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

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended March 31, 2022

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 **New Haven, CT**	**06511**
(Address of principal executive offices)	(Zip Code)

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

N/A
(Former name, former address and former fiscal year, if changed since last report)

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

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

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 Exchange Act). Yes ☐ No ☒

The number of shares of the registrant's common stock, $0.001 par value per share, outstanding at May 5, 2022 was 27,985,145.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q 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 commercialization plans for IGALMI™;
- our plans relating to clinical trials for our 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 IGALMI™ and any product candidates 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 LLC.

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 "Summary Risk Factors," Part II, Item 1A. "Risk Factors," and Part I, Item 2. "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this Quarterly Report on Form 10-Q. 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 Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q, unless otherwise specified or the context otherwise requires, the terms "we," "our," "us," the "Company" or "BTI" refer to BioXcel Therapeutics, Inc. and "BioXcel LLC" refers to the Company's former parent company and significant stockholder, BioXcel LLC and its predecessor, BioXcel Corporation. All brand names or trademarks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners, including IGALMI™, which is a trademark of BioXcel Therapeutics, Inc.

We may use our website as a distribution channel of material information about the Company. Financial and other important information regarding the Company is routinely posted on and accessible through the Investors & Media section of its website at *www.bioxceltherapeutics.com*. In addition, you may automatically receive email alerts and other information about the Company when you enroll your email address by visiting the "Email Alerts" option under the News / Events menu of the Investors & Media section of our website at *www.bioxceltherapeutics.com*.

SUMMARY RISK FACTORS

Our business is subject to numerous risks and uncertainties, including those described in Part II, Item 1A. "Risk Factors" in this Quarterly Report on Form 10-Q. You should carefully consider these risks and uncertainties when investing in our common stock. The principal risks and uncertainties affecting our business include the following:

- 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 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.
- 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 have limited experience in drug discovery and drug development, and we only recently had our first drug approved, which has not yet been commercialized. We may never have another drug approved.
- In the near term, we are dependent on the success of IGALMI™, BXCL501 and BXCL701. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize IGALMI™ or our product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, 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.
- The regulatory approval processes of the United States of America ("U.S.") Food and Drug Administration ("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.
- Clinical trials are expensive, time-consuming and difficult to design and implement, and involve an uncertain outcome.
- Our products or 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.
- BioXcel LLC'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.
- If we are required by the FDA or similar regulatory authorities to obtain approval (or clearance, or certification) of a companion diagnostic device in connection with approval of one of our product candidates, and we do not obtain or face delays in obtaining FDA approval (or clearance, or certification) of a companion diagnostic device, we will not be able to commercialize the product candidate and our ability to generate revenue will be materially impaired.
- Even if we obtain regulatory approval for current or future product candidates, we will still face extensive and ongoing regulatory requirements and obligations and any product candidates, if approved, may face future development and regulatory difficulties.

- If IGALMI™ or our other product candidates, if approved, do not gain market acceptance, our business will suffer because we might not be able to fund future operations.

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

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

- 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.

- 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.

- 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.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

BIOXCEL THERAPEUTICS, INC. AND SUBSIDIARIES

BALANCE SHEETS

(amounts in thousands, except per share amounts)
(unaudited)

	March 31, 2022	December 31, 2021
ASSETS		
Current assets		
Cash and cash equivalents	$ 200,435	$ 232,968
Inventory	682	—
Prepaid expenses	4,967	2,888
Other current assets	1,478	956
Total current assets	$ 207,562	$ 236,812
Property and equipment, net	1,337	1,294
Operating lease right-of-use assets	1,181	1,247
Other assets	965	86
Total assets	$ 211,045	$ 239,439
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 5,065	$ 4,678
Accrued expenses	10,577	11,492
Due to related party	56	204
Other current liabilities	299	293
Total current liabilities	$ 15,997	$ 16,667
Long-term portion of operating lease liabilities	1,028	1,105
Total liabilities	$ 17,025	$ 17,772
Commitments and contingencies (Note 11)		
Stockholders' equity		
Common stock, $0.001 par value, 100,000 shares authorized as of March 31, 2022 and December 31, 2021; 27,980 shares issued and outstanding as of March 31, 2022 and December 31, 2021	$ 28	$ 28
Preferred stock, $0.001 par value, 10,000 shares authorized; no shares issued and outstanding as of March 31, 2022 and December 31, 2021	—	—
Additional paid-in-capital	471,252	467,427
Accumulated deficit	(277,260)	(245,788)
Total stockholders' equity	$ 194,020	$ 221,667
Total liabilities and stockholders' equity	$ 211,045	$ 239,439

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

BIOXCEL THERAPEUTICS, INC. AND SUBSIDIARIES

STATEMENTS OF OPERATIONS

(amounts in thousands, except per share amounts)
(unaudited)

	Three months ended March 31,	
	2022	2021
Operating expenses		
Research and development	$ 18,559	$ 14,741
General and administrative	12,921	11,638
Total operating expenses	$ 31,480	$ 26,379
Loss from operations	$ (31,480)	$ (26,379)
Other income (expense)		
Interest income	15	10
Interest expense	(7)	(7)
Net loss and comprehensive loss	$ (31,472)	$ (26,376)
Basic and diluted net loss per share attributable to common stockholders	$ (1.12)	$ (1.08)
Weighted average shares outstanding - basic and diluted	27,980	24,524

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

BIOXCEL THERAPEUTICS, INC. AND SUBSIDIARIES

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

(amounts in thousands)
(unaudited)

	Common stock		Additional paid-in-capital	Accumulated deficit	Total
	Shares	Amount			
Balance as of December 31, 2020	24,417	$ 24	$ 345,529	$ (138,857)	$ 206,696
Stock-based compensation	—	—	5,565	—	5,565
Exercise of stock options	214	1	851	—	852
Net loss	—	—	—	(26,376)	(26,376)
Balance as of March 31, 2021	24,631	$ 25	$ 351,945	$ (165,233)	$ 186,737

	Common stock		Additional paid-in-capital	Accumulated deficit	Total
	Shares	Amount			
Balance as of December 31, 2021	27,980	$ 28	$ 467,427	$ (245,788)	$ 221,667
Stock-based compensation	—	—	3,825	—	3,825
Net loss	—	—	—	(31,472)	(31,472)
Balance as of March 31, 2022	27,980	$ 28	$ 471,252	$ (277,260)	$ 194,020

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

BIOXCEL THERAPEUTICS, INC. AND SUBSIDIARIES

STATEMENTS OF CASH FLOWS

(amounts in thousands)
(unaudited)

	Three months ended March 31,	
	2022	2021
OPERATING CASH FLOW ACTIVITIES:		
Net loss	$ (31,472)	$ (26,376)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	77	65
Stock-based compensation expense	3,825	5,565
Changes in operating assets and liabilities:		
Inventory	(682)	—
Prepaid expenses and other assets	(3,480)	703
Operating lease right-of-use assets	66	—
Accounts payable, accrued expenses, and other liabilities	(676)	403
Operating lease liabilities	(71)	—
Net cash used in operating activities	$ (32,413)	$ (19,640)
INVESTING CASH FLOW ACTIVITIES:		
Purchases of equipment and leasehold improvements	$ (120)	$ (316)
Net cash used in investing activities	$ (120)	$ (316)
FINANCING CASH FLOW ACTIVITIES:		
Exercise of stock options	$ —	$ 852
Net cash provided by financing activities	$ —	$ 852
Net (decrease) increase in cash and cash equivalents	$ (32,533)	$ (19,104)
Cash and cash equivalents, beginning of the period	232,968	213,119
Cash and cash equivalents, end of the period	$ 200,435	$ 194,015
Supplemental cash flow information:		
Purchases of property and equipment in accounts payable and accrued expenses	$ —	$ 9

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

BIOXCEL THERAPEUTICS, INC. AND SUBSIDIARIES

NOTES TO FINANCIAL STATEMENTS

(amounts in thousands, except per share amounts and where otherwise noted)
(unaudited)

Note 1. Nature of the Business

BioXcel Therapeutics, Inc. is a biopharmaceutical company focused on drug development that utilizes artificial intelligence ("AI") 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 proprietary, orally dissolving, thin film formulation of the adrenergic receptor agonist dexmedetomidine ("Dex"), for the treatment of agitation, and BXCL701, an orally administered, systemic innate immune activator for the treatment of a rare form of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors.

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 LLC" refers to BioXcel LLC and, its predecessor, BioXcel Corporation.

The Company was incorporated under the laws of the State of Delaware on March 29, 2017. The Company's principal office is in New Haven, Connecticut.

Impact of COVID-19 Pandemic

During the first quarter ended March 31, 2020, the novel coronavirus disease ("COVID-19") was declared a pandemic and spread to multiple regions across the globe, including the U.S. and Europe. The outbreak and government measures taken in response had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production were suspended; and demand for certain goods and services, such as medical services and supplies, spiked, while demand for other goods and services, such as travel, has fallen.

We continue to work closely with our clinical sites to monitor the potential impact of the evolving COVID-19 pandemic and the spread of its variants. We remain committed to our clinical programs and development plans. To-date, we have not experienced any significant delays in any of our ongoing or planned clinical trials. However, this could rapidly change.

Note 2. Basis of Presentation

The accompanying unaudited financial statements do not include all of the information and notes required by Generally Accepted Accounting Principles in the U.S. ("GAAP"). The accompanying year-end balance sheet was derived from audited financial statements but does not include all disclosures required by GAAP. The unaudited interim financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of March 31, 2022, the results of its operations for the three months ended March 31, 2022 and 2021 and its cash flows for the three months ended March 31, 2022 and 2021, respectively. The results for the three months ended March 31, 2022 are not necessarily indicative of results to be expected for the year ending December 31, 2022, any other interim periods or any future year or period. The accompanying unaudited financial statements of the Company should be read in conjunction with the audited financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2021 filed with the Securities and Exchange Commission on March 10, 2022.

As of March 31, 2022, the Company had cash and cash equivalents of $200,435 and an accumulated deficit of $277,260. BTI has incurred substantial net losses and negative cash flows from operating activities in nearly every fiscal period since inception and expects this trend to continue for the foreseeable future. The Company recognized net losses of $31,472 and $26,376 for the three months ended March 31, 2022 and 2021, respectively, and had net cash used in operating activities of $32,413 and $19,640 for the three months ended March 31, 2022 and 2021, respectively. The Company believes that its existing cash and cash equivalents will be sufficient to cover its cash flow requirements for at least the next twelve months from the issuance date of these financial statements. However, the Company's future requirements may change and will depend on numerous factors.

Note 3. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in accordance with GAAP requires management to make estimates and assumptions that affect amounts reported in the financial statements and notes thereto. 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 March 31, 2022 and December 31, 2021, cash equivalents were comprised primarily 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.

Inventory

Inventories are stated at the lower of cost or net realizable value.

BTI capitalizes inventory costs associated with the Company's products prior to regulatory approval, when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized; otherwise, such costs are expensed.

As of March 31, 2022, inventory balances include inventory costs prior to regulatory approval of BXCL501. Inventory consists of raw materials.

The Company periodically analyzes its inventory levels to identify inventory that may expire prior to expected sale or has a cost basis in excess of its estimated realizable value and writes down such inventories as appropriate.

Property and Equipment

Property and equipment are recorded at cost and depreciated and amortized over the shorter of their remaining lease term or their estimated useful life on a straight-line basis as follows:

Equipment	3-5 years
Furniture	7 years
Leasehold improvements	Lesser of life of improvement or lease term

Expenditures for maintenance and repairs which do not improve or extend the useful lives of the respective assets are expensed as incurred. When assets are sold or retired, the related cost and accumulated depreciation are removed from their respective accounts and any resulting gain or loss is included within general and administrative expenses in the statements of operations.

The Company follows the guidance provided by the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 360-10, *Property, Plant, and Equipment-Overall*. 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.

Leases

We determine if an arrangement is a lease at inception. Operating leases are included in operating lease right-of-use ("ROU") assets, other current liabilities, and the long-term portion of operating lease liabilities in our balance sheet.

ROU assets represent our right to use an underlying asset for the lease term and lease liabilities represent our obligation to make lease payments arising from the lease. Operating lease ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term. As our leases do not provide an implicit rate, we used an incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments. We use the implicit rate when readily determinable. The operating lease ROU asset also includes any prepaid lease payments made and excludes lease incentives. Our leases may include options to extend or terminate the lease; such options are included in determining the lease term when it is reasonably certain that we will exercise that option. Renewal options were not included in our calculation of the related asset and liability since it is not reasonably certain we will exercise the relevant option. Lease expense for lease payments is recognized on a straight-line basis over the lease term.

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 value for all share-based awards made to employees and directors, including stock options and restricted stock units ("RSUs"). The Company's 2017 Equity Incentive Plan (the "2017 Plan") became effective in August 2017. The Company's 2020 Incentive Award Plan (the "2020 Plan") became effective in May 2020. Following the effective date of the 2020 Plan, the Company ceased granting awards under the 2017 Plan, however the terms and conditions of the 2017 Plan continue to govern any outstanding awards granted thereunder.

The Company's stock-based awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period using the accelerated attribution method. The estimated fair value of stock-based awards was determined using the Black-Scholes pricing model on the date of grant. Prior to the Company's initial public offering ("IPO"), significant judgment and estimates were used to estimate the fair value of these awards. Stock awards granted by the Company subsequent to the IPO are valued using market prices at the date of grant.

The Black-Scholes pricing model is affected by the Company's stock price as well as assumptions regarding a number of subjective variables including, but not limited to, the strike price of the instrument, the Company's current stock price, the risk-free rate, the expected stock price volatility over the term of the awards, and actual and projected employee stock option exercise behaviors. The value of the awards are recognized as an expense in the statement of operations over the requisite service period using the accelerated attribution method. The Company 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, 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. Depending on the timing of payments to the service providers and the progress that the Company estimates has been made for the program as a result of the

level of service provided, the Company may record net prepaid or accrued expense relating to these costs. Such estimates are subject to change as additional information becomes available. The Company expenses research and development costs as incurred.

Expenses Accrued Under Contractual Arrangements

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 ("CROs") 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.

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 of Financial Instruments

The Company applies the provisions of ASC 820, *Fair Value Measurement,* for financial assets and liabilities measured on a recurring basis which requires disclosure that establishes a framework for measuring fair value. 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). ASC 820 requires that fair value measurements be classified and disclosed in one of three categories:

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.

As of March 31, 2022 and December 31, 2021, the Company had $200,435 and $232,968, respectively, primarily in U.S. government money market accounts (included in cash and cash equivalents) which was valued based on Level 1 inputs. There were no transfers between levels within the hierarchy during the three months ended March 31, 2022 and the year ended December 31, 2021.

Earnings (Loss) Per Share

Basic earnings (loss) per share ("EPS") is calculated in accordance with ASC 260, *Earnings Per Share*, by dividing net income or loss attributable to common stockholders by the weighted average number of shares of common stock that were outstanding. Diluted EPS is calculated by adjusting the weighted average number of shares of common stock that were outstanding for the dilutive effect of common stock equivalents. In periods in which a net loss is recorded, no effect is given to potentially dilutive securities, since the effect would be antidilutive. Securities that could potentially dilute basic EPS in the future were not included in the computation of diluted EPS because to do so would have been antidilutive.

Segment Information

The Company operates 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.

Recent Accounting Pronouncements

Recently adopted accounting pronouncements

In December 2019, the FASB issued Accounting Standards Update ("ASU") No. 2019-12*, Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes* ("ASU No. 2019-12"), which amends the existing guidance relating to the accounting for income taxes. ASU No. 2019-12 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. ASU No. 2019-12 was effective for interim and annual periods beginning after December 15, 2020. The Company adopted ASU No. 2019-12 effective January 1, 2021. The adoption of ASU No. 2019-12 did not have a material impact on the Company's financial statements.

Accounting Pronouncements effective in future periods

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments-Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments,* and subsequent amendments to the initial guidance (collectively, "Topic 326"). Topic 326 requires measurement and recognition of expected credit losses for financial assets held. Topic 326 was to be effective for reporting periods beginning after December 15, 2019, with early adoption permitted. In November 2019, the FASB issued ASU No. 2019-10, *Financial Instruments - Credit Losses (Topic 326), Derivatives and Hedging (Topic 815), and Leases (Topic 842) - Effective Dates*, which deferred the effective dates of Topic 326 for

the Company, until fiscal year 2023. The Company does not expect that the adoption of Topic 326 will have a material impact on its financial statements.

Note 4. Financing Activities

In June 2021, the Company sold, in a registered offering, 3,155 shares of its common stock at a public offering price of $31.70 per share. The Company received proceeds of $96,937, net of issuance costs of $3,076.

In May 2021, the Company entered into an Open Market Sale Agreement (the "Sale Agreement") with Jefferies LLC ("Jeffries") pursuant to which the Company could offer and sell shares of its common stock, having an aggregate offering price of up to $100,000, from time to time, through an "at the market offering" program under which Jefferies will act as sale agent. The Company sold 124 shares under the Sale Agreement in June 2021. As of December 31, 2021, the Company received proceeds of $4,056, net of issuance costs of $500.

Note 5. Transactions with BioXcel LLC

The Company entered into a Separation and Shared Services Agreement with BioXcel LLC that took effect on June 30, 2017, as amended and restated, (the "Services Agreement"), pursuant to which services provided by BioXcel LLC, through its subsidiaries in India and the U.S., will continue indefinitely, as agreed upon by the parties. These services are primarily for drug discovery, chemical, manufacturing and controls ("CMC") and administrative support.

Service charges recorded under the Services Agreement for the three months ended March 31, 2022 and 2021 were comprised as follows:

	Three months ended March 31,	
	2022	2021
Research and development	$ 306	$ 320
General and administrative	70	49
Total	$ 376	$ 369

As of March 31, 2022, $56 related to these service charges is included in due to related parties in the Company's balance sheet.

Under a Second Amended and Restated Shared Services Agreement signed in April 2022, the Company has an option, exercisable until December 31, 2024, to enter into a collaborative services agreement with BioXcel LLC pursuant to which BioXcel LLC 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 LLC reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestone payments shall not exceed $10,000 in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestone payments shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30,000 in the aggregate. In conjunction with the Second Amended and Restated Shared Services Agreement, the Company agreed to pay BioXcel LLC $18 per month to extend the option to December 31, 2024 from its current expiration of March 13, 2023.

Note 6. Earnings (Loss) Per Share

Basic EPS is calculated in accordance with ASC 260, *Earnings Per Share*, by dividing net income or loss attributable to common stockholders by the weighted average number of shares of common stock that were outstanding. Diluted EPS is calculated by adjusting the weighted average number of shares of common stock that were outstanding for the dilutive effect of common stock equivalents. In periods in which a net loss is recorded, no effect is given to

potentially dilutive securities, since the effect would be antidilutive. The calculations of basic and diluted net loss per share are as follows:

	Three months ended March 31,	
	2022	2021
Net loss (numerator)	$ (31,472)	$ (26,376)
Weighted average shares (denominator)	27,980	24,524
Basic and diluted net loss per share (in whole dollars)	$ (1.12)	$ (1.08)

Potentially dilutive securities outstanding consists of stock options and RSUs. The Company had common stock equivalents outstanding at March 31, 2022 and 2021 of 4,782 and 4,072 shares, respectively.

Note 7. Property and Equipment, net

A summary of net property and equipment is as follows:

	March 31, 2022	December 31, 2021
Computers and related equipment	$ 167	$ 167
Furniture	680	572
Leasehold improvements	1,156	1,133
Work in process	13	24
Total property and equipment	$ 2,016	$ 1,896
Accumulated depreciation	(679)	(602)
Total property and equipment, net	$ 1,337	$ 1,294

Depreciation expense was $77 and $65 for the three months ended March 31, 2022 and 2021, respectively.

Note 8. Accrued Expenses

Accrued expenses consist of the following:

	March 31, 2022	December 31, 2021
Accrued research and development expenses	$ 5,409	$ 5,762
Accrued compensation and benefits	1,861	3,968
Accrued professional expenses	2,789	1,324
Accrued taxes	366	302
Other accrued expenses	152	136
Total accrued expenses	$ 10,577	$ 11,492

Note 9. Stock-Based Compensation

2017 Equity Incentive Plan

The Company’s 2017 Plan became effective in August 2017. Following the effective date of the Company's 2020 Plan (as defined below), the Company ceased granting awards under the 2017 Plan, however, the terms and conditions of the 2017 Plan continue to govern any outstanding awards granted thereunder.

