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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 September 30, 2021

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 November 5, 2021 was 27,980,345.

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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 plans relating to clinical trials for BXCL501, BXCL701 and our other product candidates;
- our plans for 505(b)(2) regulatory path approval;
- our plans to research, develop and commercialize our current and future product candidates;
- our plans to seek to enter into collaborations for the development and commercialization of certain product candidates;
- the potential benefits of any future collaboration;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our estimates regarding expenses, future revenue, capital requirements and need for additional financing;
- developments relating to our competitors and our industry;
- the impact of government laws and regulations;
- the impact of COVID-19 on our business, including our preclinical studies and clinical trials: 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.

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 sections 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 section of ours 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 have never had a drug approved.
- In the near term, we are dependent on the success of BXCL501 and BXCL701. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize BXCL501, BXCL701 and our other product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.
- 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 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 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.
- 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 BXCL501, BXCL701 or any product candidate, we will still face extensive and ongoing regulatory requirements and obligations and any product candidates, if approved, may face future development and regulatory difficulties.
- If our products 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 BXCL501, BXCL701 or our other 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.

BALANCE SHEETS

(amounts in thousands)

	September 30, 2021 (unaudited)	December 31, 2020
ASSETS		
Current assets		
Cash and cash equivalents	$ 252,912	$ 213,119
Prepaid expenses and other current assets	4,017	3,946
Total current assets	256,929	217,065
Property and equipment, net	1,336	1,273
Operating lease right-of-use assets	1,312	1,511
Other assets	1,755	87
Total assets	$ 261,332	$ 219,936
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 3,439	$ 3,979
Accrued expenses	10,716	7,469
Due to related party	154	157
Other current liabilities	287	237
Total current liabilities	14,596	11,842
Long-term portion of operating lease liabilities	1,180	1,398
Total liabilities	15,776	13,240
Commitments and contingencies (Note 11)		
Stockholders' equity		
Common stock, $0.001 par value, 100,000 and 50,000 shares authorized as of September 30, 2021 and December 31, 2020, respectively; 27,980 and 24,417 shares issued and outstanding as of September 30, 2021 and December 31, 2020, respectively	28	24
Preferred stock, $0.001 par value, 10,000 shares authorized; no shares issued and outstanding as of September 30, 2021 and December 31, 2020, respectively	—	—
Additional paid-in-capital	465,191	345,529
Accumulated deficit	(219,663)	(138,857)
Total stockholders' equity	245,556	206,696
Total liabilities and stockholders' equity	$ 261,332	$ 219,936

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

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Revenues	$ —	$ —	$ —	$ —
Operating expenses				
Research and development	11,933	16,317	40,183	46,595
General and administrative	14,879	8,451	40,621	14,605
Total operating expenses	26,812	24,768	80,804	61,200
Loss from operations	(26,812)	(24,768)	(80,804)	(61,200)
Other income (expense)				
Interest income	12	20	32	140
Interest expense	(11)	(5)	(34)	(23)
Net loss and comprehensive loss	$ (26,811)	$ (24,753)	$ (80,806)	$ (61,083)
Basic and diluted net loss per share attributable to common stockholders	$ (0.96)	$ (1.07)	$ (3.13)	$ (2.94)
Weighted average shares outstanding - basic and diluted	27,972	23,050	25,832	20,779

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

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

(amounts in thousands)
(unaudited)

	Common Stock		Additional Paid in	Accumulated	
	Shares	Amount	Capital	Deficit	Total
Balance as of December 31, 2019	18,087	$ 18	$ 83,565	$ (56,688)	$ 26,895
Issuance of common stock, net of issuance costs of $4,789	2,300	2	68,809	—	68,811
Purchase and cancellation of shares from BioXcel Corporation	(300)	—	(9,024)	—	(9,024)
Stock-based compensation	—	—	776	—	776
Exercise of stock options	95	—	39	—	39
Net loss	—	—	—	(14,911)	(14,911)
Balance as of March 31, 2020	20,182	$ 20	$ 144,165	$ (71,599)	$ 72,586
Stock-based compensation	—	—	1,956	—	1,956
Exercise of stock options	171	—	271	—	271
Net loss	—	—	—	(21,419)	(21,419)
Balance as of June 30, 2020	20,353	$ 20	$ 146,392	$ (93,018)	$ 53,394
Issuance of common stock, net of issuance costs of $12,991	4,000	4	187,004	—	187,008
Stock-based compensation	—	—	5,268	—	5,268
Exercise of stock options	2	—	21	—	21
Net loss	—	—	—	(24,753)	(24,753)
Balance as of September 30, 2020	24,355	$ 24	$ 338,685	$ (117,771)	$ 220,938

