agreement, pursuant to which BioXcel will allow us to continue to use the office space, equipment, services and leased employees based on the agreed upon terms and conditions for a payment of defined monthly and/or hourly fees. The office space and equipment portion of the Services Agreement ended effectively on April 30, 2018 when the Company moved to new office space to accommodate additional personnel that had been hired. Services provided by BioXcel through its subsidiaries in India and the United States will continue indefinitely, as agreed upon by the parties. These services are primarily for drug discovery and for chemical, manufacturing and controls cost. Service charges recorded under this agreement were $862 and $579 for the years ended December 31, 2019 and 2018, respectively.

Under the Services Agreement, the Company has an option, exercisable until December 31, 2020, to enter into a collaborative services agreement with BioXcel pursuant to which BioXcel shall perform product identification and related services for us utilizing EvolverAI. The parties are obligated to negotiate the collaborative services agreement in good faith and to incorporate reasonable market-based terms, including consideration for BioXcel reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestones shall not exceed $10 million in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestones shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30 million in the aggregate. BioXcel shall continue to make such product identification and related services available to us for at least five years from June 30, 2017. The parties are currently discussing extending the product identification and related services that BioXcel would provide under the collaborative services agreement, however, as of the date hereof, we have not reached a definitive agreement.

In connection with the Services Agreement, BioXcel agreed to provide the Company a line of credit, which was capped at $1,000, or the Total Funding Amount, pursuant to the terms of a grid note, the ("Grid Note"). The Grid Note was payable upon the earlier of (i) the completion of an initial public offering and (ii) December 31, 2018, together with interest on the unpaid balance of each advance made under the Grid Note, which would accrue at a rate per annum equal to the applicable federal rate for short-term loans as of the date thereof, in each case calculated based on a 365 day year and actual days elapsed.

All amounts due to BioXcel under the line of credit, the Grid Note, and for expenses paid on the Company's behalf were paid following the completion of the Company's IPO on March 20, 2018.

Note 6. Property and Equipment, net

	December 31, 2019	December 31, 2018
Computers and related equipment	$ 229	$ 169
Furniture	344	4
Leasehold improvements	642	172
	1,215	345
Accumulated depreciation and amortization	(174)	(18)
	$ 1,041	$ 327

Depreciation expense was $156 and $17 for the years ended December 31, 2019 and 2018, respectively.

Note 7. Commitments and Contingencies

Master Service Agreements

The Company has entered into a Master Services Agreement ("MSA") with a Contract Research Organization, or CRO, dated November 1, 2018 for strategic planning, expert consultation, clinical trial services, statistical programming and analysis, data processing, data management, regulatory, clerical, project management, medical device services, and other research and development services as set forth in specific work orders. This agreement is for a period of five (5) years.

Excluding the CRO's property, all improvements, inventions, processes, techniques, work product, know-how, data and information generated, conceived, reduced to practice or derived under the MSA by the CRO or its personnel and subcontractors, shall be and remain the exclusive property of the Company, and any inventions that may evolve from the foregoing shall belong to the Company.

The Company entered into a series of cancellable work orders to support its clinical trial activities, related to the first of the Company's BXCL701 clinical trials. This clinical trial is expected to cost approximately $10,000 and is anticipated to take place over the next two years. To date, the Company has incurred $1,809 in costs for the work surrounding this trial.

In the first quarter of 2019 the Company entered into a second series of cancellable work orders to support a second clinical trial related the Company's BXCL 701 product candidate. This clinical trial is expected to aggregate approximately $8,000 and it is anticipated to take place over the next three years. Approximately one half of this cost is to be reimbursed by a partner. The Company has incurred $1,353 of costs in connection with this trial and has also recorded a related receivable of $82.

In addition, an MSA was signed with a second CRO during the first quarter of 2019 to include strategic planning, expert consultation, regulatory activities, data interpretation, New Drug Application services, and research and development services, including clinical, data management, statistical and medical writing activities.

Note 8. Accrued Expenses

Accrued expenses consist of the following:

	December 31, 2019	December 31, 2018
Drugs and clinical trial expenses	$ 1,215	$ 1,887
Accrued salaries, benefits and travel related costs	1,570	774
Professional and consultant fees	126	181
Legal expenses	140	105
Other administrative accruals	69	109
	$ 3,120	$ 3,056

Note 9. Stockholders' Equity (Deficit)

Authorized Capital

The Company is authorized to issue up to 10,000,000 preferred shares with a par value of $0.001 per share. No preferred shares were issued and outstanding as of December 31, 2019 and 2018.

The Company is authorized to issue up to 50,000,000 shares of common stock with a par value of $0.001 per share. The Company had 18,087,382 and 15,663,221 shares of common stock outstanding as of December 31, 2019 and December 31, 2018, respectively.