2020 Incentive Award Plan

The Company's 2020 Plan was approved and became effective at the Company's 2020 annual meeting of stockholders on May 20, 2020, and unless earlier terminated by the Board of Directors, will remain in effect until March 26, 2030. The 2020 Plan originally authorized for issuance the sum of (i) 911 shares of the Company's common stock authorized for issuance and (ii) 233 shares of the Company's common stock, which represents the number of shares that remained available for issuance under the 2017 Plan immediately prior to the approval of the 2020 Plan by the Company's stockholders. Any shares of common stock which, immediately prior to the approval of the 2020 Plan by the Company's stockholders, were subject to awards granted under the 2017 Plan that are forfeited or lapse unexercised and are not issued under the 2017 Plan will increase the number of shares of common stock available for grant under the 2020 Plan. In addition, the number of shares available for issuance under the 2020 Plan will increase on the first day of each calendar year, beginning January 1, 2021 and ending on and including January 1, 2030, by a number of shares equal to the lesser of (A) 4% of the aggregate number of shares of the Company's common stock outstanding on the final day of the immediately preceding calendar year and (B) such smaller number of shares of common stock as determined by the Board of Directors. The shares available for issuance under the 2020 Plan increased by 1,119 shares and 977 shares on January 1, 2022 and 2021, respectively.

Stock-based awards granted under the 2020 Plan have a term of ten years with the vesting schedule determined by the Board of Directors, which is generally four years.

As of March 31, 2022, there were 986 shares available to be granted under the 2020 Plan.

Restricted stock units

During the three months ended March 31, 2022, the Company granted 97 time-based RSUs to certain employees and consultants. These RSUs generally vest over four years, with 25% vesting at the one-year anniversary of the grant date and the balance vesting ratably over the remaining three years of the vesting period. None of the time-based RSUs had vested as of March 31, 2022. Unrecognized stock-based compensation expense related to these awards was $1,494 at March 31, 2022. No RSUs were issued and outstanding as of March 31, 2021.

Stock options

A summary of the status of the Company's stock option activity for the three months ended March 31, 2022 is presented below.

	Number of shares		Weighted average exercise price per share
Outstanding as of January 1, 2022	4,000	$	18.89
Granted	840	$	15.71
Forfeited	(103)	$	32.34
Cancelled	(53)	$	53.76
Outstanding as of March 31, 2022	4,684	$	17.63
Options vested and exercisable as of March 31, 2022	2,836	$	10.72

As of March 31, 2022, the intrinsic value of options outstanding was $46,681. The intrinsic value for stock options is calculated based on the difference between the exercise prices of the underlying awards and the quoted stock price of the Company's common stock as of the reporting date.

No stock options were exercised for the three months ended March 31, 2022. The total intrinsic value of stock options exercised for the three months ended March 31, 2021 was $10,232. The total intrinsic value of stock options exercisable for the three months ended March 31, 2022 and 2021 was $41,577 and $88,450, respectively.

The weighted average grant date fair value of options granted during the three months ended March 31, 2022 and 2021 was $12.38 and $33.24, respectively.

The weighted average grant date fair value of options vested at March 31, 2022 was $7.96.

The weighted average remaining contractual life is 6.2 years for options exercisable as of March 31, 2022. The weighted average remaining contractual life is 7.4 years for options outstanding.

Stock-Based Compensation

The fair value of options granted during the three months ended March 31, 2022 and 2021 was estimated using the Black-Scholes pricing model with the following assumptions:

	Three months ended March 31, 2022			Three months ended March 31, 2021		
Expected term	6.08 years	-	6.08 years	5.50 years	-	6.25 years
Expected stock price volatility	92.74 %	-	96.69 %	95.00 %	-	98.00 %
Risk-free rate of interest	1.46 %	-	2.55 %	1.04 %	-	1.22 %
Expected dividend	0.0 %	-	0.0 %	0.0 %	-	0.0 %

In 2021, the Company began using a combination of the historical volatility of publicly traded peer companies and the limited historical information related to the Company's common stock to estimate volatility. 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 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 determined by reference to the U.S. Treasury yield curve in effect at the time of grant, with maturities approximating the expected term of the stock options. The fair value of the underlying common stock is generally determined as the closing price of the Company's common stock on the Nasdaq Capital Market on the grant date, with consideration of whether there is material nonpublic information that could impact that estimated fair value when it is released.

The Company recognized stock-based compensation expense related to stock options issued under the 2017 Plan and the 2020 Plan of $3,825 and $5,565 for the three months ended March 31, 2022 and 2021, respectively, which were comprised as follows:

	Three months ended March 31,	
	2022	2021
Research and development	$ 1,001	$ 2,032
General and administrative	2,824	3,533
Total	$ 3,825	$ 5,565

Unrecognized compensation expense related to unvested stock option awards as of March 31, 2022 was $22,835 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 1.8 years.

2020 Employee Stock Purchase Plan

The Company's 2020 Employee Stock Purchase Plan (the "ESPP") was also approved and became effective at the Company's 2020 annual meeting of stockholders on May 20, 2020. The ESPP is designed to assist eligible employees of the Company with the opportunity to purchase the Company's common stock at a discount through accumulated

payroll deductions during successive offering periods. The aggregate number of shares that may be issued pursuant to rights granted under the ESPP is 100 shares of common stock. In addition, the number of shares available for issuance under the ESPP will increase on the first day of each calendar year, beginning on January 1, 2021 and ending on and including January 1, 2030, by a number of shares of common stock equal to the lesser of (a) 1% of the shares outstanding on the final day of the immediately preceding calendar year and (b) such smaller number of shares as determined by the Board of Directors. The number of shares that may be issued or transferred pursuant to rights granted under the component of the ESPP that is intended to qualify for favorable U.S. federal tax treatment under Section 423 of the Internal Revenue Code (the "Section 423 Component") shall not exceed 500 shares. The purchase price will be determined by the administrator of the ESPP and, for purposes of the Section 423 Component, shall not be less than 85% of the fair value of a share on the first trading day or on the last trading day of the applicable offering period, whichever is lower. The shares available for issuance under the 2020 ESPP increased by 156 shares and 244 shares on January 1, 2022 and 2021, respectively. To date, no shares have been sold under the ESPP.

Note 10. Leases

BTI leases office space for its corporate headquarters at 555 Long Wharf Drive, New Haven, Connecticut (the "HQ Lease") under an operating lease. The HQ Lease was effective in February 2019, was subject to an amendment for additional office space in August 2020 and expires in February 2026. The Company has an option to renew the HQ 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. Payments under the HQ Lease are fixed.

The Company also leases equipment such as copiers and information technology equipment.

The future minimum annual lease payments under operating leases, as of March 31, 2022, are as follows:

Year ending December 31,	Amount
Remainder of 2022	$ 273
2023	372
2024	381
2025	391
2026	65
Thereafter	—
Total lease payments	$ 1,482
Less imputed interest	(155)
Total lease liability	$ 1,327
Less current portion of lease liability	(299)
Long-term portion of operating lease liability	$ 1,028

The current portion of the Company's operating lease liability of $299, as of March 31, 2022, is included in other current liabilities on the balance sheet.

The Company recorded lease expense of $98 and $109 related to its operating lease ROU asset for the three months ended March 31, 2022 and 2021, respectively.

Lease renewal options are not included in the ROU asset.

Note 11. Commitments and Contingencies

From time to time, in the ordinary course of business, the Company may be subject to litigation and regulatory examinations as well as information gathering requests, inquiries and/or investigations. The Company is not currently subject to any matters where it believes there is a reasonable possibility that a material loss may be incurred. As of March 31, 2022, there were no matters which would have a material impact on the Company's financial results.

In February 2022, the Company executed a statement of work (the "February 2022 SOW") with Innovative Clinical Research, Inc. ("ICR") under a Master Clinical Trial Agreement that BTI has with ICR. As part of the February 2022 SOW, the Company will pay ICR a minimum of $8,250 to conduct a study in support of the potential use of BXCL501 to treat agitation in patients with Alzheimer's disease.

BTI signed a distribution agreement (the "Distribution Agreement") with an affiliate of Cardinal Health, Inc. ("Cardinal") in February 2022, whereby Cardinal agreed to distribute product related to IGALMI™ in the U.S. The Distribution Agreement has an initial term of three years and automatically renews for successive one-year periods, unless terminated by either party with at least 90-day notice. Cardinal will be paid defined fees for its services under the Distribution Agreement, which can be terminated by either party for cause. The Distribution Agreement can also be terminated by BTI without cause, subject to payment of agreed termination fees.

Note 12. Subsequent Events

FDA approval of BXCL501

On April 5, 2022, the FDA approved IGALMI™ (Dex) sublingual film for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults (previously referred to herein as "BXCL501"). IGALMI™ can be self-administrated by patients under the supervision of a healthcare provider. The Company is preparing to load product into U.S. distribution channels in the third quarter of 2022.

Oaktree financing facilities

On April 19, 2022 (the "Effective Date"), the Company entered into two strategic financing agreements; a Credit Agreement and Guaranty (the "Credit Agreement") with Oaktree Fund Administration, LLC ("Oaktree") as administrative agent, and the lenders party thereto (the " Lenders"), and a Revenue Interest Financing Agreement (the "RIFA"; and together with the Credit Agreement, the "Credit Facilities") with Oaktree as administrative agent, and the purchasers party thereto (the " Purchasers"). Under the Credit Facilities, the Lenders and the Purchasers will provide up to $260,000 in gross funding to support the Company's commercial activities of IGALMI™ sublingual film. In addition, the Credit Facilities are intended to support the expansion of clinical development efforts of BXCL501, which includes a pivotal Phase 3 program for the acute treatment of agitation in patients with Alzheimer's disease, and for the Company's additional neuroscience and immuno-oncology clinical programs.

A high-level summary of the Credit Facilities is provided below.

- *The Credit Agreement*: The Credit Agreement provides up to $135,000 in senior secured term loans, of which $70,000 was funded on April 28, 2022, and the remaining tranches may be borrowed at the Company's option prior to December 31, 2024, subject to satisfaction of certain conditions, including regulatory and financial milestones. The loans under the Credit Agreement mature on the fifth anniversary of the effective date; provided that the Company may, at its option, extend the maturity date to the sixth anniversary if, prior to December 31, 2024, the Company receives approval from the FDA of a new drug application ("NDA") in respect of the use of BXCL501 for the acute treatment of agitation associated with Alzheimer's disease and satisfies certain other conditions. Borrowings under the Credit Agreement bear interest at a fixed annual rate of 10.25%, payable quarterly. Of such interest, 2.25% per annum will be payable in kind by capitalizing and adding such interest to the outstanding principal amount of loans from the first payment date on which such interest is owed through, and including, the third anniversary of such payment date, unless, with respect to any payment date, the Company elects to pay all or a portion of such interest in cash. The Company is required to pay a ticking fee equal to 0.750% per annum on the undrawn amount of the commitments. The Company may voluntarily prepay the Credit Agreement at any time subject to a prepayment fee.

  The Company's obligations under the Credit Agreement are guaranteed by BTI's existing and subsequently acquired or organized subsidiaries, subject to certain exceptions. The Credit Agreement contains customary representations and warranties and customary affirmative and negative covenants. The Company must also comply with certain financial covenants. Furthermore, the Credit Agreement contains events of default that are

customary for financings of this type. In certain circumstances, events of default are subject to customary cure periods.

- *The RIFA*: The RIFA provides up to $120,000 in financing in exchange for a capped revenue interest on net sales of IGALMI™ and any other future BXCL501 products. The financing under the RIFA may be drawn by the Company at its option prior to December 31, 2024, subject to satisfaction of certain conditions, including certain regulatory, patent and financial milestones.

  Under the terms of the RIFA, the Purchasers will receive tiered revenue interest payments on U.S. net sales of IGALMI™ and any other future BXCL501 products equal to a royalty ranging from 0.375% to 7.750% of net sales of IGALMI™ and any other future BXCL501 products in the U.S., subject to a hard cap equal to 1.75x of the total amount funded. In addition, if the conditions to the second tranche of the financing provided under the RIFA have been met, once payments equal to the hard cap have been received by the Purchasers, the Company will be required to make revenue interest payments equal to a flat 0.375% royalty on U.S. net sales of IGALMI™ and any other future BXCL501 products through, and including March 31, 2036. The Company is also required to make certain additional payments to the Purchasers from time to time to ensure that the aggregate amount of payments received by the Purchasers under the RIFA is at least equal to certain agreed upon minimum levels as of certain specified dates, subject to terms and conditions set forth in the RIFA.

  BTI has the right, but not the obligation, to buy out the Purchasers' interests in the revenue interest payments at an agreed upon repurchase price.

  The Company's obligations under the RIFA are secured by a perfected security interest in (i) accounts receivable arising from net sales of BXCL501 in the U.S. and one or more segregated bank accounts maintained for the purpose of receiving payments in respect of such accounts receivable, (ii) intellectual property that is claiming or covering BXCL501 itself or any method of using, making or manufacturing BXCL501 and (iii) regulatory approvals, clinical data, and all other assets that underlie BXCL501.

  The RIFA contains customary representations and warranties and certain restrictions on the Company's ability to incur indebtedness and grant liens on intellectual property related to BXCL501. In addition, the RIFA provides that if certain events occur, including certain bankruptcy events, failure to make payments, a change of control, an out-license or sale of all of the rights in and to BXCL501 in the U.S., in each case except a permitted licensing transaction (as defined in the RIFA) and, subject to applicable cure periods, material breach of the covenants in the RIFA, Oaktree, at the direction of the Purchasers, may require the Company to repurchase the Purchasers' interests in the revenue interest payments at an agreed upon repurchase price.

- *BTI Warrants and Rights to Purchase BTI Common Stock*: In connection with the Credit Agreement, on the Effective Date, the Company granted warrants to the Lenders to purchase up to 278 shares of our common stock (the "Warrants") at an exercise price of $20.04 per share, which represents the arithmetic average of the volume-weighted average price of the Company's common stock on the Nasdaq Capital Market during the 30 trading days preceding the issuance of the Warrants. The Warrants will expire on April 19, 2029, and may be net exercised at the holder's election. In addition, pursuant to the Credit Agreement, the Lenders have the right to purchase shares of the Company's common stock after the Effective Date, for a purchase price of $5,000 at a price per share equal to a 10% premium to the volume weighted average price of the common stock over the 30 trading days prior to the Lenders' election to proceed with such equity investment.

- *OnkosXcel Warrants*: In connection with the closing of the Credit Agreement, OnkosXcel Therapeutics, Inc. ("OnkosXcel"; a wholly owned subsidiary of BTI) granted warrants to the Lenders to purchase an ownership interest equal to 0.875% of its fully diluted capitalization as of the closing of the Credit Agreement, subject to increase to up to an aggregate of 1.75% of its fully diluted capitalization as of the closing of the Credit Agreement based on the funding of the two delayed draw tranches of loans provided for under the Credit

Agreement (the "701 Warrants"). The 701 Warrants will expire on April 19, 2029, and may be net exercised at the holder's election.

Item 2. 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 our financial statements and related notes appearing elsewhere in this report and the audited financial statements and related notes contained in our Annual Report on Form 10-K for the year ended December 31, 2021. 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, and those discussed in the section titled "Risk Factors" included elsewhere in this report. All dollar amounts in the below Management's Discussion and Analysis of Financial Condition and Results of Operations are presented in U.S. dollars, and all dollar amounts are presented in thousands, unless otherwise noted or the context otherwise provides. All share amounts are also presented in thousands.

Overview

We are a biopharmaceutical company utilizing artificial intelligence ("AI") approaches to develop transformative medicines in neuroscience and immuno-oncology. We are focused on utilizing cutting-edge technology and innovative research to develop high-value therapeutics aimed at transforming patients' lives. We employ a unique AI platform in an effort to reduce therapeutic development costs and potentially accelerate timelines while aiming to increase the possibility of success. Our approach leverages existing approved drugs and/or clinically evaluated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. We believe that this differentiated approach has the potential to reduce the expense and time associated with drug development in diseases with substantial unmet medical needs.

Our most advanced clinical development program is BXCL501, an investigational proprietary, orally dissolving, thin film formulation of dexmedetomidine ("Dex") for the treatment of agitation associated with psychiatric and neurological disorders.

On April 6, 2022 we announced that the United States of America ("U.S.") Food and Drug Administration ("FDA") had approved IGALMI™ (Dex sublingual film) for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. IGALMI™ is approved to be self-administrated by patients under the supervision of a healthcare provider. The Company is preparing to load product into U.S. distribution channels in the third quarter of 2022.

We continue to conduct clinical trials evaluating BXCL501 for the treatment of agitation in Alzheimer's disease patients, and for adjunctive treatment of patients with Major Depressive Disorder ("MDD"), as well as in the community for agitation associated with bipolar disorders and schizophrenia.

Our advanced oncology asset, BXCL701, is an investigational orally administered systemic innate immune activator for the treatment of a rare form of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors.

The novel coronavirus disease ("COVID-19") pandemic and government measures taken in response have significantly impacted, both directly and indirectly, businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen.

Since the onset of the COVID-19 pandemic, we have taken steps in line with guidance from the U.S. Centers for Disease Control and Prevention and the State of Connecticut to protect the health and safety of our employees and community. We have instituted a return-to-the-office policy and continue to evaluate that policy.

We also continue to work closely with our clinical sites to monitor the potential impact of the evolving COVID-19 pandemic and the spread of its variants. To-date, we have not experienced any significant delays in any of our ongoing or planned clinical trials. However, this could rapidly change.

Commercial and Launch Readiness

We have been actively preparing for the commercial launch of IGALMI™ for several quarters. We identified 1,700 hospital targets and 59 Integrated Delivery Networks ("IDNs") that represent 75% of the institutional opportunity of our target market. The sales management team has been in place since the fourth quarter of 2021 and the initial field Institutional Specialists have already joined the Company. The tenure of this team averages over 21 years of industry experience (14 in the hospital setting), eight product launches and over six years' experience in Central Nervous System disorders. Training will commence in the second quarter of 2022, with field launch also beginning later in the quarter.

The Market Access team is onboard and we anticipate training will also commence in the second quarter of 2022. The team will focus on contracting with the major Group Purchasing Organizations and the highest potential prescribing IDNs immediately post-training. This process typically takes six to nine months from the announcement of the Wholesale Average Cost price, therefore the Company anticipates securing contracts beginning in the second half of 2022. This process will work in parallel with field force efforts to coordinate the Pharmacy and Therapeutics committee meetings to secure formulary access at the individual hospital level.

IGALMI's™ Wholesale Acquisition Cost will be $105 per film across dosage strengths. The 120-microgram and 180-microgram doses of IGALMI™ will be packaged into heat-sealed foil pouches of 10 and 30-count films per carton.

We have completed the production of IGALMI's™ sales materials and narrative to be deployed by our field teams. This brand story has encouraged us to accelerate our build out of other avenues of communication, such as peer-influence speaker programs and focused advertising opportunities. As our field teams begin to create demand, we anticipate that these marketing efforts will drive uptake.

The Company's Medical Science Liaison and Medical Managed Care team continue to actively engage with healthcare professionals, patient support organizations, and payors to provide key insights to support our commercial strategy as well as to fully understand the needs of relevant patients, professions, and institutions. The Medical Affairs organization is also actively working on training of our commercial and sales personnel in preparation for IGALMI's™ launch. In addition, our Medical Information structure and processes were finalized, and the Company's call center is fully operational.

The Company signed a distribution agreement (the "Distribution Agreement") with an affiliate of Cardinal Health, Inc. ("Cardinal") in February 2022, whereby Cardinal agreed to distribute product related to BXCL501 in the U.S., which distribution right was contingent on FDA approval of BXCL501 in the U.S. The Distribution Agreement has an initial term of three years and automatically renews for successive one-year periods, unless terminated by either party with at least 90-day notice. Cardinal will be paid defined fees for its services under the Distribution Agreement, which can be terminated by either party for cause. The Distribution Agreement can also be terminated by BTI without cause, subject to payment of agreed termination fees.