	Common Stock		Additional Paid in	Accumulated	
	Shares	Amount	Capital	Deficit	Total
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
Issuance of common stock, net of issuance costs of $3,542	3,279	3	101,024	—	101,027
Stock-based compensation	—	—	6,769	—	6,769
Exercise of stock options	59	—	497	—	497
Net loss	—	—	—	(27,619)	(27,619)
Balance as of June 30, 2021	27,969	$ 28	$ 460,235	$ (192,852)	$ 267,411
Stock issuance costs	—	—	(34)	—	(34)
Stock-based compensation	—	—	4,885	—	4,885
Exercise of stock options	11	—	105	—	105
Net loss	—	—	—	(26,811)	(26,811)
Balance as of September 30, 2021	27,980	$ 28	$ 465,191	$ (219,663)	$ 245,556

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

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amounts in thousands)
(unaudited)

	Nine months ended September 30,	
	2021	2020
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (80,806)	$ (61,083)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	221	143
Loss on disposal of equipment	46	—
Stock-based compensation expense	17,219	8,000
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,739)	(1,059)
Operating lease right of use assets	199	213
Accounts payable, accrued expenses, and other liabilities	2,807	7,830
Operating lease liabilities	(168)	(122)
Net cash used in operating activities	(62,221)	(46,078)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of equipment and leasehold improvements	(433)	(46)
Net cash used in investing activities	(433)	(46)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net of issuance costs	100,993	255,819
Purchase and cancellation of shares from BioXcel LLC	—	(9,024)
Exercise of stock options	1,454	331
Net cash provided by financing activities	102,447	247,126
Net increase in cash and cash equivalents	39,793	201,002
Cash and cash equivalents, beginning of the period	213,119	32,426
Cash and cash equivalents, end of the period	$ 252,912	$ 233,428
Supplemental cash flow information:		
Purchases of equipment and leasehold improvements included in accounts payable and accrued expense as of December 31, 2020, but paid in the current period	103	—
Operating lease ROU assets obtained in exchange for operating lease liabilities	—	606

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

BIOXCEL THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS

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

Note 1. Nature of the Business

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes artificial intelligence to identify improved therapies in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI's two most advanced clinical development programs are BXCL501, a proprietary, orally dissolving, thin film formulation of the adrenergic receptor agonist dexmedetomidine (“Dex”), for the treatment of agitation and opioid withdrawal symptoms, 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.

The Company incurred losses of $26,811 and $24,753 for the three months ended September 30, 2021 and 2020, respectively and $80,806 and $61,083 for the nine months ended September 30, 2021 and 2020, respectively. The Company had an accumulated deficit of $219,663 as of September 30, 2021. The Company has funded its operations primarily through the sale of equity securities.

Impact of COVID-19 Pandemic

In March 2020, the World Health Organization declared the outbreak of COVID-19, a novel strain of coronavirus, a global pandemic. This pandemic has caused and is continuing to cause major disruptions to businesses and financial markets worldwide. This may affect the Company’s operations and those of third parties on which the Company relies, including causing disruptions in the supply of the Company’s product candidates and the conduct of current and planned preclinical and clinical studies. The Company may need to limit its operations and may experience limitations in employee resources. There are risks that the COVID-19 pandemic, or the spread of the variants of the disease, may be more difficult to control than currently anticipated in which case the risks described herein could increase significantly. The extent to which the COVID-19 pandemic impacts the Company’s results will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the prevalence of variants of the disease, the efficacy and adoption of vaccines, and actions to contain the coronavirus or treat its impact, among others.