Description of Common Stock

Each share of common stock has the right to one vote. The holders of common stock are entitled to dividends when funds are legally available and when declared by the board of directors.

Common Stock Issuances

On March 7, 2018, the Company's registration statement on Form S-1 relating to the Company's IPO was declared effective by the SEC. The IPO closed on March 12, 2018, and the Company issued and sold 5,454,545 shares of common stock at a public offering price of $11.00 per share, for gross proceeds of $60,000 and net proceeds of $54,102 after deducting underwriting discounts and commissions of $4,200 and other offering expenses of $1,698.

In January and February 2018, the Company issued 283,452 shares of common stock with an issuance price of $6.88 per share for gross and net proceeds of $1,950.

On May 20, 2019, the Company entered into an Open Market Sale Agreement (the "Sale Agreement") with Jefferies LLC ("Jefferies") pursuant to which the Company may offer and sell shares of its common stock, par value $0.001 per share (the "Common Stock"), having an initial offering price no greater than $20.0 million (the "Shares"), from time to time, through an "at the market offering" program under which Jefferies will act as sales agent. The Company sold 66,193 shares under the Sale Agreement for gross proceeds of $737, issuance costs of $350 or net proceeds of $387. The Sale Agreement was terminated by the Company on September 22, 2019

On September 26, 2019, the Company entered into an underwriting agreement with several underwriters in connection with the issuance and sale by the Company in a public offering of 2,303,030 shares of the Company's common stock at a public offering price of $8.25 per share, less underwriting discounts and commissions, pursuant to an effective shelf registration statement on Form S-3 (Registration No. 333-230674) and a related prospectus supplement filed with the SEC (the "September 2019 Offering"). The September 2019 Offering closed on September 30, 2019.

The Company received gross and net proceeds of approximately $19,000 and $17,423 respectively from the September 2019 Offering. The Company intends to use the net proceeds for general corporate purposes, which may include development and commercialization of their product candidates, research and development, general and administrative expenses, license or technology acquisitions, and working capital and capital expenditures.

See Note 13 - Subsequent Event for a description of the February 2020 offering.

Note 10. Stock-Based Compensation

Stock Options

The Company's 2017 Stock Incentive Plan, or the 2017 Stock Plan, became effective in August 2017 and will expire in August 2027. Under the 2017 Stock Plan, the Company may grant incentive stock options, non-statutory stock options, restricted stock awards and other stock-based awards.

As of December 31, 2019, there were 3,389,956 shares of the Company's common stock authorized for issuance under the 2017 Stock Plan. Options granted under the 2017 Stock Plan have a term of ten years with the vesting term determined by the board of directors, which is generally four years.

The fair value of options granted during the year ended December 31, 2019 was estimated using the Black-Scholes option-pricing model with the following assumptions. Stock-based awards to non-employees are re-measured at fair value each financial reporting date until performance is complete.

The weighted average fair value of options granted in 2019 and 2018 was $9.68 and $7.64 per option, respectively and were determined using the following assumptions:

	For the Year Ended December 31, 2019
Exercise price per share	$ 4.14 - $11.28
Expected stock price volatility	78.05 % - 79.48 %
Risk-free rate of interest	1.51 % - 2.53 %
Fair value of grants per share	$ 2.73 - $ 8.17
Expected Term (years)	5.0 - 7.0

Since the Company completed its IPO within the last year, it does not have a history of market prices of its common stock and, as such, volatility was estimated using historical volatilities of similar public companies. The expected term of the employee awards is estimated based on the simplified method, which calculates the expected term based upon the midpoint of the term of the award and the vesting period. The Company uses the simplified method because it does not have sufficient option exercise data to provide a reasonable basis upon which to estimate the expected term. The expected term of non-employee awards represents the awards contractual term. The expected dividend yield is 0% as the Company has no history of paying dividends nor does management expect to pay dividends over the contractual terms of these options. The risk-free interest rates are based on the United States Treasury yield curve in effect at the time of grant, with maturities approximating the expected term of the stock options.

The following table summarizes information about stock option activity during the period the Plan was in effect (in thousands, except share and per share data):

	Number of Shares	Weighted Average Exercise Price per Share	Total Intrinsic Value	Weighted Average Remaining Contractual Life (in years)
Outstanding as of January 1, 2019	2,744,153	$ 2.33	$ 7,411	8.8
Options granted	384,800	$ 9.68	$ —	9.4
Options forfeited	(15,666)	$ 8.53	$ —	—
Options exercised	(54,938)	$ 0.41	$ —	—
Outstanding as of December 31, 2019	3,058,349	$ 3.26	$ 34,725	8.0
Options vested and exercisable as of December 31, 2019	2,095,252	$ 1.67	$ 27,104	7.8

There were 331,607 shares available for grant as of December 31, 2019.