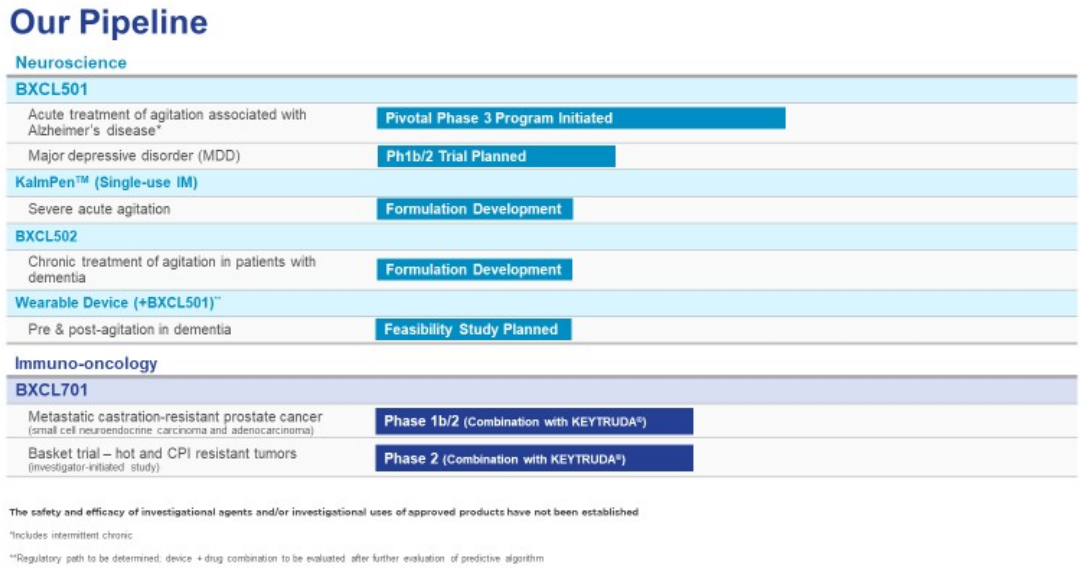
On April 1, 2022, the Company signed a commercial supply agreement with ARx, LLC ("ARx"), whereby ARx agreed to manufacture, and supply Dex product related to IGALMI™ and BXCL501. The contract has an initial term of 10 years and can be extended for successive one-year periods, subject to BXCL501 still being marketed or sold. The commercial supply agreement can be canceled upon mutual agreement, for cause or upon certain conditions being met, including BXCL501 no longer being marketed or sold by or on behalf of BTI.

As disclosed further under "Liquidity and Capital Resources" below, on April 19, 2022 (the "Effective Date"), we entered into two strategic financing agreements; a Credit Agreement and Guaranty (the "Credit Agreement") with Oaktree Fund Administration, LLC ("Oaktree") as administrative agent, and the lenders party thereto (the " Lenders"), and a Revenue Interest Financing Agreement (the "RIFA"; and together with the Credit Agreement, the "Credit Facilities") with Oaktree as administrative agent, and the purchasers party thereto (the " Purchasers"). Under the Credit Facilities, the Lenders and the Purchasers will provide up to $260,000 in gross funding to support the Company's commercial activities of IGALMI™ sublingual film. In addition, the Credit Facilities are intended to support the expansion of clinical development efforts of BXCL501, which includes a pivotal Phase 3 program for the acute

treatment of agitation in patients with Alzheimer's disease, and for the Company's additional neuroscience and immuno-oncology clinical programs.

Our Clinical Programs

The following is a summary of the status of our clinical development programs as of the date of this Quarterly Report on Form 10-Q:



BXCL501 Neuroscience Program

In indications other than approved by the FDA as IGALMI™, BXCL501 remains an investigational, proprietary, orally dissolving, thin film formulation of Dex, a selective alpha-2a receptor agonist, targeting symptoms from stress-related behaviors such as agitation. BXCL501 is our most advanced neuroscience clinical program, developed or being developed for the acute treatment of agitation related to schizophrenia, bipolar disorders, Alzheimer's disease, and as an adjunctive treatment for MDD in conjunction with the use of Selective Serotonin Reuptake Inhibitors ("SSRI") or Serotonin Norepinephrine Reuptake Inhibitors alone ("SNRI").

As a selective adrenergic agent with a sublingual or buccal route of administration, BXCL501 is designed to be easily administered and has shown a rapid onset of action in multiple clinical trials, including clinical trials studying patients with schizophrenia, bipolar disorders, Alzheimer's disease. We believe results from these studies suggest that BXCL501 has the potential to generate a calming effect without producing excessive sedation. We also believe BXCL501 is highly differentiated from antipsychotics, which often produce unwanted side effects such as excessive sedation or extra pyramidal motor effects, currently used as a standard of care to treat agitation. Managing patient agitation in neuropsychiatric and neurodegenerative disorders represents a significant challenge for physicians and caregivers. We believe BXCL501 has the potential to address these challenges while providing an efficient treatment regimen for patients.

BXCL501 Clinical Trials

Tranquility Program

The Tranquility 1 study of agitation in dementia (BXCL501-103) concluded with a total of four sites enrolling 46 subjects in Part B testing the 40 microgram ("mcg") dose versus placebo. Topline data were reviewed with respect to efficacy, safety and tolerability. The purpose of enrolling this additional cohort was to gather additional evidence supporting dose selection and statistical powering of multiple-site Phase 3 pivotal trials. All patients were able to take the film themselves and properly place it. There were no serious adverse events related to the drug, and no falls, loss of consciousness or syncopal events reported. The single discontinuation from the trial, for a patient hospitalized with sepsis, was unrelated to treatment. There were no local tolerability issues. The adverse events ("AEs") observed for 40 mcg were consistent with those previously observed for 30 mcg, 60 mcg and placebo doses. As expected, the incidence of individual and categorical AEs for the 40 mcg dose were lower than the 60 mcg group, and similar to the 30 mcg dose group.

Efficacy was measured by the change from pre-dose baseline Positive and Negative Syndrome Scale Excitatory Component ("PEC") total score at two hours, the same primary endpoint utilized in prior pivotal trials of BXCL501. The 40 mcg dose showed statistically significant reductions in PEC total score at two hours ($p<0.001$) and demonstrated statistically significant separation from placebo as early as one hour. The magnitude of change in PEC total score was greater for the 40 mcg dose than that of 30 mcg and somewhat less than the 60 mcg dose in previous cohorts. Overall, we believe the 40 mcg data support continued evaluation of both 40 and 60 mcg doses.

We believe that, taken as a whole, these data support the initiation of pivotal trials evaluating patients with acute agitation associated with possible Alzheimer's dementia.

On December 15, 2021, after our initial Breakthrough Therapy designation meetings with FDA, we announced the initiation of our program to evaluate BXCL501 for the treatment of acute agitation associated with dementia in Alzheimer's patients. Patient enrollment is currently underway. The program's two studies, TRANQUILITY II and TRANQUILITY III, are designed to evaluate the safety and efficacy of BXCL501 in adults 65 years and older across the range of illness including mild, moderate and severe illness in assisted living or residential facilities and nursing homes.

- The program will consist of two randomized, double-blind placebo-controlled, adaptive, parallel group pivotal trials, TRANQUILITY II and TRANQUILITY III.

- Each study will enroll approximately 150 dementia patients 65 years and older. Patients will self-administer 40 mcg or 60 mcg of BXCL501 or placebo whenever agitation episodes may occur.

- TRANQUILITY II will enroll patients with mild to moderately severe dementia in assisted living or residential facilities who generally require minimal assistance with activities of daily living. TRANQUILITY III will enroll patients in nursing homes with moderate to severe dementia who require moderate or greater assistance with their activities of daily living. On May 3, 2022, we announced the first patient has been dosed in this study.

- The studies are designed to assess agitation as measured by the changes from baseline in the PEC and post-authorization studies total scores. The primary efficacy endpoint for both studies will be the change in PEC total score from baseline measured at two hours after the initial dose and subsequent doses.

- Patients who complete TRANQUILITY II or TRANQUILITY III will be eligible to enroll in an open label, 52-week safety study designed to describe the safety and efficacy of BXCL501 in continued use.

The clinical trial sites for this trial are now open and enrolling patients.

Major Depressive Disorder (MDD)

We have recently expanded our development pipeline to evaluate BXCL501 as an adjunctive treatment for MDD. We expect that the initial clinical study in this program will be a double-blind, placebo-controlled, multiple ascending dose trial to evaluate the safety and tolerability of twice daily doses of BXCL501 in healthy volunteers. A Phase 2 proof of concept study is then planned to evaluate whether daily adjunctive use of BXCL501 provides a more rapid initial clinical antidepressant response than placebo when initiating SSRIs or SNRIs alone.

Pediatric Study

In June 2021, we initiated a global clinical trial designed to evaluate the safety and efficacy of BXCL501 in the acute treatment of agitation associated with pediatric schizophrenia and bipolar I or II disorder, in part to fulfill pediatric study requirements agreed to with the FDA in connection with IGALMI's™ approval. The trial protocol has been reviewed by the FDA, as well as by the European Medicines Agency ("EMA") to fulfill potential commitments to study the effects of BXCL501 in patients ages 13-17 with schizophrenia and ages 10-17 with bipolar I or II disorder. The multisite double-blind placebo controlled parallel group trial will enroll patients with schizophrenia, schizoaffective disorder, bipolar I and bipolar II disorder. Similar to our original trials in schizophrenia and bipolar disorder, (SERENITY I and II), the primary endpoint is the change from baseline PEC total score at two hours. The trial has been initiated in the U.S. and patients are currently enrolling in various sites. Clinical trial authorizations and ethics committee approvals have been obtained in Europe and we continue to work towards enrolling pediatric patients in Europe.

Additional Neuroscience Opportunities

Pipeline Opportunities for Franchise Expansion

In December 2020, the VA Connecticut Healthcare System and Yale University Medical School were awarded a grant by the U.S. Department of Defense's Congressionally Directed Medical Research Programs to evaluate BXCL501 in patients with post-traumatic stress disorder who suffer from alcohol use disorder ("AUD"). The Company is providing BXCL501 for studies that will evaluate whether BXCL501 has the potential to treat AUD in this patient population.

BTI is currently conducting studies designed to develop algorithms for wearable technologies that are designed to detect the early signs of agitation. Feasibility studies demonstrated that patients were able to wear these devices and that these devices were able to transmit data to our service vendor. The goal of this program is to establish a predictive algorithm that would allow caregivers to treat patients before they become highly agitated.

Other Neuropsychiatric / Neurodegenerative Indications and Formulations

Given the differentiated design of BXCL501 and its selective mechanism of action, we believe BXCL501 has the potential for broad applicability across several indications where agitation is a symptom of a condition or underlying disease. As an example, one goal is to treat symptoms in patients suffering from major depressive episodes. Current drugs, like serotonin uptake inhibitors, are not effective in treating some of the symptoms of depression. We believe BXCL501 could potentially alleviate some of the unresolved symptoms that result in poor clinical outcomes for these patients.

We recently identified a second neuropsychiatric drug candidate, BXCL502, through our AI-based platform. We plan to evaluate BXCL502 initially as a monotherapy and possibly as a combination with BXCL501 for the chronic treatment of agitation in patients with dementia. The active pharmaceutical ingredient ("API") underlying BXCL502 is designed to affect serotonergic signaling in the brain. Our preclinical data suggests BXCL502 has potential to treat stress-related neuropsychiatric symptoms in dementia. In previously published third-party clinical trial data, daily administration of the API of BXCL502 demonstrated improvement in behaviors using a well-established, clinically validated symptom scale. Formulation and clinical development planning are currently underway with BXCL502.

Other Product Candidates

We are targeting neuropsychiatric disorders with high unmet medical needs. Our focus is on treating stress-related symptoms, such as agitation, that are responsible for higher levels of institutionalized care. We are also using machine learning to identify new approaches for rare neurological 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 we believe can enter the clinic with the potential for an efficient development path.

OnkosXcel Therapeutics, Inc.

On April 19, 2022, we announced the formation of a wholly owned subsidiary, OnkosXcel Therapeutics, Inc. ("OnkosXcel") to develop potentially transformative medicines in oncology. OnkosXcel is focused on the sustained expansion and optimization of our oncology franchise, while providing maximum strategic and financial flexibility. OnkosXcel plans to progress the development of BXCL701, an investigational orally administered innate immune activator designed to initiate inflammation in the tumor microenvironment.

BXCL701 Immuno-Oncology Program

BXCL701 is a potential first-in-class, oral, small-molecule immunomodulator designed to stimulate both the innate and acquired immune systems by inhibiting dipeptidyl peptidase ("DPP") 8/9. DPP 8/9 behave as "checkpoints" of pyroptosis and inflammasome activation. We believe that BXCL701, if successfully developed and approved, may establish a differentiated immuno-oncology platform by modulating multiple steps in the cancer immunity cycle and, when combined with checkpoint inhibitors and/or immune activating agents, may be able to convert immuno-resistant ("cold") tumors to immuno-sensitive ("hot") tumors.

BXCL701 Clinical Trials

BXCL701 is currently being evaluated in two Phase 2 combination therapy clinical trials for the treatment of metastatic castration resistant prostate cancer ("mCRPC") in patients with either adenocarcinoma or small cell neuroendocrine cancer ("SCNC") type, and in patients with advanced solid cancers.

Small-cell neuroendocrine carcinoma ("SCNC"; Orphan Segment of Prostate Cancer) and mCRPC

BXCL701 was previously studied in multiple clinical trials and demonstrated single agent anti-tumor activity in melanoma, an immune-sensitive tumor. Our Phase 2 efficacy portion of the Phase 1b/2 trial evaluating BXCL701 in combination with KEYTRUDA® (pembrolizumab, a Programmed Cell Death Protein 1 ("PD 1") inhibitor) for SCNC continues. In addition to the efficacy cohort in SCNC patients, we are also pursuing a separate cohort for adenocarcinoma patients who have failed taxane-based chemotherapy and up to two lines of second-generation androgen pathway blockers. Topline results from the Phase 1b portion of this trial were presented at the American Society of Clinical Oncology Genitourinary Symposium in February 2021. Interim data from the Phase 2 portion of the study were also presented at the European Society for Medical Oncology conference in September 2021. We reported interim data from the Phase 2 portion of this trial for the SCNC cohort and final results for the adenocarcinoma cohort at the American Society of Clinical Oncology ("ASCO") Genitourinary Cancers Symposium in February 2022.

BXCL701 is being evaluated in combination with KEYTRUDA® (pembrolizumab) in an ongoing Phase 2 trial in mCRPC patients with either adenocarcinoma or SCNC phenotype. The first adenocarcinoma patient was enrolled in the phase 2b randomized 60-patient expansion of the study.

Basket Trial

BXCL701 is also 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 patients who are naïve to checkpoint therapy and those who are refractory to checkpoint therapy in two separate cohorts. Interim data were presented at the June 2021 ASCO annual meeting. In the second half of 2022, we expect to present additional interim efficacy data from the trial.

Other Immuno-oncology Indications

In addition to mCRPC and checkpoint inhibitor-refractory tumors, 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 potential mechanism of action. We are prioritizing indications where the immunosuppressive 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.

We believe that 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 / Programmed Cell Death Ligand 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 (i.e., chimeric antigen receptor T-cell and natural killer cell therapies);
- therapeutic vaccines; and
- Antibody-dependent cellular cytotoxicity driven monoclonal antibodies.

Other Immuno-Oncology Product Candidates

Our immuno-oncology program is based on utilizing a comprehensive map of all currently known relationships that link immuno-evasion and immuno-activation pathways and targets with thousands of pharmacological agents and tumor indications. This comprehensive map permits 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 to select and prioritize additional development opportunities to expand the current portfolio and broaden the addressable market for our lead programs through the identification of new indications. This includes exploring additional combination therapy approaches to expand BXCL701's target indications beyond neuroendocrine prostate cancer and castration resistant prostate cancer.

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 U.S. 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, maintain, and strengthen, 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 April 26, 2022, our patent portfolio included five Patent Cooperation Treaty ("PCT") applications, 18 U.S. utility applications, one issued U.S. utility patent, six U.S. provisional patent applications, 103 pending non-U.S. applications, 13 allowed or granted non-U.S. patents (including five in Japan), one design patent application, which is a U.S. design application, and 34 allowed or registered design patents (including two in Japan). U.S. Pat. No. 10,792,246, directed to our proprietary sublingual thin-film formulation of Dex, was issued on October 6, 2020 and has a term set to expire no earlier than 2039. U.S. Patent No. 10,792,246 is now listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the "Orange Book"). We have filed applications in the core patent family protecting BXCL501 in the U.S., Taiwan, and other major markets. We expect that patents issuing from these applications, if any, will expire no earlier than 2039. We have also filed applications in additional patent families that are relevant to BXCL501. We have applications pending in the U.S., 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 15 regions, including the U.S., 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. We have one U.S. application and one European application directed to intravenous administration of Dex. We expect that patents issuing from these applications, if any, will expire no earlier than 2039. We continue to file new applications on an ongoing basis, including provisional applications directed to treating mania and dementia. If patents issue from those cases, we expect them to expire no earlier than 2041 and 2042, respectively.

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 U.S. and 14 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 multiple 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.

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 U.S., 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 U.S. 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 (a.k.a., the "Hatch-Waxman Act"). The 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 investigational new drug ("IND"), and the submission date of a new drug application ("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 patent expiration. The U.S. Patent and Trademark Office ("USPTO"), 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 U.S. Relevant patent laws and their interpretation outside of the U.S. are also uncertain. Changes in either the patent laws or their interpretation in the U.S. 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 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.

Basis of Presentation

The Company's financial statements are prepared in accordance with U.S Generally Accepted Accounting Principles ("GAAP").

Components of Our Results of Operations

Revenues

We have not recognized any revenue since inception.

Operating Costs and Expenses

Research and Development

Our research and development expenses reflect costs incurred for the research and development of our clinical and preclinical product candidates, which includes payments to BioXcel LLC. Research and development expense primarily consist of salary, benefits and non-cash stock-based compensation for our research and development personnel, costs incurred under agreements with contract research organizations ("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 preclinical and clinical trial materials and lab supplies, and depreciation and other expenses.

We expense research and development costs as incurred.

Our research and development costs by program for the three months ended March 31, 2022 and 2021 were as follows:

	Three months ended March 31,	
	2022	2021
Direct external costs		
BXCL501	$ 8,516	$ 6,160
BXCL701	2,915	2,072
Other research and development programs	552	347
Total direct external costs	$ 11,983	$ 8,579
Internal personnel costs	5,852	5,658
Sub-total direct costs	$ 17,835	$ 14,237
Indirect costs and overhead	852	629
Research and development tax credit	(128)	(125)
Total research and development expenses	$ 18,559	$ 14,741

General and Administrative

General and administrative expenses primarily consist of salaries, benefits and non-cash stock-based compensation for our executive and administrative personnel. General and administrative expenses also include legal expenses to pursue patent protection of our intellectual property, professional fees for audit and tax services and insurance charges.

We expect that our general and administrative expenses will increase as we expand our clinical programs. We also 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 may also 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 3 to the financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Results of Operations

Comparison of the Three Months Ended March 31, 2022 and 2021

Revenues

We have not recognized any revenues since inception.

Research and Development Expense

Research and development expenses for the three months ended March 31, 2022 and 2021 were $18,559 and $14,741, respectively, and were comprised as follows:

	Three months ended March 31,			
	2022	2021	Change	% Change
Personnel and related costs	$ 4,876	$ 3,626	$ 1,250	34 %
Non-cash stock-based compensation	975	2,032	(1,057)	(52)%
Professional fees	3,924	3,751	173	5 %
Clinical trials expense	6,315	3,386	2,929	87 %
Chemical, manufacturing and controls cost	1,940	1,713	227	13 %
Travel and other costs	657	358	299	84 %
Research and development tax credit	(128)	(125)	(3)	(2)%
Total research and development expenses	$ 18,559	$ 14,741	$ 3,818	26 %

The overall increase of $3,818 for the three months ended March 31, 2022 is primarily attributable to:

- An increase in personnel costs related to our efforts to enlarge our clinical team as we expanded our clinical trials for the use of BXCL501 for agitation in patients with Alzheimer's disease and MDD. These efforts also contributed to the increase in the chemical, manufacturing and controls ("CMC") costs, a portion of which were recorded as inventory at March 31, 2022 (i.e., active pharmaceutical ingredient we believe will be used for commercial production). CMC costs increased in 2022 compared to 2021 as we produced materials related to our clinical trials of BXCL501 for the treatment of Alzheimer's disease. Following IGALMI's™ approval by the FDA, we will continue to capitalize costs related to commercial production of IGALMI™ as inventory and will continue to expense those CMC costs related to clinical trials.

- A decrease in non-cash stock-based compensation which is the result of lower grant date fair values arising from lower prices of the Company's common stock.

- An increase in clinical trial expenses due to the initiation of the TRANQUILITY study of BXCL 501 for the potential treatment of agitation in patients with Alzheimer's disease.