Additionally, while the potential economic impact brought by, and the duration of, the COVID-19 pandemic is difficult to assess or predict, the impact of the coronavirus on the global financial markets may reduce the Company’s ability to access capital, which could negatively impact the Company’s short-term and long-term liquidity, and the Company’s ability to complete its preclinical and clinical studies on a timely basis, or at all. The ultimate impact of COVID-19 is highly uncertain and subject to change. The Company does not yet know the full extent of potential delays or impacts on its business, financing, preclinical and clinical trial activities or the global economy as a whole. However, these effects could have a material, adverse impact on the Company’s liquidity, capital resources, operations and business and those of the third parties on which the Company relies.

Note 2. Basis of Presentation

The accompanying unaudited financial statements do not include all of the information and footnotes required by Generally Accepted Accounting Principles in the United States of America ("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 September 30, 2021, the results of its operations for the three and nine months ended September 30, 2021 and 2020 and its cash flows for the nine months ended September 30, 2021 and 2020, respectively. The results for the three and nine months ended September 30, 2021 are not necessarily indicative of results to be expected for the year ending December 31, 2021, 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 as of and for the year ended December 31, 2020 included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2020 filed with the Securities and Exchange Commission (the "SEC") on March 12, 2021.

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. 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 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 our financial statements requires us to make estimates, judgments and assumptions that may affect the reported amounts of assets, liabilities, equity and expenses. We base our estimates on historical experience and on various other assumptions that we believe are reasonable, the results of which form the basis for making judgments about the carrying values of assets, liabilities and equity and the amount of expenses.

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 September 30, 2021 and December 31, 2020, cash equivalents were comprised primarily of U.S. government 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.

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 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 in income (loss) from operations.

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

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 lease did 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 lease terms may include options to extend or terminate the lease 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. 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 market values for all share-based awards made to employees, directors, and non-employees, including stock options. 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 Company's 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 option awards are valued at fair value on the date of grant and that fair value is recognized over the requisite service period. The estimated fair value of stock option awards was determined using the Black-Scholes option pricing model on the date of grant. Significant judgment and estimates were used to estimate the fair value of awards, granted prior to the Company's IPO in March 2018. Stock awards granted by the Company subsequent to the IPO are valued using market prices at the date of grant.

ASC 718 requires companies to estimate the fair value of share-based awards on the date of grant using an option-pricing model. The Black-Scholes option-pricing model was used as its method of determining fair value. This model is affected by the Company's stock price as well as assumptions regarding a number of subjective variables. These subjective variables include, but are not limited to, the expected stock price volatility over the term of the awards, and projected employee stock option exercise behaviors. The value of the award is recognized as an expense in the statement of operations over the requisite service period. The 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 and manufacturing costs and other expenses that are directly related to the Company's research and development activities. At the end of the reporting period, the Company compares payments made to third party service providers to the estimated progress toward completion of the research or development objectives. Depending on the timing of payments to the service providers and the progress that the Company estimates has been made as a result of the service

provided, the Company may record net prepaid or accrued expense relating to these costs. 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 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 Measurements and Disclosures*" 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 September 30, 2021 and December 31, 2020, the Company had $252,912 and $213,119, respectively, 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 nine months ended September 30, 2021 and the year ended December 31, 2020.

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 common stock outstanding. Diluted EPS is calculated by adjusting weighted average common shares outstanding for the dilutive effect of common stock options and warrants. 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 ASU No. 2019-12, *Income Taxes (Topic 740)* 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 is 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.

In August 2018, the FASB issued ASU No. 2018-15, *Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*. We adopted this standard effective January 1, 2020. ASU No. 2018-15 requires that certain implementation costs for cloud computing arrangements are capitalized and amortized over the term of associated hosted cloud computing arrangement service and that capitalized implementation costs are classified in prepaid expenses and other assets. ASU No. 2018-15 also provides classification guidance on these implementation costs as well as additional quantitative and qualitative disclosures. The adoption of ASU No. 2018-15 did not have an effect 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: ASU No. 2018-19, ASU No. 2019-04 and No. ASU 2019-05 (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 for the Company, until fiscal year 2023. The Company does not expect that the adoption of ASU No. 2016-13 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, par value $0.001 per share (the "Common Stock"), having an aggregate offering price of up to $100,000 (the "Shares"), 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 September 30, 2021, the Company received proceeds of $4,056, net of issuance costs of $500.