The Company recognized stock-based compensation expense under the 2017 Stock Plan of $3,070 and $2,872 for the years ended December 31, 2019 and 2018, respectively.

Unrecognized compensation expense related to unvested awards as of December 31, 2019 was $2,772 and will be recognized over the remaining vesting periods of the underlying awards. The weighted-average period over which such compensation is expected to be recognized is 1.3 years.

BioXcel Charges

BioXcel has granted stock options to its employees under its own Equity Incentive Plan ("BioXcel Plan"). Stock-based compensation expense from the BioXcel Plan is allocated to the Company over the period over which those stock option awards vest and are based the on the percentage of time spent on Company activities compared to BioXcel activities. The BioXcel stock option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of these BioXcel stock option awards was determined using

the Black Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of these awards, as they are not publicly traded.

Stock based compensation expense, net of forfeitures, recognized by the Company in its statements of operations related to BioXcel equity awards totaled approximately $72 and $210 for the years ended December 31, 2019 and 2018, respectively.

Total stock based compensation charges were approximately $3,142 and $3,082 for the years ended December 31, 2019 and 2018, respectively. The Company charged $1,791 and 1,351 to research and development and general and administrative expense for the year ended December 31, 2019, respectively. The Company charged $1,843 and 1,239 to research and development and general and administrative expense for the year ended December 31, 2018, respectively.

Note 11. Income Taxes

There is no provision for income taxes because the Company has historically incurred operating losses and maintains a full valuation allowance against its net deferred tax assets. The reported amount of income tax expense for the years differs from the amount that would result from applying domestic federal statutory tax rates to pretax losses primarily because of changes in valuation allowance.

Pursuant to incorporation of the Company as a C corporation on March 29, 2017, BioXcel became the sole owner of the Company, and contributed certain assets to the Company in a tax free transaction. From the date of incorporation, the Company is a standalone C corporation subject to corporate income tax and the deferred taxes of the Company have been calculated accordingly.

The significant components of the Company's net deferred tax assets at December 31, 2019 and 2018 are shown below. In determining the realizability of the Company's net deferred tax asset, the Company considered numerous factors, including historical profitability, estimated future taxable income, and the industry in which it operates. Based on this information the Company has provided a valuation allowance for the full amount of its net deferred tax asset because the Company has determined that it is more likely than not that it will not be realized.

	2019	2018
Federal net operating losses	$ 3,445	$ 3,840
State net operating losses	972	1,300
Stock based compensation	860	552
Federal and state tax credits	1,254	366
Capitalized research and development costs	8,288	—
Accrued expense	432	203
Total gross deferred tax assets	15,251	6,261
Less: valuation allowance	(15,251)	(6,261)
Net deferred tax assets	$ —	$ —

A reconciliation between the Company's effective tax rate and the federal statutory rate for the years ended December 31, 2019 and 2018 are as follows:

	2019	2018
Federal Statutory Rate	21.0 %	21.0 %
Stock based compensation	(1.4)	(2.2)
Federal and state credits	2.7	1.6
State Taxes	5.0	6.3
Change in valuation allowance	(27.3)	(26.5)
Other	-	(0.2)
Effective Tax Rate	0.0 %	0.0 %

At December 31, 2019, the Company had approximately $16,406 of gross federal and state net operating loss carry-forwards. If not utilized, the federal and state net operating loss carry-forwards will begin to expire in 2037. The federal net operating loss incurred after December 31, 2017 will be carried forward indefinitely. The utilization of such net operating loss carry-forwards and realization of tax benefits in future years depends predominantly upon having taxable income. The Company also has approximately $1,254 of federal research and development credits which will begin to expire in 2037 if not utilized.

Utilization of the net operating loss, or NOL, and research tax credit carryforwards may be subject to a substantial annual limitation due to ownership change limitations that has occurred or that could occur in the future, as required by Section 382 of the Code, as well as similar state and foreign provisions. These ownership changes may limit the amount of NOL and research tax credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an "ownership change" as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points of the outstanding stock of a company by certain stockholders or public groups. To date, the Company's NOLs have not been subject to Section 382 limitation.

Entities are also required to evaluate, measure, recognize and disclose any uncertain income tax provisions taken on their income tax returns. The Company has analyzed its tax positions and has concluded that as of December 31, 2019 there were no uncertain positions. Interest and penalties, if any, as they relate to income taxes assessed, are included in the income tax provision. There was no income tax related interest and penalties included in the income tax provision. The Company's U.S. federal and state net operating losses have occurred since its inception in 2017 and as such, tax years subject to potential tax examination could apply from that date because the utilization of net operating losses from prior years opens the relevant year to audit by the IRS and/or state taxing authorities.