- An increase in travel and other costs as the Company resumed a more traditional travel schedule.

The State of Connecticut provides companies with the opportunity to exchange certain research and development credit carryforwards for cash in exchange for foregoing the carryforward of the research and development credit. The program provides for such exchange of the research and development credit at a rate of 65% of the annual research and development credit. The benefit for such exchange is recorded as a reduction of research and development expenses.

General and Administrative Expense

General and administrative expenses for the three months ended March 31, 2022 and 2021 were $12,921 and $11,638, respectively, and were comprised as follows:

	Three months ended March 31,			
	2022	2021	Change	% Change
Personnel and related costs	$ 3,206	$ 2,290	$ 916	40 %
Non-cash stock-based compensation	2,850	3,533	(683)	(19)%
Professional fees	4,131	2,611	1,520	58 %
Commercial	1,483	2,418	(935)	(39)%
Insurance	570	466	104	22 %
Travel and other costs	681	320	361	113 %
Total general and administrative expenses	$ 12,921	$ 11,638	$ 1,283	11 %

The overall increase of $1,283 for the three months ended March 31, 2022 is primarily attributable to:

- An increase in personnel and related costs due to our continuing efforts to expand our teams in preparation for the commercial launch of BXCL501 in the U.S.
- A decrease in non-cash stock-based compensation due to lower grant date fair values arising from lower prices of the Company's common stock.
- An increase in professional fees due to the expanding growth of our operations, primarily for corporate legal fees and consulting and recruiting costs related to our preparation for the commercial launch of BXCL501 in the U.S., as well as the formation of OnkosXcel.
- A decrease in commercial related fees which were primarily a result of costs being incurred in prior periods for certain campaigns related to preparation of the commercial launch of BXCL501 in the U.S. Similar fees were not incurred in the current quarter.
- Increased travel and other costs as the Company resumed a more traditional travel schedule.

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 March 31, 2022, we had cash and cash equivalents of $200,435, working capital of $191,565 and stockholders' equity of $194,020. Net cash used in operating activities was $32,413 and $19,640 for the three months ended March 31, 2022 and 2021, respectively. We incurred losses of approximately $31,472 and $26,376 for the three months ended March 31, 2022 and 2021, respectively. 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 March 31, 2022, along with the Credit Facilities entered into on the Effective Date (discussed in more detail below), will enable us to fund our operating expenses and capital expenditure requirements for at least one year from the date of this Quarterly Report on Form 10-Q.

We may obtain additional financing through sales of the Company's equity securities, entering into strategic partnership arrangements and/or short-term borrowings from banks, stockholders or other related parties, if needed, or a combination of any of the foregoing. There are no assurances that we will be successful in obtaining an adequate level of financing as and when needed to finance our operations on terms acceptable to us or at all, particularly in light of the economic downturn and ongoing uncertainty related to the COVID-19 pandemic. If we are unable to secure adequate additional funding as and when needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more product candidates. In addition, the magnitude and duration of the COVID-19 pandemic and its impact on our liquidity and future funding requirements is uncertain as of the filing date of this Quarterly Report on Form 10-Q, as the pandemic continues to evolve globally. See "Risk Factors—The COVID-19 pandemic, or other pandemics, epidemics or outbreaks of an infectious disease may materially and adversely impact our business, including our preclinical studies and clinical trials." in Part II, Item 1A. of this Quarterly Report on Form 10-Q for a further discussion of the potential impact of the COVID-19 pandemic on our business.

Sources of Liquidity

We have focused our efforts on raising capital and building the products in our pipeline. Since our inception, our operations have been financed primarily from proceeds from the sale of equity securities, including our IPO, private placements of our common stock, and registered offerings of our common stock and an Open Market Sale Agreement

(the "Sale Agreement") with Jefferies LLC ("Jeffries"). 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 May 2021, we entered into the Sale Agreement with Jefferies pursuant to which we can offer and sell shares of our common stock, having an aggregate offering price of up to $100,000, from time to time, through an "at the market offering" program under which Jefferies will act as sale agent. No proceeds were received under the Sale Agreement for the three months ended March 31, 2022 and 2021.

As described further below, on the Effective Date, we entered into the Credit Agreement with Oaktree as administrative agent, and the Lenders, and the RIFA with Oaktree as administrative agent, and the Purchasers. Pursuant to the Credit Agreement, the Lenders agreed to loan us up to $135,000 in senior secured term loans. On April 28, 2022, we borrowed the first tranche of $70,000 of loans. The remaining two tranches of the commitments under the Credit Agreement may be borrowed at our option prior to December 31, 2024 as follows:

- $35,000 upon satisfaction of certain conditions, including receipt of certain regulatory and financial milestones; and

- $30,000 upon satisfaction of certain conditions, including specified minimum net sales of the Company attributable to sales of BXCL501 for a trailing twelve consecutive month period.

Pursuant to the RIFA, the Purchasers agreed to provide us with up to $120 million in financing for our near-term commercial activities of IGALMI™, development and commercialization of BXCL501 and other general corporate purposes. The funding of the first $30,000 payment was conditioned upon our receipt of BXCL501 FDA approval, which occurred on April 5, 2022 with the FDA's approval of IGALMI™. The remaining commitments under the RIFA may be drawn at our option prior to December 31, 2024 as follows:

- $45,000 payment upon satisfaction of certain conditions, including receipt of certain regulatory and patent related milestones and specified minimum net sales of BXCL501 during any consecutive twelve-month period; and

- $45,000 payment upon satisfaction of certain conditions, including receipt of certain regulatory and patent related milestones and specified minimum net sales of BXCL501 during any consecutive twelve-month period.

In connection with the Credit Agreement, on the Effective Date, we granted to the Lenders certain warrants to purchase up to 278 shares of our common stock, rights to purchase up to $5 million of our common stock and warrants in OnkosXcel described in greater detail below.

Cash Flows

	Three months ended March 31,	
	2022	2021
Cash (used in) provided by:		
Operating activities	$ (32,413)	$ (19,640)
Investing activities	$ (120)	$ (316)
Financing activities	$ —	$ 852

Operating Activities

Net cash used in operating activities for the three months ended March 31, 2022 was $32,413, which was primarily attributable to our net loss of $31,472, a $676 net decrease in accounts payable, accrued expenses and other liabilities, and a $3,480 increase in prepaid expenses, other current assets and other assets, partially offset by $3,825 in non-cash stock-based compensation and $77 of depreciation and amortization.

Net cash used in operating activities for the three months ended March 31, 2021 was $19,640 which was primarily attributable to our net loss of $26,376, partially offset by $5,565 in non-cash stock-based compensation, a $403 increase in accounts payable, accrued expenses and other liabilities, and a $703 decrease in prepaid expenses and other assets.

Investing Activities

Net cash used in investing activities for the three months ended March 31, 2022 was $120 and was primarily attributable to the purchase of equipment.

Net cash used in investing activities for the three months ended March 31, 2021 was $316 and was attributable to the purchase of equipment.

Financing Activities

Net cash provided by financing activities for the three months ended March 31, 2021 was $852 and was attributable to proceeds received from the exercise of stock options.

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 commercialize IGALMI™ and as we expand our clinical trials of and seek marketing approval for BXCL501, BXCL502 and BXCL701, while pursuing development of additional product candidates. We expect to continue to incur net losses in the near term. 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 our product candidates;
- conduct additional research and development with our product candidates;
- seek to identify, acquire, license, develop and commercialize 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 and commercial efforts;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- fully develop a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize IGALMI™ and any product candidates for which we may obtain regulatory approval; and
- continue to operate as a public company.

We believe that our existing cash and cash equivalents as of March 31, 2022, along with the Credit Facilities the Company entered into on the Effective Date (discussed in more detail below), will be sufficient to enable us to fund operating expenses and capital expenditure requirements for at least the next 12 months from the date of the issuance of the financial statements included in this Quarterly Report on Form 10-Q, including funding our ongoing research and development efforts and commercialization preparation. We expect that we will need to obtain substantial additional funding in order to fund our ongoing operations. 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 our 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 our product candidates that we otherwise would seek to develop or commercialize ourselves.

Credit Agreement

The loans under the Credit Agreement mature on the fifth anniversary of the Effective Date, provided that we may, at our option, extend the maturity date to the sixth anniversary of the Effective Date if, prior to December 31, 2024, we receive approval from the FDA of an NDA in respect of the use of BXCL501 for the acute treatment of agitation associated with Alzheimer's disease and satisfy certain other conditions. The loans under the Credit Agreement bear interest at a fixed annual rate of 10.25%, payable quarterly. Of such interest, 2.25% per annum will be payable in kind by capitalizing and adding such interest to the outstanding principal amount of loans on each quarterly interest payment date from the first payment date on which interest is owed through, and including, the third anniversary of such payment date, unless, with respect to any payment date, we elect to pay all or a portion of such interest in cash. We are required to pay a ticking fee equal to 0.750% per annum multiplied by the daily undrawn amount of the commitments commencing 120 days after the funding of the first tranche of the loans, payable quarterly through the termination of the commitments.

We may voluntarily prepay the Credit Agreement at any time subject to a prepayment fee as described in the Credit Agreement.

Our obligations under the Credit Agreement will be guaranteed by our existing and subsequently acquired or organized subsidiaries, subject to certain exceptions. Our obligations under the Credit Agreement and the related guarantees thereunder are secured, subject to customary permitted liens and other agreed upon exceptions, by (i) a pledge of all of the equity interests of all of our existing and any future direct subsidiaries, and (ii) a perfected security interest in all of our and the guarantors' tangible and intangible assets (except that the guarantees provided by the BXCL701 Subsidiaries (as defined below) are unsecured).

The Credit Agreement contains customary representations and warranties and customary affirmative and negative covenants, including, among other things, restrictions on indebtedness, liens, investments, mergers, dispositions, prepayment of other indebtedness, and dividends and other distributions, subject to certain exceptions, including specific exceptions with respect to product commercialization and development activities. We must also comply with certain financial covenants, including (i) maintenance of cash or permitted cash equivalent investments in accounts controlled by Oaktree for the Lenders, of at least (a) $15,000 from the Effective Date until the date on which the second tranche of loans are funded (the "Step-Up Date") and (b) $20,000 from and after the Step-Up Date, provided, in the case of (a) and (b), that following any Permitted BXCL701 Release Event (as defined below), such amount will increase by $12,500, and following such time as unaffiliated third parties hold ownership of at least 30% of the equity interests in the BXCL701 Subsidiaries (as defined below), such amount will increase by an additional $5,000 (provided, that such amount will in no event exceed 50% of the aggregate amount of loans outstanding at any time); and (ii) a minimum revenue test, measured quarterly beginning with our fiscal quarter ending on December 31, 2023, that requires our and our subsidiaries' consolidated net revenue for the six consecutive month period ending on the last day

of each such fiscal quarter to not be less than a minimum revenue amount specified in the Credit Agreement. Our failure to comply with the financial covenants will result in an event of default, subject to certain cure rights with respect to the revenue covenant.

Notwithstanding the foregoing, the Credit Agreement permits OnkosXcel (together with OnkosXcel Employee Holdings LLC and their respective subsidiaries, the "BXCL701 Subsidiaries") to receive third-party investment or transfer all or substantially all of their assets to an unaffiliated third party, in each case subject to terms and conditions set forth in the Credit Agreement, including the escrow of certain proceeds received by us and our subsidiaries (other than the BXCL701 Subsidiaries) in respect of these disposition events and, under circumstances set forth in the Credit Agreement, the mandatory prepayment of such escrowed amounts. Our equity interests in the BXCL701 Subsidiaries have been pledged in support of our obligations under the Credit Agreement, and the BXCL701 Subsidiaries have provided direct guarantees of our obligations under the Credit Agreement on an unsecured basis. However, the pledge, guarantee and other obligations of the BXCL701 Subsidiaries under the Term Laon will be released upon certain agreed upon events ("Permitted BXCL701 Release Events"), including an initial public offering by the BXCL701 Subsidiaries or the ownership by unaffiliated third parties of at least 20% of the equity interests in the BXCL701 Subsidiaries.

The Credit Agreement contains events of default that are customary for financings of this type relating to, among other things, payment defaults, breach of covenants, breach of representations and warranties, cross default to material indebtedness, bankruptcy-related defaults, judgment defaults, breach of the financial covenants described above, and the occurrence of certain change of control events. In certain circumstances, events of default are subject to customary cure periods. Following an event of default and any applicable cure period, the Lenders will have the right upon notice to terminate any undrawn commitments and may accelerate all amounts outstanding under the Credit Agreement, in addition to other remedies available to them as our secured creditors.

The RIFA

In exchange for the RIFA funds, we agreed to make payments to the Purchasers equal to a royalty ranging from 0.375% to 7.75% on net sales of BXCL501 in the U.S., subject to a hard cap (the "Hard Cap") equal to 175% of the total amount funded in respect of the RIFA as of any date (the "Funded Amount"). We are required to make additional payments to the Purchasers from time to time to ensure that the aggregate amount of payments received by the Purchasers under the RIFA divided by the Funded Amount (such equation, as of any date of determination, the "MOIC") is at least equal to agreed upon minimum levels as of certain dates of determination, subject to terms and conditions set forth in the RIFA.

Commencing with the calendar quarter after the Hard Cap is received by the Purchasers, we have agreed to pay the Purchasers a flat 0.375% royalty on net sales of BXCL501 in the U.S. (the "Tail Royalty Payments") through, and including, March 31, 2036. However, no Tail Royalty Payments will be owed unless the conditions to the second tranche of RIFA funds have been met (irrespective of whether we elect to receive such RIFA funds).

Our obligations under the RIFA are secured, subject to customary permitted liens and other agreed upon exceptions and subject to an intercreditor agreement between Oaktree for the Credit Agreement and RIFA, by a perfected security interest in (i) accounts receivable arising from net sales of BXCL501 in the U.S. and one or more segregated bank accounts maintained for the purpose of receiving payments in respect of such accounts receivable, (ii) intellectual property that is claiming or covering BXCL501 itself or any method of using, making or manufacturing BXCL501 and (iii) regulatory approvals, clinical data, and all other assets that underlie BXCL501.

At any time after the funding of the first RIFA tranche, we will have the right, but not the obligation (the "Call Option"), to buy out the Purchasers interest in the RIFA at a repurchase price (the "Put/Call Price") equal to, as of any date of determination, an amount sufficient that, giving effect to the payment of the Put/Call Price and all other payments made by us to the Purchasers pursuant to the RIFA, (i) the MOIC equals 1.225x if such date is before the one-year anniversary of the date the first RIFA funds were made, (ii) the MOIC equals 1.375x if such date is on or after the one-year anniversary of the date the first RIFA funds were made and before the two-year anniversary of the date the first RIFA funds were made, (iii) the MOIC equals 1.525x if such date is on or after the two-year anniversary of the date the first RIFA funds were made and before the three-year anniversary of the date the first RIFA funds were made,

and (iv) the MOIC equals 1.750x if such date is on or after the three-year anniversary of the date the first RIFA funds were made. If we exercise the Call Option prior to the third anniversary of the Effective Date, then the Purchasers will not be entitled to any Tail Royalty Payments. However, if we exercise the Call Option on or after the third anniversary of the Effective Date, then we will be required to buy out the Purchasers' interest in the Tail Royalty Payments in addition to the RIFA payments, and the applicable Put/Call Price will be an amount equal to, as of any date of determination, an amount sufficient that, giving effect to the payment of the Put/Call Price and all other payments made by us to the Purchasers pursuant to the RIFA, the MOIC equals 2.25x.

The RIFA contains customary representations and warranties and certain restrictions on our ability to incur indebtedness and grant liens on intellectual property related to BXCL501. In addition, the RIFA provides that if certain events occur, including certain bankruptcy events, failure to make payments, a change of control, an out-license or sale of all of the rights in and to BXCL501 in the U.S., in each case except a permitted licensing transaction (as defined in the RIFA) and, subject to applicable cure periods, material breach of the covenants in the RIFA, Oaktree, at the direction of the Purchasers, may require us to repurchase the Purchasers' interests in the RIFA payments and Tail Royalty Payments at the Put/Call Price. In addition, we may terminate the RIFA if a change of control occurs before the first RIFA funding is made.

BTI Warrants and Rights to Purchase Our Common Stock

In connection with the Credit Agreement, on the Effective Date, we granted warrants to the Lenders to purchase up to 278 shares of our common stock (the "Warrants") at an exercise price of $20.04 per share, which represents the arithmetic average of the volume-weighted average price of our common stock on the Nasdaq Capital Market during the 30 trading days preceding the issuance of the Warrants. The Warrants will expire on April 19, 2029, and may be net exercised at the holder's election. In addition, pursuant to the Credit Agreement, the Lenders have the right to purchase shares of our common stock after the Effective Date, for a purchase price of $5,000 at a price per share equal to a 10% premium to the volume weighted average price of the common stock over the 30 trading days prior to the Lenders' election to proceed with such equity investment. We entered into a registration rights agreement with the Lenders, pursuant to which we agreed to file a registration statement on Form S-3 to register the shares issuable upon exercise of the Warrants and, if issued, the shares related to the equity investment, for resale.

OnkosXcel Warrants

In connection with the closing of the Credit Agreement, OnkosXcel granted the Lenders warrants to purchase an ownership interest equal to 0.875% of its fully diluted capitalization as of the closing of the Credit Agreement, subject to increase to up to an aggregate of 1.75% of its fully diluted capitalization as of the closing of the Credit Agreement based on the funding of the two delayed draw tranches of loans provided for under the Credit Agreement (the "701 Warrants"). The exercise price per unit of the 701 Warrants will be set upon the earlier of the closing of the next sale (or related series of related sales) by OnkosXcel of equity securities of OnkosXcel with aggregate proceeds of not less than $20,000 to unrelated third parties (the "Next Equity Financing") at an exercise price per unit equal to a 10% premium over the price per unit of the equity securities sold by OnkosXcel in such Next Equity Financing or, in the event of a sale of OnkosXcel prior to the Next Equity Financing or an initial public offering constituting the Next Equity Financing, the lesser of (x) 75% of the fair market value of the consideration to be paid for a unit upon the consummation of such transaction and (y) 150% of the valuation applicable to the initial profits units issued by OnkosXcel after the closing of the Credit Agreement. The 701 Warrants will expire on April 19, 2029. The 701 Warrants may be net exercised at the holder's election.

Other Contractual Obligations and Commitments

In February 2022, the Company executed a statement of work (the "February 2022 SOW") with Innovative Clinical Research, Inc. ("ICR") under a Master Clinical Trial Agreement that BTI has with ICR. As part of the February 2022 SOW, the Company will pay ICR a minimum of $8,250 to conduct a study of BXCL501 for the potential treatment of agitation in patients with Alzheimer's disease.

As discussed above, we signed the Distribution Agreement with Cardinal in February 2022, whereby Cardinal agreed to distribute product related to BXCL501 in the U.S. Cardinal will be paid defined fees for its services under the Distribution Agreement, which can be terminated by either party for cause. The Distribution Agreement can also be terminated by BTI without cause, subject to payment of agreed termination fees.

Subsequent to March 31, 2022, we entered into the Second Amendment to the Second Amended and Restated Separation and Shared Services Agreement pursuant to which the parties agreed to extend the Company's option to enter into a Collaborative Services Agreement with BioXcel LLC through December 31, 2024, for which the Company has agreed to pay BioXcel LLC $18 per month, prorated for any partial month, as applicable, for the period beginning March 13, 2023 and ending December 31, 2024. In addition, we and BioXcel LLC, a significant stockholder of the Company, entered into the BioXcel Trademark License Agreement, pursuant to which BioXcel LLC has granted us a royalty-free license to use the BIOXCEL trademark in connection with marketing, promoting and selling any products and services in the field of neuroscience, for which we have agreed to pay BioXcel LLC a one-time fee of $135.

In addition, on April 1, 2022, the Company signed a commercial supply agreement with ARx, LLC ("ARx"), whereby ARx agreed to manufacture, and supply Dex product related to IGALMI™ and BXCL501. The commercial supply agreement contemplates specified minimum annual payments, which increase in intervals at specified points in time during the term of the agreement.

Critical Accounting Policies and Estimates

Our critical accounting policies and estimates are described in "Management's Discussion and Analysis of Financial Condition and Results of Operations-Critical Accounting Policies and Estimates" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2021. We have reviewed and determined that those critical accounting policies and estimates remain our critical accounting policies and estimates as of and for the three months ended March 31, 2022. No changes were made to our critical accounting policies during the period presented.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk. As of March 31, 2022, we had $200,435 of cash and cash equivalents. Our cash and cash equivalents are primarily held in U.S. Government money market funds. 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 three months ended March 31, 2022 and 2021, respectively. The loans under the Credit Agreement bear interest at a fixed annual rate of 10.25%, payable quarterly, and consequently we do not have material interest rate exposure due to our indebtedness.