In July 2020, the Company sold in a registered offering 4,000 shares of its common stock at a public offering price of $50.00. The Company received proceeds of approximately $186,974, net of issuance costs of $13,026 (with $34 recorded as a true up in the fourth quarter of 2020). Under the terms of the Underwriting Agreement entered into by the Company in connection with the July 2020 offering, certain stockholders of the Company granted the underwriters an option exercisable for thirty days to purchase up to an additional 600 shares of common stock at the public offering price less underwriting discounts and commissions, which was not exercised. The Company intends to use the net proceeds of the offering to fund ongoing clinical trials, commercialization preparation and for general corporate purposes.

In February 2020, the Company sold in a registered offering 2,300 shares of its common stock at a public offering price of $32.00 per share. The Company received proceeds of $68,811, net of issuance costs of $4,789. The Company used $9,024 of the proceeds to purchase and cancel 300 shares of common stock from BioXcel LLC.

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 United States will continue indefinitely, as agreed upon by the parties. These services are primarily for drug discovery, chemical, manufacturing and controls cost, and administrative support.

Service charges recorded under this agreement for the three and nine months ended September 30, 2021 and 2020 were comprised as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Research and development	$ 269	$ 270	$ 899	$ 926
General and administrative	72	44	166	44
Total	$ 341	$ 314	$ 1,065	$ 970

As of September 30, 2021, $154 related to these service charges is included in due to related parties in the Company's balance sheet.

Under the Services Agreement, the Company has an option, exercisable until March 12, 2023, 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. As of September 30, 2021, this option has not been exercised.

Note 6. 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 common stock outstanding. Diluted EPS is calculated by adjusting weighted average common shares outstanding for the dilutive effect of common stock options and warrants. 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. The calculations of basic and diluted net loss per share are as follows (in thousands, except per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Net loss (numerator)	$ (26,811)	$ (24,753)	$ (80,806)	$ (61,083)
Weighted average share, in thousands (denominator)	27,972	23,050	25,832	20,779
Basic and diluted net loss per share	$ (0.96)	$ (1.07)	$ (3.13)	$ (2.94)

Potentially dilutive securities outstanding consists solely of stock options. The Company had options outstanding to purchase 4,152 and 3,834 shares of common stock as of September 30, 2021 and 2020, respectively.

Note 7. Property and Equipment, net

Property and Equipment, net consisted of the following

	September 30, 2021	December 31, 2020
Computers and related equipment	$ 167	$ 260
Furniture	572	369
Leasehold improvements	1,123	650
Work in process	—	356
	1,862	1,635
Accumulated depreciation	(526)	(362)
	$ 1,336	$ 1,273

Depreciation expense was $76 and $47 for the three months ended September 30, 2021 and 2020, respectively, and $221 and $143 for the nine months ended September 30, 2021 and 2020, respectively.

Note 8. Accrued Expenses and Other Current Liabilities

Accrued expenses consist of the following:

	September 30, 2021	December 31, 2020
Research and development expenses	$ 4,694	$ 3,264
Accrued compensation and benefits	3,495	2,066
Accrued professional expenses	2,188	1,288
Accrued taxes	199	697
Other accrued expenses	140	154
	$ 10,716	$ 7,469

Other current liabilities as of September 30, 2021 and December 31, 2020 consists of $287 and $237, respectively, for the current portion of operating lease liabilities.

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 under the 2017 Plan immediately prior to the approval of the 2020 Plan by the Company’s stockholders, that 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. On January 1, 2021, the shares available for issuance under the 2020 Plan increased by 977 shares.

Options 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 September 30, 2021, there were 496 shares available to be granted under the 2020 Plan.

A summary of the status of the Company's stock option activity for the nine months ended September 30, 2021 is presented below (in thousands, except per share amounts):

	Number of Shares	Weighted Average Exercise Price per Share
Outstanding as of January 1, 2021	3,798	$ 16.15
Granted	800	$ 37.82
Forfeited	(159)	$ 42.26
Cancelled	(3)	$ 45.98
Exercised	(284)	$ 5.12
Outstanding as of September 30, 2021	4,152	$ 20.07
Options vested and exercisable as of September 30, 2021	2,609	$ 9.13

As of September 30, 2021, the intrinsic value of options outstanding was $66,583. 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.