Note 12. Leases

The Company entered into an agreement to lease approximately 11,040 square feet of space on the 12th floor of the building located at 555 Long Wharf Drive, New Haven, Connecticut that commenced February 22, 2019, or the Commencement Date. The premises were occupied in March 2019.

The term of the 12th floor lease continues from the Commencement Date through the last day of the calendar month immediately following the seventh anniversary of the Commencement Date.

The Company's improvement costs were approximately $642 and are being amortized over the life of the lease.

Maturities of the operating lease liability are as follows:

Year ending December 31,	Amount
2020	$ 208
2021	196
2022	219
2023	225
2024	230
Thereafter	276
Total lease payments	1,354
Less imputed interest	(161)
Total lease liability	1,193
Less current portion	(164)
Operating lease liability	$ 1,029

The current portion of the Company's operating lease liability of $164 as of December 31, 2019 is included in other current liabilities on the balance sheet.

The Company recorded lease expense of $155 related to its operating lease right-of-use asset for the year ended December 31, 2019.

The Company has an option to renew the lease for one additional five-year term at 95% of the then-prevailing market rates but not less than the rental rate at the end of the initial lease term.

Note 13. Subsequent Event

On February 19, 2020, the Company entered into an underwriting agreement with several underwriters pursuant to its shelf registration statement on Form S-3 in connection with the issuance and sale by the Company in a public offering of 2,000,000 shares of the Company's common stock at a public offering price of $32.00 per share. In addition, BTI has granted the underwriters a 30-day option to purchase up to an additional 300,000 shares of common stock from BTI at the public offering price, less underwriting discounts and commissions. Gross proceeds to BTI from the offering were approximately $64,000, before deducting underwriting discounts and commissions and offering expenses. To the extent the underwriters exercise their option to purchase additional shares of common stock, the Company intends to use the net proceeds from the sale of additional shares to repurchase shares of common stock (which shares will then be canceled) from BioXcel Corporation at a price equal to the price paid by the underwriters for such shares in the public offering, less underwriting discounts and commissions.

On February 21, 2020 the underwriters exercised their option to purchase an additional 300,000 shares of BTI stock. Subsequent to the underwriters exercise BTI purchased 300,000 shares from BioXcel Corporation at a price equal to the price paid by the underwriters for such shares in the public offering less underwriting discounts and commissions at a per share price of $30.08.

The offering closed on February 24, 2020.

Exhibit 31.1

CERTIFICATIONS

I, Vimal Mehta, Ph.D., certify that:

1. I have reviewed this Annual Report on Form 10-K for the fiscal year ended December 31, 2019 of BioXcel Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 9, 2020

By: /s/ Vimal Mehta
Vimal Mehta, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Richard Steinhart, certify that:

1. I have reviewed this Annual Report on Form 10-K for the fiscal year ended December 31, 2019 of BioXcel Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 9, 2020

By: /s/ Richard Steinhart
Richard Steinhart
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of BioXcel Therapeutics, Inc. (the "Company") for the fiscal year ended December 31, 2019, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 9, 2020

By: /s/ Vimal Mehta
Vimal Mehta, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report on Form 10-K of BioXcel Therapeutics, Inc. (the "Company") for the fiscal year ended December 31, 2019, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 9, 2020

By: /s/ Richard Steinhart
Richard Steinhart
Chief Financial Officer
(Principal Financial Officer)

bioxcel therapeutics

EXECUTIVE OFFICERS

Vimal D. Mehta, Ph.D.
President and Chief Executive Officer

Richard I. Steinhart
Senior Vice President and Chief Financial Officer

Frank Yocca, Ph.D.
Senior Vice President and Chief Scientific Officer

Vincent O'Neill, M.D.
Senior Vice President and Chief Medical Officer

BOARD OF DIRECTORS

Peter Mueller, Ph.D.
President of the Mueller Health Foundation

Vimal D. Mehta, Ph.D.
President and Chief Executive Officer of BioXcel Therapeutics, Inc.

Krishnan Nandabalan, Ph.D.
President, Secretary and Chief Scientific Officer of BioXcel Corporation

Sandeep Laumas, M.D.
Executive Chairman and Chief Executive Officer of Innovate Biopharmaceuticals

Michal Votruba, M.D.
Director of Gradus/RSJ Life Sciences Fund

AVAILABLE INFORMATION

We make available free of charge under the Investor Relations section of our website, www.bioxceltherapeutics.com , filings we make with the Securities and Exchange Commission and other information about the Company. Filings we make with the Securities and Exchange Commission may also be accessed free of charge on the Securities and Exchange Commission's publicly available website, www.sec.gov.

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