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 do 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.

Capital Market Risk. We currently have no product revenues and depend on funds raised through other sources. One source of funding includes future debt or equity offerings. Our ability to raise funds in this manner depends upon, among other things, capital market forces affecting our stock price, and on the state of the capital markets generally.

Item 4. 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 March 31, 2022. 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 (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 rules and forms of the Securities and Exchange Commission (the "SEC"). 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 March 31, 2022, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

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 March 31, 2022 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may be subject to litigation and claims arising in the ordinary course of business. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have a material adverse effect on our business, operating results, cash flows or financial condition.

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 Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the fiscal year ended December 31, 2021. 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 only one product approved for commercial sale, and have limited experience in obtaining marketing approvals, manufacturing products on a commercial scale, and conducting 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 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 are transitioning from a company with primarily a research and development focus to a company also 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 $31.5 million and $26.4 million for the three months ended March 31, 2022 and 2021, respectively. As of March 31, 2022, we had stockholders' equity of $194 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We have only had one product candidate recently approved for marketing in the U.S., none in any other jurisdiction, and may never receive approval beyond the one product approved to date. It could be several years, if ever, before we have a commercialized product that generates significant revenues through sales of IGALMI™ or our product candidates, if approved. 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;
- fully develop a sales, marketing, and distribution infrastructure to commercialize IGALMI™ and any other product candidates 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 products or 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 IGALMI™ and 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.

Although we have obtained FDA approval for IGALMI™, 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 additional product candidates. If we are required by the FDA, or other regulatory authorities such as the 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.

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 our current and any future product candidates; seek to identify and develop additional product candidates; acquire or in-license other product candidates or technologies; seek regulatory approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure to support the commercialization of any products for which we may obtain marketing approval; 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 cash and cash equivalents as of March 31, 2022 will be sufficient to fund our ongoing research and development efforts and commercialization preparation for at least twelve months from the date of the issuance of the financial statements included in this Quarterly Report on Form 10-Q. We will be required to expend significant funds to commercialize IGALMI™ in the U.S. and advance the development of BXCL501, BXCL701, BXCL502 and our other product candidates. In addition, while we may seek one or more collaborators for future development of our current product candidates 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 prior equity offerings 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. Additionally, market volatility resulting from the COVID-19 pandemic or other factors could also adversely impact our ability to access capital as and when needed. 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 our 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 IGALMI™ and 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 and establish a commercial infrastructure;

- revenue received from commercial sales, if any, of IGALMI™ and 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.

We have significant indebtedness and other contractual obligations that could impair our liquidity, restrict our ability to do business and thereby harm our business, results of operations and financial condition. We may not have sufficient cash flow from operations to satisfy our obligations under our Credit Agreement.

As of April 30, 2022, we had indebtedness of $70 million outstanding under the Credit Agreement with the Lenders, pursuant to which the Lenders loaned $70 million and agreed to loan us up to an additional $65 million in senior secured term loans, and under the RIFA, we may obtain up to $120 million in additional funding. Under the RIFA, we are required to make tiered revenue interest payments on U.S. net sales of IGALMI™ and any other future BXCL501 products equal to a royalty ranging from 0.375% to 7.750% of net sales of IGALMI™ and any other future BXCL501 products in the U.S, as well as certain additional payments to the Purchasers from time to time, to ensure that the aggregate amount of payments received by the Purchasers under the RIFA is at least equal to certain agreed upon minimum levels as of certain specified dates, subject to terms and conditions set forth in the RIFA.

Our ability to make scheduled payments or to refinance these and other outstanding debt obligations depends on our financial and operating performance, which will be affected by prevailing economic, industry and competitive conditions and by financial, business and other factors beyond our control. A failure to pay our debt, fixed cost and other obligations or a breach of our contractual obligations could result in a variety of adverse consequences, including the exercise of remedies by our creditors and lessors. In such a situation, it is unlikely that we would be able to cure our breach, fulfill our obligations, make required payments or otherwise cover our fixed costs, which would have a material adverse effect on our business, results of operations and financial condition.

In addition, historically we have relied on debt and equity financings as our primary sources of liquidity. If our future cash flows and capital resources are insufficient to fund our debt service obligations, we may be forced to reduce or delay capital expenditures, sell assets, seek additional capital or seek to restructure or refinance our indebtedness. Any refinancing of our indebtedness could be at higher interest rates and may require us to comply with more onerous covenants. These alternative measures may not be successful and may not permit us to meet our scheduled debt service obligations. In the absence of such cash flows and resources, we could face substantial liquidity problems and might be required to sell material assets or operations to attempt to meet our debt service obligations. If we cannot meet our debt service obligations, the holders of our indebtedness may accelerate such indebtedness and, to the extent such indebtedness is secured, foreclose on our assets. In such an event, we may not have sufficient assets to repay all of our indebtedness.

In addition, incurring indebtedness generally requires that a portion of cash flow from operating activities be dedicated to interest and principal payments. Debt service requirements could reduce our ability to use our cash flow to fund operations and capital expenditures, to capitalize on future business opportunities, including additional acquisitions, or to pay dividends or increase dividends. In addition, our indebtedness may reduce our flexibility to operate our business, adjust to changing business conditions, restrict us from making strategic acquisitions or cause

us to make non-strategic divestitures or obtain additional financing. Any of these risks could materially adversely affect our business, results of operations or financial condition.

Restrictive covenants in our Credit Agreement and RIFA each place limits on our ability to conduct our business. The Credit Agreement contains customary representations and warranties and customary affirmative and negative covenants, including, among other things, restrictions on indebtedness, liens, investments, mergers, dispositions, prepayment of other indebtedness, and dividends and other distributions, subject to certain exceptions, including specific exceptions with respect to product commercialization and development activities. We must also comply with certain financial covenants under the Credit Agreement that requires we maintain a minimum level of cash and revenues. The RIFA contains customary representations and warranties and certain restrictions on our ability to incur indebtedness and grant liens on intellectual property related to BXCL501. In addition, the RIFA provides that if certain events occur, including certain bankruptcy events, failure to make payments, a change of control, an out-license or sale of all of the rights in and to BXCL501 in the U.S., in each case except a permitted licensing transaction (as defined in the RIFA) and, subject to applicable cure periods, material breach of the covenants in the RIFA, Oaktree, at the direction of the Purchasers, may require us to repurchase certain of the Purchasers' interests.

Risks Related to the Discovery and Development of Product Candidates

We have limited experience in drug discovery and drug development.

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 acquired our product candidates from to have conducted research and development in accordance with the applicable protocol, legal, regulatory and scientific standards, accurately reported the results of all clinical trials conducted prior to our acquisition of the applicable product candidate, and correctly collected and interpreted the data from these studies and trials. To the extent any of these activities did not occur, our expected development time and costs could increase, 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 IGALMI™, and three of our product candidates, BXCL501, BXCL701 and BXCL502. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize IGALMI™ 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 have only one product that recently received regulatory approval and may never be able to develop additional marketable product candidates. We are continuing to invest a significant portion of our efforts and financial resources in the commercialization of BXCL501 and development of BXCL701 and BXCL502, as well as 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 IGALMI™, and of BXCL501, BXCL701, BXCL502 and our other product candidates will depend on several factors, including the following:

- acceptance of an IND application by the FDA or acceptance of comparable applications by foreign regulatory authorities allowing us to conduct clinical trials of our product candidates in the U.S. or in foreign jurisdictions;
- 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 U.S. 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 commercialize IGALMI™ or 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 because 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. The results and related findings and conclusions based on such preliminary data are subject to change, and have in the past changed, 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 recently obtained regulatory approval for our first product candidate for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder, which has not yet been successfully commercialized. It is possible that none of our product candidates, or any product candidates we may seek to develop in the future, will ever obtain regulatory approval.

Our current product candidates, or any that may be developed in the future, 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 NDA or other submission or to obtain regulatory approval in the U.S. 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 comparable 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 only submitted one NDA to the FDA and have not submitted any similar marketing applications to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates currently in development, or any than may be developed in the future, will be successful in clinical trials or receive regulatory approval. Further, our product candidates currently in development, or any than may be developed in the future, may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for additional 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 product candidates, if approved.

We plan to seek regulatory approval to commercialize our product candidates in the U.S., the European Union (“EU”) 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.

In addition, the FDA’s and other regulatory authorities’ policies with respect to clinical trials may change and additional government regulations may be enacted. For instance, the regulatory landscape related to clinical trials in the EU recently evolved. The EU Clinical Trials Regulation (“CTR”), which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. While the Clinical Trials Directive required a separate clinical trial application (“CTA”), to be submitted in each member state, to both the competent national health authority and an independent ethics committee, the CTR introduces a centralized process and only requires the submission of a single application to all member states concerned. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state. The assessment procedure of the CTA has been harmonized as well, including a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state’s decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed. The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. For clinical trials whose CTA was made under the Clinical Trials Directive before January 31, 2022, the Clinical Trials Directive will continue to apply on a transitional basis for three years. Additionally, sponsors may still choose to submit a CTA under either the Clinical Trials Directive or the CTR until January 31, 2023 and, if authorized, those will be

governed by the Clinical Trials Directive until January 31, 2025. By that date, all ongoing trials will become subject to the provisions of the CTR.

If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

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, BXCL502 and our other product candidates, there can be no assurance that the FDA, or other comparable foreign regulatory authorities, 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 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;
- diversion of healthcare resources to combat epidemics, such as the COVID-19 pandemic;
- obtaining institutional review board ("IRB") approval at each site, or independent ethics committee approval at any sites outside the U.S.;
- 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 ("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 ("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.

In addition, disruptions caused by the COVID-19 pandemic may increase the likelihood that we encounter such difficulties or delays in initiating, enrolling, conducting or completing our planned and ongoing clinical trials. For example, in April 2021, PLACIDITY enrollment was voluntarily paused to assess challenges posed in opening relevant clinical sites and enrolling delirium patients in the ICU settings, including as a result of the burden COVID-19 placed on the ICU.

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 ("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. For example, if the current conflict between Russia and Ukraine spreads to other regions, it may adversely impact our ability to conduct trials.

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. Our ability to enroll patients in our clinical trials may be impacted by governmental restrictions and diversion of healthcare resources resulting from the COVID-19 pandemic. Many pharmaceutical companies are conducting clinical trials in patients with the disease indications that our product candidates are designed to 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, BXCL502 and our other product candidates in patients, in many cases, is ongoing and it is possible that there may be side effects associated with their use. Results of our trials could reveal a high and unacceptable severity and prevalence of these or other side effects. For example, in our Phase 2 clinical trial of BXCL701 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.

If we observe drug-related adverse events or other unacceptable safety concerns in clinical trials, 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 observed in our

clinical trials and upon commercialization of any of our product candidates that may receive regulatory approval. 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 we or others later identify undesirable side effects caused by IGALMI™ or any other product candidate that receives marketing approval, 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 ("REMS") or create a medication guide outlining the risks of such side effects for distribution to patients, or similar risk management measures;
- 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 or product candidate, if approved, and could significantly harm our business, results of operations and prospects.

BioXcel LLC'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 BioXcel LLC's 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 BioXcel LLC's 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 novel. Although we obtained FDA approval for IGALMI™, because our approach is novel, 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.

BioXcel LLC's EvolverAI may fail to help us discover and develop additional potential product candidates.

Any drug discovery that we are conducting using BioXcel LLC's EvolverAI may not be successful in identifying compounds that have commercial value or therapeutic utility. BioXcel LLC's EvolverAI may initially show promise in identifying potential product candidates, yet fail to yield viable additional product candidates for clinical development or potential 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 BioXcel LLC's 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 obtained Fast Track designation for BXCL501 for the acute treatment of mild-to-moderate agitation associated with schizophrenia, bipolar disorder, or dementia, 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 candidate 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. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the applicable FDA review team during product development and, once an NDA is submitted, the product candidate may be eligible for priority review if the relevant criteria are met. A Fast Track product candidate may also be eligible for rolling review, where 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. We obtained Fast Track designation for BXCL501 for the acute treatment of mild-to-moderate agitation associated with schizophrenia, bipolar disorder, or dementia, and we may seek Fast Track designation for other indications 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.

A Breakthrough Therapy designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We obtained Breakthrough Therapy Designations for BXCL501 for the acute treatment of agitation associated with dementia, and we may seek additional Breakthrough Therapy designations for our product candidates if the clinical data support such a designation for one or more product candidates. A Breakthrough Therapy is defined as a drug or biologic

that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Product candidates designated as Breakthrough Therapies by the FDA also receive the benefits associated with Fast Track designation, including the potential for rolling review of an NDA.

Designation as a Breakthrough Therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a Breakthrough Therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under non-expedited FDA review procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, 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.

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. The Hatch-Waxman Act added Section 505(b)(2) to the Federal Food, Drug and Cosmetic Act ("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).

If we are required by the FDA, or similar regulatory authorities, to obtain approval (or clearance, or certification) of a companion diagnostic device in connection with approval of one of our product candidates, and we do not obtain or face delays in obtaining approval (or clearance, or certification) of a companion diagnostic device, we will not be able to commercialize the product candidate and our ability to generate revenue will be materially impaired.

According to FDA guidance, if the FDA determines that a companion diagnostic device is essential to the safe and effective use of a novel therapeutic product or indication, the FDA generally will not approve the therapeutic product or new therapeutic product indication if the companion diagnostic is not also approved or cleared for that indication. If a satisfactory companion diagnostic is not commercially available, we may be required to create or obtain one that would be subject to regulatory approval requirements. For example, we may decide to collaborate with patient diagnostic companies during our clinical trial enrollment process for BXCL701 to help identify patients with tumor gene alterations that we believe may be most likely to respond to treatment with BXCL701. The process of obtaining or creating such diagnostic is time consuming and costly.

Companion diagnostics are developed in conjunction with clinical programs for the associated product and are subject to regulation as medical devices by the FDA and comparable foreign regulatory authorities, and, to date, the FDA has generally required premarket approval of companion diagnostics for cancer therapies. Generally, when a companion diagnostic is essential to the safe and effective use of a therapeutic product, the FDA requires that the companion diagnostic be approved before or concurrent with approval of the therapeutic product and before a product can be commercialized. The approval of a companion diagnostic as part of the therapeutic product's labeling limits the use of the therapeutic product to only those patients who express the specific genetic alteration that the companion diagnostic was developed to detect.

If the FDA, or a comparable foreign regulatory authority, requires approval (or certification or clearance) of a companion diagnostic for any of our product candidates, whether before or after the product candidate obtains marketing approval, we and/or third-party collaborators may encounter difficulties in developing and obtaining approval (or clearance, or certification) for these companion diagnostics. Any delay or failure by us or third-party collaborators to develop or obtain regulatory approval (or clearance, or certification) of a companion diagnostic could delay or prevent approval or continued marketing of our related product candidates. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process for the companion diagnostic or in transferring that process to commercial partners or negotiating insurance reimbursement plans, all of which may prevent us from completing our clinical trials or commercializing our product candidates, if approved, on a timely or profitable basis, if at all.

Approval, clearance or certification of companion diagnostics may be subject to further legislative or regulatory reforms notably in the EU. On May 25, 2017, the new In Vitro Medical Devices Regulation No. 2017/746 ("IVDR") entered into force. The IVDR repeals and replaces the EU In Vitro Diagnostic Medical Devices Directive. Unlike directives, which must be implemented into the national laws of the EU member states, regulations are directly applicable (i.e., without the need for adoption of EU member states laws implementing them) in all EU member states and are intended to eliminate current differences in the regulation of medical devices among EU member states. The IVDR, among other things, is intended to establish a uniform, transparent, predictable and sustainable regulatory framework across the EU for medical devices and ensure a high level of safety and health while supporting innovation. The IVDR becomes effective in May 2022. However, on October 14, 2021, the European Commission proposed a "progressive" roll-out of the IVDR to prevent disruption in the supply of in vitro diagnostic medical devices. The European Parliament and Council adopted the proposed regulation on December 15, 2021. The IVDR will fully apply on May 26, 2022, but there will be a tiered system extending the grace period for many devices (depending on their risk classification) before they have to be fully compliant with the regulation.

The regulation of companion diagnostics in the EU will be subject to further requirements once the IVDR becomes fully effective since the IVDR introduces a new classification system for companion diagnostics, which are now specifically defined as diagnostic tests that support the safe and effective use of a specific medicinal product, by identifying patients that are suitable or unsuitable for treatment. Companion diagnostics will have to undergo a conformity assessment by a notified body. Before it can issue a CE certificate, the notified body must seek a scientific opinion from the EMA on the suitability of the companion diagnostic to the medicinal product concerned if the medicinal product falls exclusively within the scope of the centralized procedure for the authorization of medicines, or the medicinal product is already authorized through the centralized procedure, or a marketing authorization application for the medicinal product has been submitted through the centralized procedure. For other substances, the notified body can seek the opinion from a national competent authority or the EMA.

These modifications may make it more difficult and costly for us to obtain regulatory clearances, approvals or certifications for our companion diagnostics or to manufacture, market or distribute our products after clearance, approval or certification is obtained.

Even though the FDA has approved IGALMI™ for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder, we will still face extensive and ongoing regulatory requirements and obligations for IGALMI™ and for any product candidates for which we obtain approval.

Any regulatory approvals that we may receive for IGALMI™ or any of our product candidates will require the submission of reports to regulatory authorities and surveillance to monitor the safety and efficacy of the product, may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post-approval study or risk management requirements. For example, the FDA-approved label for IGALMI™ includes certain warnings and precautions regarding hypotension, orthostatic hypotension, bradycardia, somnolence, and QT interval prolongation. The FDA may also require a REMS in order to approve a product candidate, which could entail requirements for a medication guide, physician training and communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

In addition, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for IGALMI™ are and will remain subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as on-going compliance with cGMPs, and GCPs for any clinical trials that we conduct post-approval. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA and other regulatory authorities for compliance with cGMP regulations and standards. If we or a regulatory authority discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facilities where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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 policies of the FDA and other regulatory authorities 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. 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 U.S. or abroad.

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

The FDA and other regulatory authorities strictly regulate marketing, labeling, advertising and promotion of prescription drugs. These regulations include standards and restrictions for direct-to-consumer advertising, industry-sponsored scientific and educational activities, promotional activities involving the internet and off-label promotion. Any regulatory approval that the FDA or any other regulatory authority may grant is limited to those specific diseases and indications for which a product is deemed to be safe and effective. For example, the FDA-approved label for IGALMI™ is currently limited to the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults.

While physicians in the U.S. may choose, and are generally permitted, to prescribe drugs for uses that are not described in the product's labeling and for uses that differ from those tested in clinical trials and approved by the regulatory authorities, our ability to promote the products is narrowly limited to those indications that are specifically approved by the FDA. These "off-label" uses are common across medical specialties and may constitute an appropriate treatment for some patients in varied circumstances. For example, other formulations of Dex, the active ingredient in IGALMI™, have been approved for uses beyond those authorized in IGALMI™ approved labeling, such as for use in sedation of surgical patients, and we are continuing to develop BXCL501 for potential use in patients with dementia, MDD, Alzheimer's disease and other indications. We do not market or promote IGALMI™ for these uses.