The total intrinsic value of stock options exercised for the nine months ended September 30, 2021 and 2020 was $11,942 and $8,743, respectively.

The weighted average grant date fair value of options granted during the nine months ended September 30, 2021 and 2020 was $29.25 and $32.53, respectively.

The weighted average grant date fair value of options vested at September 30, 2021 was $6.74.

The weighted average remaining contractual life is 6.5 years for options exercisable as of September 30, 2021.

Stock-Based Compensation

The fair value of options granted during the nine months ended September 30, 2021 and 2020 was estimated using the Black-Scholes option-pricing model with the following assumptions.

	For the Nine Months Ended September 30, 2021	For the Nine Months Ended September 30, 2020
Expected Term	5.50 years - 6.25 years	5.50 years - 6.25 years
Expected stock price volatility	95.00 % - 98.00 %	79.02 % - 86.71 %
Risk-free rate of interest	0.96 % - 1.22 %	0.33 % - 2.34 %
Expected dividend	0.0 % - 0.0 %	0.0 % - 0.0 %

Prior to the Company's IPO, it did not have a history of market prices of its common stock and, as such, volatility was estimated using historical volatilities of similar public companies. In 2021, the Company began using a combination of the historical volatility of similar public companies and the limited historical information related to the Company's common stock. 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 based on the United States Treasury yield curve in effect at the time of grant, with maturities approximating the expected term of the stock options.

Unrecognized compensation expense related to unvested awards as of September 30, 2021 was $25,894 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 1.7 years.

Total stock-based compensation charges for the three and nine months ended September 30, 2021 and 2020 were comprised as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Research and development	$ 951	$ 2,203	$ 5,155	$ 3,587
General and administrative	3,935	3,065	12,064	4,413
Total	$ 4,886	$ 5,268	$ 17,219	$ 8,000

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 market value of a share on the first trading day or on the last trading day of the applicable offering period, whichever is lower. On January 1, 2021, the shares available for issuance under the 2020 ESPP increased by 244 shares. To date, no shares have been sold under the ESPP.

Note 10. Leases

In August 2018, the Company entered into an agreement to lease approximately 11,040 square feet of space on the 12th floor of the building located at 555 Long Wharf Drive, New Haven, Connecticut (the "12th Floor Lease) which was effective February 22, 2019. The 12th Floor Lease expires in February 2026.

In August 2020, the Company entered into an amendment to the 12th Floor Lease wherein the Company leased an additional 7,245 square feet of space on the 12th floor of the building located at 555 Long Wharf Drive, New Haven, Connecticut (the "12th Floor Lease Amendment"). The 12th Floor Lease Amendment expires in February 2026.

The future minimum annual lease payments under these operating leases as of September 30, 2021 are as follows:

Year ending December 31,	Amount
Remainder of 2021	89
2022	363
2023	372
2024	381
2025	391
Thereafter	64
Total lease payments	1,660
Less imputed interest	(193)
Total lease liability	1,467
Less current portion of lease liability	(287)
Long-term portion operating lease liability	$ 1,180

The current portion of the Company’s operating lease liability of $287 as of September 30, 2021 is included in other current liabilities on the balance sheet.

The Company recorded lease expense of $85 and $130 related to its operating lease right-of-use asset for the three months ended September 30, 2021 and 2020, respectively. The Company recorded lease expense of $280 and $237 related to its operating lease right-of-use asset for the nine months ended September 30, 2021 and 2020, respectively.

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

Note 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.

The Company received a demand letter pursuant to Section 220 of the Delaware General Corporation Law (“DGCL”) from a stockholder seeking disclosure of certain of the Company's records. The Company responded to those demands, stating its belief that the demand letter failed to fully comply with the requirements of Section 220 of the DGCL. On June 15, 2021, the stockholder filed a complaint in Delaware Chancery Court seeking to compel inspection of books and records pursuant to Section 220 of the DGCL. Pursuant to a negotiated settlement agreement, the matter was dismissed with prejudice on August 10, 2021.