Regulatory authorities in the U.S. generally do not regulate the behavior of physicians in their choice of treatments. Regulatory authorities do, however, restrict communications by pharmaceutical companies on the subject of off-label use. If we are found to have promoted our products for any off-label uses, the U.S. federal government (and other foreign governments) could levy civil, criminal and/or administrative penalties, and seek fines against us. The FDA, or other regulatory authorities, could also require 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 IGALMI™ or our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA and foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory and policy changes, the FDA's or foreign regulatory authorities' ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's or foreign regulatory authorities' ability to perform routine functions. Average review times at the FDA and foreign regulatory authorities 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, such as the EMA, following its relocation to Amsterdam and corresponding staff changes, may also slow the time necessary for new drug or modifications to approved drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the COVID-19 pandemic, in March 2020, the FDA announced its intention to postpone most inspections of foreign manufacturing facilities, and on March 18, 2020, the FDA temporarily postponed routine surveillance inspections of domestic manufacturing facilities. Subsequently, in July 2020, the FDA resumed certain on-site inspections of domestic manufacturing facilities subject to a risk-based prioritization system. The FDA utilized this risk- based assessment system to assist in determining when and where it was safest to conduct prioritized domestic inspections. Additionally, on April 15, 2021, the FDA issued a guidance document in which the FDA described its plans to conduct voluntary remote interactive evaluations of certain drug manufacturing facilities and clinical research sites, among other facilities. According to the guidance, the FDA may request such remote interactive evaluations where the FDA determines that remote evaluation would be appropriate based on mission needs and travel limitations. In May 2021, the FDA outlined a detailed plan to move toward a more consistent state of inspectional operations, and in July 2021, the FDA generally resumed standard inspectional operations of domestic facilities. More recently, the FDA has continued to monitor and implement changes to its inspectional activities to ensure the safety of its employees and those of the firms it regulates as it adapts to the evolving COVID-19 pandemic. Regulatory authorities outside the U.S. have adopted similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We may conduct certain of or portions of our clinical trials for our product candidates outside of the U.S. and the FDA may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We may choose to conduct one or more of our clinical trials or a portion of our clinical trials for our product candidates outside the U.S. The acceptance of study data from clinical trials conducted outside the U.S. or another jurisdiction by the FDA or comparable foreign regulatory authority may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the U.S., the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the U.S. or the applicable jurisdiction. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction.

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

In addition to regulations in the U.S., should we or our collaborators pursue marketing approvals for IGALMI™, and for BXCL501, BXCL701, BXCL502 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 IGALMI™, and for BXCL501, BXCL701, BXCL502 and our other product candidates in Europe and other jurisdictions outside the U.S. 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 U.S. And, regulatory 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 ("UK"), the UK formally withdrew from the EU on January 31, 2020 and ratified a trade and cooperation agreement governing its future relationship (commonly referred to as "Brexit"). The agreement, which was applied provisionally from January 1, 2021 and entered into force on May 1, 2021, addresses trade, economic arrangements, law enforcement, judicial cooperation and a governance framework including procedures for dispute resolution, among other things. Because the agreement merely sets forth a framework in many respects and will require complex additional bilateral negotiations between the UK and the EU as both parties continue to work on the rules for implementation, significant political and economic uncertainty remains about how the precise terms of the relationship between the parties will differ from the terms before withdrawal.

Since January 1, 2021, the UK operates under a distinct regulatory regime to the EU. EU pharmaceutical laws only apply in respect of the UK to Northern Ireland (as set out in the Protocol on Ireland/Northern Ireland). EU . laws which have been transposed into UK law through secondary legislation continue to be applicable as "retained EU law". While the UK has indicated a general intention that new laws regarding the development, manufacture and commercialization of medicinal products in the UK will align closely with EU law, there are limited detailed proposals for future regulation of medicinal products. The trade and cooperation agreement includes specific provisions concerning medicinal products, which include the mutual recognition of cGMP, inspections of manufacturing facilities for medicinal products and cGMP documents issued (such mutual recognition can be rejected by either party in certain circumstances) but does not foresee wholesale mutual recognition of UK and EU pharmaceutical regulations. For example, it is not clear to what extent the UK will adopt legislation aligned with, or similar to, the EU CTR which became applicable on January 31, 2022 and which significantly reforms the assessment and supervision processes for clinical trials throughout the EU. On January 17, 2022, the UK Medicines and Healthcare products Regulatory Agency ("MHRA") launched an eight-week consultation on reframing the UK legislation for clinical trials. The consultation closed on 14 March 2022 and aims to streamline clinical trials approvals, enable innovation, enhance clinical trials transparency, enable greater risk proportionality, and promote patient and public involvement in clinical trials. The outcome of the consultation will be closely watched and will determine whether the UK chooses to align with the regulation or diverge from it to maintain regulatory flexibility. A decision by the UK not to closely align its regulations with the new approach that will be adopted in the EU may have an effect on the cost of conducting clinical trials in the UK as opposed to other countries and/or make it harder to seek a marketing authorization in the EU for our product candidates on the basis of clinical trials conducted in the UK.

Therefore, there remains political and economic uncertainty regarding to what extent the regulation of medicinal products will differ between the UK and the EU in the future. Any divergences will increase the cost and complexity of running our business, including with respect to the conduct of clinical trials. Brexit also materially impacted the regulatory regime with respect to the approval of our product candidates. Great Britain is no longer covered by the EU's procedures for the grant of marketing authorizations (Northern Ireland is covered by the centralized authorization procedure and can be covered under the decentralized or mutual recognition procedures). As of 1 January 2021, all existing centralized marketing authorizations were automatically converted into UK marketing authorizations effective in Great Britain and issued with a UK marketing authorization number on January 1, 2021 (unless marketing authorization holders opted out of this scheme). A separate marketing authorization is now required to market drugs in Great Britain. It is currently unclear whether the regulator in the UK, the MHRA, is sufficiently prepared to handle the increased volume of marketing authorization applications that it is likely to receive. Any delay in obtaining, or an inability to obtain, any regulatory approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in Great Britain 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 Great Britain for our product candidates, which could significantly and materially harm our business. The UK's withdrawal from the EU and the associated uncertainty has had and may continue to have a significant adverse effect on global

economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and restrict the ability of key market participants to operate in certain financial markets. Asset valuations, currency exchange rates and credit ratings may be especially subject to increased market volatility. Any of these factors could have a significant adverse effect on our business, financial condition, results of operations and prospects.

Further, the UK's withdrawal from the EU has resulted in the relocation of the EMA from the UK to the Netherlands. This relocation has caused, and may continue to cause, disruption in the administrative and medical scientific links between the EMA and the MHRA, including delays in granting clinical trial authorization or marketing authorization, disruption of importation and export of active substance and other components of new drug formulations, and disruption of the supply chain for clinical trial product and final authorized formulations. The cumulative effects of the disruption to the regulatory framework may add considerably to the development lead time to marketing authorization and commercialization of products in the EU and/or the UK.

If we are found in violation of federal, state or foreign healthcare "fraud and abuse" laws, we may be required to pay significant fines and penalties, including, without limitation, debarment, suspension or exclusion from participation in federal, state or similar healthcare programs, which may adversely affect our business, financial condition and results of operations.

In the U.S., we are subject to various federal and state healthcare "fraud and abuse" laws, including anti- kickback laws, false claims laws and other laws intended to reduce fraud and abuse in federal and state healthcare programs, which could affect us, and our ability to successfully commercialize our products in the U.S. We may have to comply with similar laws and regulations outside the U.S. 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 healthcare 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;

- 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 healthcare 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 ("HIPAA") prohibits persons or entities from knowingly and willfully executing a scheme to defraud any healthcare 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 healthcare 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;

- the federal physician sunshine requirements under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act ("ACA"), which requires certain manufacturers of drugs, devices, biologics, and medical supplies to report annually to the U.S. Centers for Medicare & Medicaid Services information related to payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain non-physician practitioners (physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists, anesthesiologist assistants, and certified nurse midwives), and teaching hospitals, and ownership and investment interests held by physicians 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; and 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

- 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 or foreign 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, 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 IGALMI™, and for any of our other product candidates that may receive regulatory approval, 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 or product candidates 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 healthcare 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 obtained Orphan Drug Designation for BXCL701 for the treatment of pancreatic cancer, melanoma, acute myeloid leukemia and soft tissue sarcoma and we 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 U.S. and EU, 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 U.S., or a patient population greater than 200,000 individuals in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, orphan drug designation is granted by the European Commission based on a scientific opinion of the EMA's Committee for Orphan Medicinal Products. A medicinal product may be designated as orphan if its sponsor can establish that (i) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects no more than 5 in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (iii) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the medicinal product will be of significant benefit to those affected by the condition. The application for orphan designation must be submitted before the application for marketing authorization.

In the U.S., 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 or condition for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the U.S. provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same disease or condition for seven years. In limited circumstances, the applicable exclusivity period is ten years in the EU. The EU exclusivity period can be reduced to six years if, at the end of the fifth year, it is established that 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 January 2021, the FDA granted Orphan Drug Designation to BXCL701 for the treatment of soft tissue sarcoma. 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 BXCL701 for the treatment of pancreatic cancer and melanoma. We may seek Orphan Drug Designations for BXCL701 in other diseases or conditions 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 disease or condition before we do. If that were to happen, our applications for that disease or condition may not be approved until the competing company's period of exclusivity expires. In addition, exclusive

marketing rights in the U.S. and abroad may be limited if we seek approval for an indication broader than the orphan-designated disease or condition or may be lost if the FDA or foreign regulatory authorities 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 ingredients may be approved for the same disease or condition. Even after an orphan drug is approved, the FDA or foreign regulatory authorities can subsequently approve the same drug with the same active ingredient for the same condition if the FDA or foreign regulatory authorities conclude 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 or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process, and does not 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 IGALMI™ or any product candidate for which we may obtain regulatory approval.

We have limited experience in marketing and selling drug products. We have not entered into arrangements for the sale and marketing of IGALMI™ or BXCL501, BXCL701, BXCL502 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 the 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 thereof, to market our products will be expensive and time-consuming and could delay any product launch. Further, we can give no assurances that we will 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 U.S., the EU 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 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.

Although we obtained FDA approval for IGALMI™, our products and product candidates may not be accepted by physicians or the medical community in general.

There can be no assurance that IGALMI™, or BXCL501, BXCL701, BXCL502 and our other product candidates or any other product candidate successfully developed by us, independently or with partners, if aapproved, will be accepted by physicians, hospitals and other healthcare facilities. IGALMI™ competes, and BXCL501, BXCL701, BXCL502 and any future product candidates we develop will compete, with a number of products manufactured and marketed by major pharmaceutical and biotechnology companies. The degree of market acceptance of IGALMI™ and any drugs we develop depends on a number of factors, including:

- our demonstration of the clinical efficacy and safety of our products and product candidates;
- timing of market approval and commercial launch of our products and product candidates;
- the clinical indication(s) for which our products and product candidates are approved;
- product label and package insert requirements;
- advantages and disadvantages of our products and 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 healthcare laws, regulations, and medical policies; and
- availability of coverage and reimbursement in select jurisdictions, and future changes to coverage and 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 U.S. and other countries, sales of IGALMI™ and any other products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations.

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. IGALMI™ and any other products for which we receive regulatory approval 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, state and foreign government proposals and healthcare reforms are likely which could limit the prices that can be charged for IGALMI™ and 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 in the U.S., by the possible effect of such current or future legislation on amounts that private insurers will pay and by other healthcare reforms that may be enacted or adopted in the future.

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 U.S. 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 healthcare services to contain or reduce healthcare 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 U.S., new healthcare legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably.

For example, in the U.S., 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 contained 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 imposed 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 existed in the Medicare Part D prescription drug program, manufacturers are now required to provide a discount on branded prescription drugs equal to 70% of the government-negotiated price, for drugs provided to certain beneficiaries who fall within the "donut hole." Similarly, the ACA increased the level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1% of the average manufacturer price and required collection of rebates for drugs paid by Medicaid managed care organizations. The ACA also included 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.

Since its enactment, there have been judicial, executive and Congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, President Biden issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace from February 15, 2021 through August 15, 2021. The executive order instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA.

In addition, other legislative changes have been proposed and adopted in the U.S. 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 2030, except for a temporary suspension from May 1, 2020 through March 31, 2022, and a 1% reduction from April 1, 2022 through June 30, 2022, unless additional Congressional action is taken. And, the American Taxpayer Relief Act of 2012, 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. More recently, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, beginning January 1, 2024.

The cost of prescription pharmaceuticals in the U.S. has also been the subject of considerable discussion. There have been several Congressional inquiries, as well as legislative and regulatory initiatives and executive orders designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. Members of Congress and the Biden Administration have indicated they will continue to pursue legislative or administrative measures to control prescription drug costs, although the likelihood of such measures being adopted remains uncertain. For example, the Build Back Better Act, if enacted, would introduce substantial drug pricing reforms, including the establishment of a drug price negotiation program within the U.S. Department of Health and Human Services that would require manufacturers to charge a negotiated "maximum fair price" for certain selected drugs or pay an excise tax for noncompliance, and the establishment of rebate payment requirements on manufacturers of certain drugs payable under Medicare Parts B and D. If the Build Back Better Act is not enacted, similar or other drug pricing proposals could appear in future legislation. We cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

Individual states in the U.S. 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, are 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 U.S. 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 governments, 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.

If we fail to comply with reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the United States, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, results of operations and financial condition.

Upon marketing IGALMI™, we expect to participate in the Medicaid Drug Rebate Program ("MDRP") and other federal and state government pricing programs in the U.S., and we may participate in additional government pricing programs in the future. These programs generally require manufacturers to pay rebates or otherwise provide discounts to government payors in connection with drugs that are dispensed to beneficiaries of these programs. As a condition of having federal funds being made available for covered outpatient drugs under Medicaid and Medicare Part B, a manufacturer must enroll in the MDRP. Under this program, the manufacturer must pay a rebate to state Medicaid programs for each unit of a covered outpatient drug dispensed to a Medicaid beneficiary and paid for by a state Medicaid program. Medicaid drug rebates are based on pricing data that the manufacturer must report on a monthly and quarterly basis to the U.S. Centers for Medicare & Medicaid Services ("CMS"), the federal agency that administers the

MDRP and Medicare programs. For the MDRP, these data include the average manufacturer price ("AMP") for each drug and, in the case of an innovator product, the best price ("BP"). If a manufacturer becomes aware that its MDRP price reporting submission for a prior period was incorrect or has changed as a result of recalculation of the pricing data, the manufacturer must resubmit the corrected data for up to three years after those data originally were due. In addition, there is increased focus by the Office of Inspector General within the U.S. Department of Health and Human Services on the methodologies used by manufacturers to calculate AMP, and BP, to assess manufacturer compliance with MDRP reporting requirements. If a manufacturer fails to provide information timely or is found to have knowingly submitted false information to the government, the manufacturer may be subject to civil monetary penalties and other sanctions, including termination from the MDRP, which would result in payment not being available for its covered outpatient drugs under Medicaid or, if applicable, Medicare Part B. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against a manufacturer under the Federal False Claims Act and other laws and regulations.

Federal law requires that a manufacturer that participates in the MDRP also participate in the Public Health Service's 340B drug pricing program (the "340B program") in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B program is administered by the Health Resources and Services Administration ("HRSA") and requires a participating manufacturer to charge statutorily defined covered entities no more than the 340B "ceiling price" for its covered outpatient drugs used in an outpatient setting. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients. The 340B ceiling price is calculated using a statutory formula, which is based on the AMP and rebate amount for the covered outpatient drug as calculated under the MDRP. In general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. Manufacturers must report 340B ceiling prices to HRSA on a quarterly basis, and HRSA publishes them to 340B covered entities. HRSA has finalized regulations regarding the calculation of the 340B ceiling price and the imposition of civil monetary penalties on manufacturers that knowingly and intentionally overcharge covered entities for 340B-eligible drugs. HRSA has also finalized an administrative dispute resolution process through which 340B covered entities may pursue claims against participating manufacturers for overcharges, and through which manufacturers may pursue claims against 340B covered entities for engaging in unlawful diversion or duplicate discounting of 340B drugs. In addition, legislation may be introduced that, if passed, would further expand the 340B program, such as adding further covered entities or requiring participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

In order to be eligible to have drug products paid for with federal funds under Medicaid and Medicare Part B and purchased by certain federal agencies and grantees, a manufacturer also must participate in the U.S. Department of Veterans Affairs ("VA") Federal Supply Schedule ("FSS") pricing program. Under the VA/FSS program, the manufacturer must report the Non-Federal Average Manufacturer Price ("Non-FAMP") for its covered drugs to the VA and charge certain federal agencies no more than the Federal Ceiling Price, which is calculated based on Non-FAMP using a statutory formula. These four agencies are the VA, the U.S. Department of Defense, the U.S. Coast Guard, and the U.S. Public Health Service (including the Indian Health Service). The manufacturer must also pay rebates on products purchased by military personnel and dependents through the TRICARE retail pharmacy program. If a manufacturer fails to provide timely information or is found to have knowingly submitted false information, the manufacturer may be subject to civil monetary penalties.

Individual states continue to consider and have enacted legislation to limit the growth of healthcare costs, including the cost of prescription drugs and combination products. A number of states have either implemented or are considering implementation of drug price transparency legislation that may prevent or limit our ability to take price increases at certain rates or frequencies. Requirements under such laws include advance notice of planned price increases, reporting price increase amounts and factors considered in taking such increases, wholesale acquisition cost information disclosure to prescribers, purchasers, and state agencies, and new product notice and reporting. Such legislation could limit the price or payment for certain drugs, and a number of states are authorized to impose civil monetary penalties or pursue other enforcement mechanisms against manufacturers who fail to comply with drug price transparency requirements, including the untimely, inaccurate, or incomplete reporting of drug pricing information. If we are found to have violated state law requirements, we may become subject to penalties or other enforcement mechanisms, which could have a material adverse effect on our business.

Pricing and rebate calculations are complex, vary among products and programs, and are often subject to interpretation by manufacturers, governmental or regulatory agencies, and the courts. The terms, scope and complexity of these government pricing programs change frequently, as do interpretations of applicable requirements for pricing and rebate calculations. Responding to current and future changes may increase our costs and the complexity of compliance will be time consuming. Any required refunds to the U.S. government or responding to a government investigation or enforcement action would be expensive and time consuming and could have a material adverse effect on our business, results of operations and financial condition. Price recalculations under the MDRP also may affect the ceiling price at which we may be required to offer products under the 340B program. Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we fail to submit required price data on a timely basis, or if we are found to have charged 340B covered entities more than the statutorily mandated ceiling price. In the event that CMS were to terminate a manufacturer's Medicaid rebate agreement, no federal payments would be available under Medicaid or Medicare for its covered outpatient drugs. We cannot assure you that submissions we make will not be found to be incomplete or incorrect.

Risks Related to Our Relationship with BioXcel LLC

BioXcel LLC 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 March 31, 2022, BioXcel LLC owned approximately 31% of the economic interest and voting power of our outstanding common stock. Drs. Vimal Mehta and Krishnan Nandabalan are the co-founders and serve as senior executives and members of the board of BioXcel LLC. See "The management of and beneficial ownership in BioXcel LLC by our executive officers and our directors may create, or may create the appearance of, conflicts of interest." below. Even though BioXcel LLC 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 LLC does not preclude the possibility of stockholder litigation, including but not limited to derivative litigation nominally against BioXcel LLC and against its directors and officers and also against us and our directors and officers.

The commercial terms of the Separation and Shared Services Agreement (the "Services Agreement") and the Amended and Restated Asset Contribution Agreement (the "Contribution Agreement") that we entered into with BioXcel LLC have not been negotiated on behalf of BioXcel LLC by persons consisting solely of disinterested BioXcel LLC directors.

No assurance can be given that any stockholder of BioXcel LLC will not claim in a lawsuit that such terms in fact are not in the best interests of BioXcel LLC and its stockholders, that the directors and officers of BioXcel LLC breached their fiduciary duties in connection with such agreements and that any disclosures by BioXcel LLC to its stockholders regarding these agreements and the relationship between BioXcel LLC 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 LLC 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 LLC to provide us with certain services for our business.

We rely, in part, on BioXcel LLC and access to its EvolverAI, a research and development engine created and owned by BioXcel LLC, to identify, research and develop potential product candidates in neuroscience and immuno-oncology. We negotiated the Services Agreement with BioXcel LLC pursuant to which BioXcel LLC shall perform product identification and related services for us utilizing its EvolverAI. Under the Services Agreement, we have an option, exercisable until December 31, 2024, to enter into a collaborative services agreement with BioXcel LLC

pursuant to which BioXcel LLC shall perform product identification and related services for us utilizing its 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 LLC reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestone payments shall not exceed $10 million in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestone payments 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 LLC shall continue to make such product identification and related services available to us until at least December 31, 2024. In addition, BioXcel LLC 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 LLC may identify on its own and not in connection with BioXcel LLC's provision of services to us under the Services Agreement. This first right to negotiate is valid for a period of five years from the date of our IPO. If our rights and access to BioXcel LLC's collaborative services and to its EvolverAI were to become limited, terminated, or if we were otherwise precluded from conducting research and development using its EvolverAI, or if BioXcel LLC 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 LLC, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. We also cannot ensure that BioXcel LLC retains sufficient resources or personnel or otherwise to conduct its operations. BioXcel LLC 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 LLC.

The management of and beneficial ownership in BioXcel LLC by our executive officers and our directors may create, or may create the appearance of, conflicts of interest.

The management of and beneficial ownership in BioXcel LLC by our executive officers and our directors may create, or may create the appearance of, conflicts of interest. For example, each of our Chief Executive Officer and a director on our Board, Vimal Mehta, Ph.D., and our Chief Digital Officer and a director on our Board, Krishnan Nandabalan, Ph.D., is a manager of BioXcel LLC, as well as a director, officer and stockholder of BioXcel LLC, BTI's former parent company. Additionally, as of March 31, 2022, Dr. Mehta and Dr. Nandabalan, through their beneficial ownership of BioXcel LLC, beneficially owned approximately 32% of the Company. Management and ownership by our executive officers and directors in BioXcel LLC, creates, or, may create the appearance of, conflicts of interest when these individuals are faced with decisions that could have different implications for BioXcel LLC 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. 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 LLC with respect to our past and ongoing relationships could harm our business operations.

Disputes may arise between BioXcel LLC 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 LLC and us;
- labor, tax, employee benefit, indemnification and other matters arising from the separation of BTI from BioXcel LLC;
- distribution and supply obligations;

- employee retention and recruiting;
- business combinations involving us;
- sales or distributions by BioXcel LLC of all or any portion of its ownership interest in us;
- the nature, quality and pricing of services BioXcel LLC has agreed to provide us; and
- business opportunities that may be attractive to both BioXcel LLC and us.

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

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

BioXcel LLC 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 LLC 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, biotechnology, 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 their 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 LLC will be able to develop, acquire or integrate new technologies, that these new technologies will meet our and BioXcel LLC'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 BioXcel LLC's EvolverAI obsolete. BioXcel LLC'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 LLC 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 our commercial supplies of IGALMI™, and we intend to rely on third parties to produce commercial supplies of any other 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.

We entered into a commercial supply agreement with ARx pursuant to which ARx agreed to exclusively manufacture and supply us with all of our worldwide demand of thin film formulation of Dex to be used for the commercial supply of IGALMI™ and for ongoing clinical trials of our product candidate BXCL501, subject to certain alternative supply provisions. If ARx is unable to produce our supply of dexmedetomidine, our business would be harmed because there can be no assurance that we will be able to identify or enter into agreements with alternative suppliers on a timely basis on acceptable terms, if at all. An interruption in our ability to sell our products to customers could occur if we encounter delays or difficulties in securing dexmedetomidine, or if the quality supplied does not meet our specifications, or if we cannot then obtain an acceptable substitute. If any of these events occur, our business and

operating results could be harmed. Our specified minimum annual payment could adversely affect our cash flows, such as in times when we have sufficient inventory and would otherwise be able to use our cash for other purposes.

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 or similar foreign requirements. Drug manufacturing facilities are subject to inspection before the FDA or foreign regulatory authorities will issue an approval to market a new drug product, and ARx and any other manufacturers that we may use must adhere to the cGMP or similar foreign regulations prescribed by the FDA or foreign regulatory authorities.

As such, 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, ARx and/or our other 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 LLC, we, ARx or our other 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. Moreover, as a result of the COVID-19 pandemic, third-party manufacturers have been and may in the future be affected, which could disrupt their activities and, as a result, we could face difficulty sourcing key components necessary to produce supply of our product candidates, which may negatively affect our preclinical and clinical development activities. 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, including ARx, 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 any approved products.

In order to conduct clinical trials of our product candidates and commercialize any approved product candidates, we, or our manufacturers, including ARx, 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. 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 IGALMI™ or product candidates.

If we are not able to establish 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 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 ("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 GCP 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 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

The COVID-19 pandemic or other pandemics, epidemics or outbreaks of an infectious disease may materially and adversely impact our business, including our preclinical studies and clinical trials.

The COVID-19 pandemic and government measures taken in response have had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. We have taken steps to protect our workforce and have instituted a modified return-to-the-office policy that we continue to evaluate.

As a result of the COVID-19 pandemic, outbreaks from variants of COVID-19, or other pandemics, epidemics or outbreaks of infectious disease, we may experience disruptions that could severely impact our business, preclinical studies and clinical trials, including:

- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site data monitoring, due to limitations on travel imposed or recommended by governments, employers and others or interruption of clinical trial subject visits and study procedures, which may impact the integrity of subject data and clinical study endpoints;

- interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;

- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems;

- interruptions in preclinical studies due to restricted or limited operations resulting from restrictions on our on-site activities;

- limitations on employee resources that would otherwise be focused on the conduct of our preclinical studies and clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;

- impacts from prolonged remote work arrangements, such as strains on our business continuity plans, cybersecurity risks, and inability of certain employees to perform their work remotely; and

- interruption or delays to our sourced discovery and clinical activities.

The COVID-19 pandemic continues to rapidly evolve. The extent to which the pandemic impacts our business, preclinical studies and clinical trials will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as rate of infection, the duration of the pandemic and subsequent waves of infection, the prevalence of new variants of the virus, travel restrictions and social distancing in the U.S. and other countries, business closures or business disruptions, the availability, adoption and effectiveness of any vaccines or treatments and the effectiveness of actions taken in the U.S. and other countries to contain and address the disease. If we or any of the third parties with whom we engage were to experience shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted. Additionally, concerns over the economic impact of COVID-19 pandemic have caused extreme volatility in financial and other capital markets which has and may continue to adversely impact our stock price and our ability to access capital markets.

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.

In addition to our employees, we have access to certain of BioXcel LLC's employees and resources through the various agreements we have entered into with BioXcel LLC. 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 to manage our operations and clinical trials, establish independent manufacturing, continue our research and development activities, and commercialize any approved product candidates. 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 our current, and any future, 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 preclinical 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 and a member of our Board, as well as the other principal members of our management, scientific, clinical teams and commercial readiness teams. 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. We have been relying on our commercial readiness team in connection with the commercialization of IGALMI™. 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. For example, we are continuing to build our commercial readiness team following the departure of certain senior executives. 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, had difficulty hiring and retaining highly skilled personnel with appropriate qualifications, and we have experienced increased costs to recruit such personnel. We expect to experience such difficulties in the future. The pool of qualified personnel with experience working within the biopharmaceutical and biotechnology 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 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 the fair value of 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 and Ethics, 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, pandemics such as the COVID-19 pandemic, 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, including ARx, to manufacture IGALMI™ and our product candidates and to 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.

Data breaches or cyber-attacks could disrupt our business operations and information technology systems, adversely impact our financial results or result in the loss or exposure of confidential or sensitive product candidate, clinical trial, employee or Company information.

Our information technology systems have been and may in the future be attacked or breached by individuals or organizations intending to obtain sensitive data regarding our business, our product candidates, clinical trials or other third parties with whom we do business; harm or disrupt our business operations; or otherwise misappropriate information or Company funds. A security compromise of our information technology systems or business operations could occur through a variety of methods such as from cyber-attacks and cyber-intrusions over the Internet, malware, computer viruses, email spoofing, attachments to e-mails, persons inside our organization or persons with access to systems inside our organization. The risk of such intrusions, threats to data and information technology systems and breaches has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We use our information technology systems to protect confidential or sensitive product candidate, clinical trial, employee and Company information. Any attack on such systems that results in the unauthorized release or loss of such information could have a material adverse effect on our business reputation, increase our costs and expose us to material legal claims and liability. If the unauthorized release or loss of product candidate, clinical trial, employee or other confidential or sensitive data were to occur, our operations and financial results and our share price could be adversely affected.

While we maintain some of our own critical information technology systems, we also depend on third parties to provide important information technology services relating to several key business functions. Our measures to prevent, detect and mitigate these threats, including password protection, firewalls, backup servers, threat monitoring and periodic penetration testing, may not be successful in preventing a data breach or limiting the effects of a breach. Because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may also experience security breaches that may remain undetected for an extended period.

Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence. Furthermore, the security measures employed by third-party service providers may prove to be ineffective at preventing breaches of their systems. Although we maintain insurance for our business, the coverage under our policies may not be adequate to compensate us for all losses that may occur.

Actual or perceived failures to comply with applicable data protection, privacy and security laws, regulations, standards and other requirements could adversely affect our business, results of operations, and financial condition.

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal data, such as information that we may collect in connection with clinical trials in the U.S. and abroad. Additionally, our use of AI and machine learning may be subject to laws and evolving regulations regarding the use of AI or machine learning, controlling for data bias, and antidiscrimination. Implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future, and we cannot yet determine the impact future laws, regulations, standards, or perception of their requirements may have on our business. This evolution may create uncertainty in our business, affect our ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us to comply with federal, state or foreign laws or regulation, our internal policies and procedures or our contracts governing our processing of personal information could result in negative publicity, government investigations and enforcement actions, claims by third parties and damage to our reputation, any of which could have a material adverse effect on our operations, financial performance and business.

As our operations and business grow, we may become subject to or affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities. In the U.S., HIPAA imposes, among other things, certain standards relating to the privacy, security, transmission and breach reporting of individually identifiable health information. Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. Such laws and regulations will be subject to interpretation by various courts and other governmental authorities, thus creating potentially complex compliance issues for us and our future customers and strategic partners. In addition, the California Consumer Privacy Act ("CCPA") went into effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability, and many similar laws have been proposed at the federal level and in other states in the U.S. Further, the California Privacy Rights Act ("CPRA") recently passed in California, which will impose additional data protection obligations on covered businesses, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data, and opt outs for certain uses of sensitive data. It will also create a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. The majority of the provisions will go into effect on January 1, 2023, and additional compliance investment and potential business process changes may be required. In the event that we are subject to or affected by HIPAA, the CCPA, the CPRA or other domestic privacy and data protection laws, any liability from failure to comply with the requirements of these laws could adversely affect our financial condition.

In Europe, the General Data Protection Regulation ("GDPR") went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the U.S., and the efficacy and longevity of current transfer mechanisms between the EU and the U.S. remains uncertain. For example, in 2016, the EU and U.S. agreed to a transfer framework for data transferred from the EU to the U.S., called the Privacy Shield, but the Privacy Shield was invalidated in July 2020 by the Court of Justice of

the EU ("CJEU"). The European Commission issued revised standard contractual clauses ("SCCs") on June 4, 2021 to account for the decision of the CJEU and recommendations made by the European Data Protection Board. The revised SCCs must be used for relevant new data transfers from September 27, 2021; existing standard contractual clauses arrangements must be migrated to the revised clauses by December 27, 2022. The new SCCs apply only to the transfer of personal data outside of the EEA and not the UK; the UK's Information Commissioner's Office launched a public consultation on its draft revised data transfers mechanisms in August 2021. There is some uncertainty around whether the revised clauses can be used for all types of data transfers, particularly whether they can be relied on for data transfers to non-EEA entities subject to the GDPR. As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the standard contractual clauses cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results.

Further, from January 1, 2021, companies have had to comply with the GDPR and also the UK GDPR, which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR (i.e., fines up to the greater of £17,500 or 4% of global turnover). The European Commission adopted an adequacy decision in favor of the UK, enabling data transfers from EU member states to the UK without additional safeguards. However, the UK adequacy decision will automatically expire in June 2025 unless the European Commission re-assesses and renews/extends that decision and remains under review by the Commission during this period. The relationship between the UK and the EU in relation to certain aspects of data protection law remains unclear, and it is unclear how UK data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the UK will be regulated in the long-term. These changes may lead to additional costs and increase our overall risk exposure.

Although we work to comply with applicable laws, regulations and standards, our contractual obligations and other legal obligations, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction to another, and may conflict with one another or other legal obligations with which we must comply. Any failure or perceived failure by us or our employees, representatives, contractors, consultants, collaborators, or other third parties to comply with such requirements or adequately address privacy and security concerns, even if unfounded, could result in additional cost and liability to us, damage our reputation, and adversely affect our business and results of operations.

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 third-party product candidates and technologies. 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 our net operating losses and tax credits to offset future taxable income and income tax liabilities may be limited.

At December 31, 2021, the Company had federal net operating loss carryforwards ("NOLs") of approximately $139 million and state NOLs of approximately $139 million. If not utilized, the federal and state NOLs, which are subject to expiration, will begin to expire in 2037. Federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely but may only be used to offset 80% of our taxable income in taxable years beginning after December 31, 2020. As of December 31, 2021, we also had approximately $6.5 million of federal orphan drug credits and research and development credits, or tax credits, which will begin to expire in 2037 if not utilized. The utilization of such NOLs and tax credits and realization of tax benefits in future years depends upon our having taxable income and income tax liabilities.

In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-ownership change NOLs and tax 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 rolling three-year period. We may have experienced ownership changes in the past, and future changes in our stock ownership, many of which are outside of our control, could result in ownership changes in the future. Our state NOLs or tax 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 tax credits. We have recorded a full valuation allowance related to our NOLs and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

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 U.S. and in certain foreign jurisdictions. Patents issue from non-provisional applications, which are typically filed from provisional patent applications or from PCT applications that enter the national phase. Neither provisional patent applications nor PCT applications issue directly as patents. We own PCT patent applications relating to our platform technologies covering methods of use and applications of the platform technologies.

As of April 26, 2022, our patent portfolio included five Patent Cooperation Treaty ("PCT") applications, 18 U.S. utility applications, one issued U.S. utility patent, six U.S. provisional patent applications, 103 pending non-U.S. applications, 13 allowed or granted non-U.S. patents (including five in Japan), one design patent application, which is a U.S. design application, and 34 allowed or registered design patents (including two in Japan). 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 U.S. or in foreign jurisdictions outside of the U.S. Changes in either the patent laws or interpretations of patent laws in the U.S. 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 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 various procedural, document submission, fee payment and other requirements 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 have been or will be submitted to the FDA for approval under Section 505(b)(2) of the 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 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 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, 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 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 U.S., 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 U.S. may be maintained in secrecy until the patents are issued;
- patent applications in the U.S. 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 U.S. patent applications on inventions similar to ours that claim 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 U.S. 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 and 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 adversely 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 LLC. 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 U.S.; 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 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 LLC, 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 USPTO, 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;
- speculative trading in and short sales of our stock, as well as trading phenomena such as the “short squeeze”;
- 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.

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

As of March 31, 2022, our directors, executive officers and BioXcel LLC, and their respective affiliates, beneficially owned approximately 37% 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.

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 (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 (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 of 2002 (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 IPO; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

We are also a smaller reporting company, and we will remain a smaller reporting company until, as of fiscal year end, we determine that either (1) our annual revenues are at least $100 million and our voting and non-voting common stock held by non-affiliates is at least $250 million measured on the last business day of our most recent second fiscal

quarter or (2) our voting and non-voting common stock held by non-affiliates is at least $700 million measured on the last business day of our most recent 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 of the Sarbanes-Oxley Act, 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 result 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 million 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. 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 U.S. 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 and will continue to incur significant additional legal, accounting and other expenses that we did not incur as a privately held subsidiary of BioXcel LLC. The obligations of being a public company in the U.S. require significant expenditures and place significant demands on our management and

other personnel, including costs resulting from public company reporting obligations under the Exchange Act and the rules and regulations regarding corporate governance practices, including those under the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection 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 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 LLC. Our continued compliance with applicable requirements and to keep pace with new regulations requires management and other personnel to devote a substantial amount of their time, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems.

General Risk Factors

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.

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.

If we fail to comply with the rules under the Sarbanes-Oxley Act 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. We have discovered material weaknesses in the past. If future 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.

In 2017, the U.S. government 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. Future changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of any foreign earnings, and the deductibility of expenses under future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Not applicable.

Item 6. Exhibits

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
3.1	Amended and Restated Certificate of Incorporation, as amended.	10-Q	001-38410	3.1	8/10/2021	
3.2	Amended and Restated Bylaws.	8-K	001-38410	3.2	3/13/2018	
10.1	BioXcel Trademark License Agreement, between the Company and BioXcel LLC, dated April 9, 2022					*
10.2	Second Amendment to the Second Amended and Restated Separation and Shared Services Agreement, between the Company and BioXcel LLC, dated April 19, 2022					*
10.3	Registration Rights Agreement, dated April 19, 2022, among the Company and Oaktree-TCDRS Strategic Credit, LLC, Oaktree-Forrest Multi-Strategy, LLC, Oaktree-TBMR Strategic Credit Fund C, LLC, Oaktree-TBMR Strategic Credit Fund F, LLC, Oaktree-TBMR Strategic Credit Fund G, LLC, Oaktree-TSE 16 Strategic Credit, LLC, INPRS Strategic Credit Holdings, LLC, Oaktree Strategic Income II, Inc., Oaktree Specialty Lending Corporation, Oaktree Strategic Credit Fund, Oaktree GCP Fund Delaware Holdings, L.P., Oaktree Diversified Income Fund Inc., Oaktree AZ Strategic Lending Fund, L.P., Oaktree Loan Acquisition Fund, L.P., Oaktree LSL Fund Delaware Holdings EURRC, L.P., and Q Boost Holding LLC.	8-K	001-38410	4.2	4/19/2022	
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.					*
31.2	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.					*
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.					**
32.2	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.					**
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document					*
101.SCH	Inline XBRL Taxonomy Extension Schema Document					*
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document					*

101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document	*
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document	*
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document	*
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).	*

* Filed herewith.
** Furnished herewith.

SIGNATURES

Pursuant to the requirements 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: May 9, 2022

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

Dated: May 9, 2022

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

Exhibit 10.1

Execution Version

BioXcel Trademark License Agreement

This BioXcel Trademark License Agreement ("**Agreement**") is by and between BioXcel LLC ("**Licensor**") and BioXcel Therapeutics, Inc. ("**Licensee**") (collectively, the Licensee and Licensor may be referred to herein as the "**Parties**," or each individually, a "**Party**"). The Agreement is effective as of April 19, 2022 (the "**Effective Date**").

WHEREAS, Licensor is the former parent company of Licensee, and currently holds a 31% interest in Licensee. Licensee is engaged in developing, marketing, promoting and selling any medical products and services in the field of neuroscience ("**Business**");

WHEREAS, Licensor owns the BIOXCEL trademark ("**Licensed Mark**"), in which it has common law rights and trademark registrations for the same identified in **Schedule 1** attached hereto;

WHEREAS, Licensee wishes to use the Licensed Mark, and Licensor is willing to grant to Licensee a license to use the Licensed Mark in connection with developing medicines in the field of neuroscience, to facilitate Licensee's conduct of the Business; and

WHEREAS, Licensee has entered into that certain Credit Agreement and Guaranty with the subsidiary guarantors party thereto, the lenders party thereto and Oaktree Fund Administration, LLC, as administrative agent for the lenders (in such capacity, the "**Administrative Agent**"), dated as of April 19, 2022 (as amended, amended and restated, supplemented or modified from time to time, the "**Credit Agreement**").

NOW, THEREFORE, in consideration of the mutual covenants and agreements hereinafter set forth and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows.

1. License.

1.1 License Grant. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee during the Term (as defined in Section 6.1) an exclusive (solely during the Initial Period, and solely within the field of neuroscience), royalty-free license to use the Licensed Mark in connection with developing, marketing, promoting and selling any products and services in the field of neuroscience in any territory where the Business is conducted by Licensee in whole or in part.

1.2 Sublicensing. During the Initial Period only, Licensee shall have the right to grant sublicenses under the license rights granted under Section 1.1. Licensee's right to grant sublicenses under the Licensed Mark is subject to the following: (a) no sublicense may exceed the scope of rights granted to Licensee under this Agreement; (b) in the event of expiration or termination of this Agreement, all sublicense rights will terminate automatically effective as of the expiration or termination date of this Agreement; (c) in the event of expiration of the Initial Period, all sublicense rights will terminate automatically effective as of the expiration of the Initial Period; (d) Licensee shall require all sublicensees to agree in writing to be bound by the applicable terms and conditions of this Agreement including all terms that preserve or protect the rights of Licensor in Licensed Mark; and (e) Licensee shall be responsible and liable for the acts or omissions of such sublicensees that constitute a breach of any of the terms

and conditions of this Agreement as if such acts or omissions were the acts or omissions of Licensee. Within seven (7) days of any grant of a sublicense of the Licensed Mark, Licensee must provide Licensor notice of such grant, and a copy of any document reflecting or relating to such grant, including documents concerning subsection (d) of this Section. Upon expiration of the Initial Period, Licensee may not grant sublicenses under this Agreement without Licensor's prior written consent. Any purported sublicenses in violation of this Section will be void and of no force and effect.