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 Quarterly Report on Form 10-Q and the audited financial statements and related notes contained in our Annual Report on Form 10-K for the year ended December 31, 2020. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, such as information with respect to our plans and strategy for our business and expectations related to the clinical development of our product candidates, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Summary Risk Factors" and "Risk Factors" sections of this Quarterly Report, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis. 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 clinical stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. Our 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. We believe this differentiated approach has the potential to reduce the cost and time of drug development in diseases with a substantial unmet medical need.

Our two most advanced clinical development programs are BXCL501, an investigational proprietary, orally dissolving, thin film formulation of the adrenergic receptor agonist dexmedetomidine ("Dex"), for the treatment of agitation associated with psychiatric and neurological disorders, and BXCL701, 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.

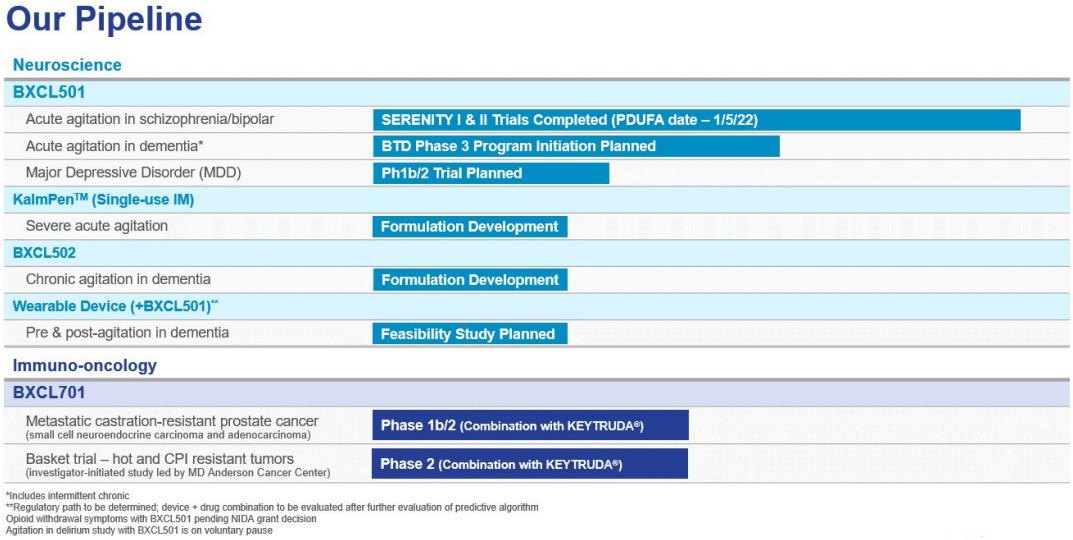
During the first quarter of 2020 the novel coronavirus disease, or COVID-19, was declared a pandemic and has continued to spread to multiple regions across the globe, including the United States and Europe. The pandemic and government measures taken in response have significantly impacted, both directly 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.

In 2020 and 2021, we took steps in line with guidance from the U.S. Centers for Disease Control and Prevention ("CDC") and the State of Connecticut to protect the health and safety of our employees and community. In particular, we implemented a work-from-home policy for all employees. We continue to assess the impact of the COVID-19 pandemic to best mitigate risk and continue business operations. Beginning late in the first quarter of 2021, and in line with State of Connecticut guidelines, we opened our office on a voluntary basis up to full capacity. In May 2021 the State of Connecticut lifted all business and office restrictions in the State. We have instituted a return to the office policy and continue to evaluate that policy.

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. Through September 30, 2021, we have not experienced any significant delays to our ongoing or planned clinical trials, except for challenges in accessing elderly care facilities and ICU settings. However, this could rapidly change.