1.3 Assignment. During the Initial Period only, and after providing prior fourteen (14) days written notice to Licensor, Licensee shall have the right to assign or otherwise transfer this Agreement, or any right or obligation hereunder, to (a) an Affiliate of Licensee; or (b) any person or entity who becomes the owner of the portion of the Business with which the Licensed Mark has been associated (or in connection with such person's or entity's acquisition of such portion of the Business), following the occurrence and during the continuance of an Event of Default. Licensee shall require the assignee or transferee, as applicable, to acknowledge and agree in writing to assume and be bound by all of the applicable terms and conditions of this Agreement, which Licensee shall promptly provide to Licensor. Upon expiration of the Initial Period, Licensee may not assign or transfer any of its rights or obligations under this Agreement without Licensor's prior written consent. Any purported assignment or transfer in violation of this Section will be void and of no force and effect. Licensor agrees that Licensee may pledge or grant a lien in its rights under this Agreement, subject to the terms and conditions of this Agreement. Licensor is permitted to assign or transfer its rights or obligations under this Agreement.

"**Affiliate**" of a Party, as used in this Agreement means any other Party that directly or indirectly, through one or more intermediaries, Controls, is Controlled by, or is under common Control with, such Party.

"**Control**", as used in this Agreement, means the power to direct or cause the direction of the management and policies of a Party, whether through the ownership of voting securities, by contract, or otherwise/direct, and "Controlled by" and "under common Control with" have correlative meanings.

"**Event of Default**", as used in this Agreement, has the meaning ascribed to it in the Credit Agreement.

1.4 Exclusivity. During the Initial Period only, Licensor shall not (a) use the Licensed Mark in the field of neuroscience, nor (b) license the Licensed Mark to anyone except for Licensor's own Affiliates and the Licensee. Licensor shall (i) ensure that any such licenses granted to Licensor's Affiliates will prohibit use of the Licensed Mark in connection with any products or services within the field of neuroscience and will identify Licensee as an express intended beneficiary for the purpose of enforcing the same, and (ii) cause its Controlled Affiliates to not use the Licensed Mark in the field of neuroscience. During the Initial Period only, Licensee may use its reasonable efforts to exercise rights granted to Licensee under Section 3.3 to ensure Licensor's non-Controlled Affiliates do not use the Licensed Mark in the field of neuroscience. Upon expiration of the Initial Period, nothing in

this Agreement shall restrict Licensor's right to use the Licensed Mark or freely license the Licensed Mark to anyone.

1.5 Reservation of Rights. Licensor hereby reserves all rights not expressly granted to Licensee under this Agreement.

2. Quality Control.

2.1 Quality Standards. Licensor will not, and will require that those of its Affiliates (except for Licensee) who Licensor permits or grants rights to use the Licensed Mark to not, take any actions to tarnish, dilute or impair the value of the Licensed Mark or the associated goodwill. Licensee acknowledges and is familiar with the high standards and reputation for quality the Licensed Mark represents, and Licensee shall, at all times, conduct the Business and use the Licensed Mark in a manner at least consistent with such quality standards and reputation.

2.2 Use of the Licensed Mark. Licensee shall comply with Licensor's then current guidelines and specifications regarding the style, appearance, and usage of the Licensed Mark and shall ensure that all uses of the Licensed Mark under this Agreement comply with all applicable laws. Licensee shall use proper notice symbols and legends as instructed by Licensor.

2.3 Licensor's Quality Control. Licensor shall, and shall require its Affiliates (except for Licensee) who Licensor permits or grants rights to use the Licensed Mark to, exercise quality control over all uses of the Licensed Mark under this Agreement to maintain the validity of the Licensed Mark and protect the goodwill associated therewith. For the purpose of monitoring Licensee's compliance with Licensor's quality standards and the other requirements set forth in this Section 2, at Licensor's reasonable request (a) Licensor (or its representative) may inspect the Licensee's facilities, on reasonable notice and during normal business hours; and (b) Licensee shall submit to Licensor a representative sample of any use of the Licensed Mark by Licensee for Licensor's review and approval, subject to Section 2.4.

2.4 Approvals. Licensor acknowledges and agrees that all uses of the Licensed Mark made by the Licensee as of the Effective Date meet Licensor's quality standards and the other requirements set forth in this Section 2 and are hereby deemed approved by Licensor. Approval of any use by a Licensee of the Licensed Mark, once given by Licensor, will continue without need for future approval (subject to Licensor's right to revoke approval), so long as that Licensee's use of the Licensed Mark in connection with the Business continues to be substantially consistent with such previously approved use.

3. Ownership and Protection of the Licensed Mark.

3.1 Acknowledgment of Ownership. Licensee acknowledges that Licensor owns and will retain all right, title, and interest in and to the Licensed Mark. All use by the Licensee of the Licensed Mark, anywhere in the world, and all goodwill accruing therefrom, will inure solely to the benefit of Licensor. Licensor acknowledges that nothing in this Agreement transfers to Licensor any right, title, and interest in and to Licensee's BIOXCEL THERAPEUTICS and BT BIOXCEL THERAPEUTICS trademark (including the right for Licensee to enforce its rights in and to the BIOXCEL THERAPEUTICS and BT BIOXCEL THERAPEUTICS trademarks and the right to receive damages or proceeds in connection therewith), which are separate and distinct from the Licensed Mark, and to which this Agreement does not apply except as expressly stated. Upon Licensee's request, Licensor shall execute a letter of consent to assist Licensee with Licensee's registration of the BIOXCEL THERAPEUTICS and BT BIOXCEL THERAPEUTICS marks, and will do so within fourteen (14) days after agreeing to mutually-acceptable language for such letter which shall not be unreasonably delayed.

3.2 Registration and Maintenance. Licensor has the sole right and discretion and at its expense, to file, prosecute, and maintain all applications and registrations for the Licensed Mark and Licensee shall not, except for any applications filed by Licensee in connection with Licensee's use or ownership of BIOXCEL THERAPEUTICS or BT BIOXCEL THERAPEUTICS, apply for or assist anyone else to apply for any registration of any trademark, service mark, trade name or indicia confusingly similar to the Licensed Mark. At Licensee's reasonable request and in order to support Licensee's Business, Licensor shall file additional applications for Licensor to register the Licensed Mark or modify Licensor's existing registrations for the Licensed Mark in connection with use of the Licensed Mark in the field of neuroscience as permitted under this Agreement (all such registrations or amendments are "**Additional Registrations**"). Licensee shall provide, at the request of Licensor and, unless otherwise agreed, at Licensor's expense, all necessary assistance with such filing, maintenance, and prosecution such as by way of example and without limitation, providing specimens of Licensee's use of the Licensed Mark as permitted under this Agreement as necessary for Licensor to seek Additional Registrations.

3.3 Enforcement.

(a) Each Party shall promptly notify the other of any actual, suspected, or threatened infringement, dilution, or other conflicting use of the Licensed Mark by any third party of which it becomes aware.

(b) Licensor shall have the right, but no obligation, to bring or take any such action as it determines is necessary to halt any such infringement, dilution, or other conflicting use of the Licensed Mark, and to control the conduct of such enforcement action, including settlement. During the Initial Period, Licensee shall have the right, but no obligation, to bring or take any such enforcement action to halt any infringement, dilution, or other conflicting use of the Licensed Mark in the field of neuroscience ("**Neuroscience Enforcement Action**"), and to control the conduct of such Neuroscience Enforcement Action, including settlement so long as any

settlement is approved by Licensor, with such approval to not be unreasonably withheld.

(c) The Party taking such action (the "**Enforcing Party**") shall be responsible for the expenses of such enforcement action, including attorneys' fees, and the other Party shall provide such assistance as may be reasonably requested by the Enforcing Party, at the Enforcing Party's expense, in connection with any such enforcement action (including being joined as a party to such action as necessary to establish and maintain standing). Any monetary recovery resulting from such enforcement action shall first be used to pay the legal expenses of the Enforcing Party and then to reimburse any legal expenses incurred by the other Party in cooperating in such action as requested by the Enforcing Party, and any remaining amounts shall belong solely to the Enforcing Party.

4. Indemnification. Each Party, subject to the limitations on Licensor's obligations listed below, shall indemnify, defend, and hold harmless the other Party, and their respective Affiliates, officers, directors, employees, agents, and representatives against all losses, liabilities, claims, damages, actions, fines, penalties, expenses, or costs (including court costs and reasonable attorneys' fees) ("**Losses**") arising out of or in connection with any third-party claim, suit, action, or proceeding relating to use of the Licensed Mark under this or a breach of this Agreement by such Party.

5. Disclaimer; Limitation of Liability.

5.1 Disclaimer. EACH PARTY EXPRESSLY DISCLAIMS ALL REPRESENTATIONS AND WARRANTIES, WHETHER EXPRESS, IMPLIED, STATUTORY, OR OTHERWISE, IN CONNECTION WITH THIS AGREEMENT AND THE LICENSED MARK, INCLUDING ANY WARRANTIES OF TITLE, NON-INFRINGEMENT, MERCHANTABILITY, OR FITNESS FOR A PARTICULAR PURPOSE. WITHOUT LIMITING THE GENERALITY OF THE FOREGOING, LICENSOR MAKES NO REPRESENTATION OR WARRANTY THAT THE LICENSED MARK IS VALID OR THAT THE EXERCISE BY LICENSEE OF ANY RIGHTS GRANTED UNDER THIS AGREEMENT WILL NOT INFRINGE THE RIGHTS OF ANY PERSON OR ENTITY.

5.2 Limitation of Liability. EXCEPT FOR AND WITHOUT LIMITING LICENSEE'S OBLIGATIONS UNDER SECTION 4 AND EXCEPT FOR LICENSEE'S LIABILITY FOR INDEMNIFICATION UNDER SECTION 4, NO PARTY WILL BE LIABLE TO THE OTHER PARTY FOR ANY CONSEQUENTIAL, INCIDENTAL, INDIRECT, EXEMPLARY, SPECIAL, OR PUNITIVE DAMAGES RELATING TO THIS AGREEMENT OR USE OF THE LICENSED MARK HEREUNDER, WHETHER ARISING OUT OF BREACH OF CONTRACT, TORT (INCLUDING NEGLIGENCE), OR OTHERWISE, REGARDLESS OF WHETHER SUCH DAMAGE WAS FORESEEABLE AND WHETHER SUCH PARTY HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES.

6. Term and Termination.

6.1 Term. This Agreement begins on the Effective Date and will remain in force until terminated pursuant to Section 6.3 or 6.4 ("**Term**"). The Parties' rights and obligations pursuant to Sections 3.1, 3.2 and 4 shall survive the Term of this Agreement.

6.2 Initial Period and Perpetual License. "**Initial Period**" means the period beginning on the Effective Date and expiring upon satisfaction of the Obligations (other than inchoate indemnity obligations). "**Obligations**," as used in this Section, has the meaning ascribed to the term in the Credit Agreement. In the event that Obligations (other than inchoate indemnity obligations) are satisfied due to any foreclosure by Oaktree Fund Administration, LLC of Licensor's assets securing the Credit Agreement, then the license in the Licensed Mark granted under Section 1.1 will be granted in perpetuity and such license will survive termination of this Agreement.

6.3 Termination by Licensor. During the Term, Licensor may terminate this Agreement immediately upon written notice to Licensee if Licensee materially breaches this Agreement and fails to cure such breach within sixty (60) days after receiving written notice thereof. Upon expiration of the Initial Period, Licensor may terminate this Agreement at any time without cause by providing Licensee thirty (30) days' prior written notice.

6.4 Termination by Licensee. Licensee may terminate this Agreement at any time without cause, and without incurring any additional obligation, liability, or penalty under this Agreement, by providing at least thirty (30) days' prior written notice to Licensor.

7. General Provisions.

7.1 Payment. As part of the consideration for Licensor entering into this Agreement, Licensee shall pay to Licensor the consideration set forth in **Schedule 2** attached hereto.

7.2 Amendments. No amendment to this Agreement shall be effective unless it is in writing and signed by the Licensor and Licensee.

7.3 No Third-Party Beneficiaries. Except for the right of Licensor's officers, directors, employees, agents, and representatives to enforce their rights under Section 4, this Agreement solely benefits the Parties and their respective permitted successors and assigns. Nothing in this Agreement, express or implied, confers on any other person or entity any legal or equitable right, benefit, or remedy of any nature whatsoever under or by reason of this Agreement.

7.4 Counterparts. This Agreement may be executed in counterparts, each of which is deemed an original, but all of which together are deemed to be one and the same agreement. Any Party may execute this Agreement by digital, electronic, or ink signature.

7.5 Severability. If any term or provision of this Agreement is invalid, illegal, or unenforceable in any jurisdiction, such invalidity, illegality, or unenforceability will not affect any other term or provision of this Agreement or invalidate or render unenforceable such term or provision in any other jurisdiction.

7.6 Governing Law. This Agreement, including the schedules hereto, is governed by, and construed in accordance with, the laws of the State of New York, without regard to the conflict of laws provisions thereof to the extent such principles or rules would require or permit the application of the laws of any jurisdiction other than those of the State of New York.

7.7 Waiver. Except as otherwise set forth in this Agreement, no failure to exercise, or delay in exercising, any rights, remedy, power, or privilege arising from this Agreement will operate or be construed as a waiver thereof, nor will any single or partial exercise of any right, remedy, power, or privilege hereunder preclude any other or further exercise thereof or the exercise of any other right, remedy, power, or privilege.

7.8 Notices. All correspondence or notices required or permitted to be given under this Agreement must be in writing, in English, and addressed to the other Party at its address set out below (or to any other address that the receiving Party may designate from time to time). Each Party shall deliver all notices by personal delivery, nationally recognized overnight courier (with all fees prepaid), email (with confirmation of transmission), or certified or registered mail (in each case, return receipt requested, postage prepaid). Such communications must be sent to the respective Parties at the following addresses (or at such other address for a Party as specified in a notice given in accordance with this Section):

BioXcel LLC
780 East Main Street
Branford, Connecticut 06405
Email: [***]
Attention: [***]

BioXcel Therapeutics, Inc.
555 Long Wharf Drive, 12th Floor
New Haven, Connecticut 06516
Email: [***]
Attention: [***]

7.9 Entire Agreement. This Agreement, including and together with any related exhibits, schedules, attachments, and appendices, constitutes the sole and entire agreement of Licensor and Licensee with respect to the subject matter contained herein, and supersedes all prior and contemporaneous understandings, agreements, representations, and warranties, both written and oral, regarding such subject matter.

[Remainder of Page Intentionally Left Blank]

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their respective officers thereunto duly authorized.

BioXcel LLC

By /s/ Ankush Sethi
Name: Ankush Sethi
Title: Chief Operating Officer

BioXcel Therapeutics, Inc.

By /s/ Richard Steinhart
Name: Richard Steinhart
Title: Senior Vice President and Chief Financial Officer

SCHEDULE 1

BioXcel LLC's Trademark Registrations for BIOXCEL

Schedule 2

Payments

In consideration for the rights and licenses granted by Licensor to Licensee under this Agreement, Licensee shall pay to Licensor an initial one-time payment of $135,000, which shall be paid within thirty (30) business days of the Effective Date. Promptly after the Effective Date, but no later than sixty (60) days of the Effective Date, the Parties shall determine the arm's length value of such rights and licenses granted by Licensor to Licensee under this Agreement in accordance with § 482 of the Internal Revenue Code of 1986, as amended and the Treasury Regulations promulgated thereunder, as determined by a reputable valuation or accounting firm, jointly engaged by the Licensee and Licensor.

All payments hereunder shall be made in the lawful currency of the United States of America. All payments hereunder will be made by wire transfer of immediately available funds into the following account:

Bank name: [***]
Routing Number: [***]
Account Number: [***]

All sums stated in this Agreement, including this **Schedule 2**, are exclusive of value-added tax, goods and services tax, sales tax, consumption tax and similar or related taxes, duties, levies or charges. Each Party shall be responsible for its own income taxes. Licensee shall make all payments to be made by it under this Agreement without any deduction or withholding for or on account of tax, unless such a deduction or withholding is required by law, in which case Licensee shall make such deduction or withholding as required by law and make the relevant payment gross of such deduction or withholding. To the extent Licensee is required to withhold taxes, Licensee agrees to provide Licensor with written evidence of such withholding and payment to the relevant authority. The Parties shall reasonably cooperate in obtaining any available waivers, allowances, or exemptions with respect to any taxes or fees arising in connection with any payments under this Agreement. Each Party shall promptly advise the other of the receipt of any notices from any tax authority regarding amounts due and payments made under this Agreement and shall provide such assistance in connection with any tax examination relating to this Agreement as the other Party shall reasonably request.

Exhibit 10.2

Execution Version

SECOND AMENDMENT TO SECOND AMENDED & RESTATED SEPARATION AND SHARED SERVICES AGREEMENT

This Second Amendment to Second Amended & Restated Separation and Shared Services Agreement ("***Second Amendment***"), effective as of April 19, 2022 (the "***Second Amendment Effective Date***"), is made by and between BioXcel LLC, a Delaware limited liability company, located at 2614 Boston Post Road Guilford, CT 06437 ("***BioXcel***"), and BioXcel Therapeutics, Inc., a Delaware corporation located at 555 Long Wharf Drive, New Haven, CT 06511 ("***BTI***"). BioXcel and BTI may be referred to herein individually as a "***Party***", or collectively as the "***Parties***." Capitalized terms used herein without definition shall have the respective meanings ascribed to such terms under the Shared Services Agreement (as defined below).

WHEREAS, BioXcel (as successor in interest to BioXcel Corporation) and BTI are parties to that certain Second Amended & Restated Shared Services Agreement, dated March 6, 2020 (as amended by that certain First Amendment to Second Amended & Restated Separation and Shared Services Agreement (the "***First Amendment***"), and as may be further amended, restated, amended and restated, or as otherwise modified from time to time, the "***Shared Services Agreement***"); and

WHEREAS, BioXcel and BTI desire to further amend the Shared Services Agreement in respect of BTI's option thereunder to enter into a Collaborative Services Agreement with BioXcel.

NOW, THEREFORE, the Parties hereby agree as follows:

A. Amendments. The Shared Services Agreement is hereby amended as follows, effective as of the Second Amendment Effective Date:

1. Section 2(d) of the Shared Services Agreement shall be deleted in its entirety and replaced with the following:

> d. EvolverAI Collaborative Services. On or before December 31, 2024, BTI shall have the option to enter into a Collaborative Services Agreement with BioXcel by which BioXcel shall perform product identification and related services for BTI utilizing the EvolverAI Platform. The Parties agree to negotiate the terms of such Collaborative Services Agreement in good faith and that such agreement will 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 shall 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 $30M in the aggregate. BioXcel shall continue to make such product identification and related services available to BTI until at least December 31, 2024. As consideration for the foregoing, BTI has agreed to pay BioXcel consideration in the amount of $18,000 per month, prorated for any partial month, as applicable, for the period beginning March 13, 2023 and ending December 31, 2024.

B. Miscellaneous.

1. Except to the extent amended by this Second Amendment, all of the definitions, terms, provisions and conditions set forth in the Shared Services Agreement are ratified and confirmed and shall remain in full force and effect.

2. As of the Second Amendment Effective Date, the Shared Services Agreement and this Second Amendment shall be read and construed together as a single agreement, and any and all references in the Shared Services Agreement to "this Agreement", "hereunder", "herein", "hereof" or words of like import referring to the Shared Services Agreement shall be deemed a reference to the Shared Services Agreement as amended by this Second Amendment.

3. This Second Amendment may be signed in any number of counterparts (facsimile and electronic transmission included), each of which shall be deemed an original, but all of which shall constitute one and the same instrument. Signatures to this Second Amendment may be provided by PDF file and shall be deemed to be original signatures.

[Signature page follows]

IN WITNESS WHEREOF, BioXcel and BTI, intending to be legally bound, have caused this Second Amendment to be signed by their duly authorized representatives.

BIOXCEL LLC

By: /s/ Ankush Sethi
Name: Ankush Sethi
Title: Chief Operating Officer

BIOXCEL THERAPEUTICS, INC.

By: /s/ Richard Steinhart
Name: Richard Steinhart
Title: Senior Vice President and Chief Financial Officer

Exhibit 31.1

CERTIFICATIONS

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

1. I have reviewed this Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2022 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: May 9, 2022

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 Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2022 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: May 9, 2022

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 Quarterly Report on Form 10-Q of BioXcel Therapeutics, Inc. (the "Company") for the quarterly period ended March 31, 2022, 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: May 9, 2022

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 Quarterly Report on Form 10-Q of BioXcel Therapeutics, Inc. (the "Company") for the quarterly period ended March 31, 2022, 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: May 9, 2022

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