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

BXCL501 is 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 and opioid withdrawal symptoms. BXCL501 is our most advanced neuroscience clinical program, currently being developed for the acute treatment of agitation related to schizophrenia, bipolar disorders, dementia and delirium, the symptoms associated with opioid withdrawal as well as for the treatment of major depressive disorder (MDD) in conjunction with the use of Selective Serotonin / Norepinephrine Reuptake Inhibitors (“SS/NRIs”). 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, dementia and opiate use disorder. 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

SERENITY I and SERENITY II

In May 2021, we announced the FDA had notified us that our NDA submission was accepted for filing. The FDA assigned a Prescription Drug User Fee Act (“PDUFA”) date of January 5, 2022. During the quarter we continued to address information requests from the FDA. We also have plans underway to submit a Marketing Authorization Application (“MAA”) to the European Medicines Agency (“EMA”) for BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders I & II in the first half of 2022.

TRANQUILITY

In January 2021, we announced topline results from the TRANQUILITY trial, a phase 1b/2 randomized, placebo-controlled, adaptive ascending dose-finding study that enrolled 54 patients with agitation related to dementia. Overall, BXCL501 was well tolerated and demonstrated statistically significant, clinically meaningful, rapid, and durable reductions in agitation with the 60 mcg dose as measured by multiple scales.

With positive results from TRANQUILITY, we have been advancing the BXCL501 dementia clinical program in close consultation with the FDA.

We held an end of Phase 2 meeting with the FDA in May 2021 to discuss the BXCL501 program for the acute treatment of agitation in dementia patients. Over the last several months we have had multiple interactions with the FDA, to further discuss development plans and align on key design features for the pivotal Phase 3 program. We currently plan to commence the BXCL501 Phase 3 program for the acute treatment of agitation in patients with Alzheimer's disease in the fourth quarter of 2021. Alzheimer's disease is the most prevalent type of dementia in the United States.

RELEASE

The RELEASE trial was a multicenter, randomized, double-blind, placebo-controlled, ascending dose Phase 1b/2 trial designed to evaluate the safety, pharmacokinetics, tolerability, and efficacy of escalating doses of BXCL501 versus placebo, following discontinuation of morphine maintenance in patients with opioid use disorder who are physically dependent on opioids.

BXCL501 was well tolerated, with no severe or serious adverse events reported across all doses evaluated. Although improvements were not observed in group mean total efficacy scores, the study showed numerical improvements in retention rates in multiple BXCL501 dose cohorts compared to the placebo.

We believe the favorable tolerability results observed in multiple dose regimens within the RELEASE trial provide valuable insights that support investigation across additional indications and treatment settings.

PLACIDITY

Agitation associated with delirium is a serious condition that affects patients in many hospital settings: ICUs, surgical and medical wards, and emergency departments. Currently, there are no FDA-approved medications for delirium or agitation associated with delirium.

On February 25, 2021, we announced initiation of the Phase 2 PLACIDITY trial of BXCL501. PLACIDITY enrollment was voluntarily paused to assess challenges posed in opening relevant clinical sites and enrolling delirium patients in ICU settings. These challenges include the burden COVID-19 has placed on the ICU. The PLACIDITY trial status has not changed.

PEDIATRIC STUDY

In June 2021, we initiated a global clinical trial designed to determine the safety and efficacy of BXCL501 in agitation associated with pediatric schizophrenia and bipolar disorder. The trial has been reviewed by the FDA to ensure we meet the Pediatric Equity Research Act ("PREA") requirements as well as the EMA to fulfill commitments to study the effects of BXCL501 in children, ages 10-17. The multisite double-blind placebo controlled parallel group trial will enroll patients with schizophrenia, schizoaffective disorder, bipolar I and bipolar II disorder. Similar to the SERENITY I and II trials, the primary endpoint is the change from baseline PEC total score at 2 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 sites are now being activated.

Major Depressive Disorder (MDD)

We have recently expanded the market potential of BXCL501 as an adjunctive treatment for Major Depressive Disorder ("MDD"). In October 2021, we held a pre-IND meeting with the FDA to discuss the clinical strategy to evaluate the use of BXCL501 in conjunction with patients receiving SS/NRIs. We are preparing to submit an IND to the FDA and expect to initiate the first clinical trial in the first half of 2022.

Additional Opportunities

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 suffering from post-traumatic stress disorder related to alcohol and substance abuse disorder. For the first time we will be investigating BXCL501 as a potential chronic treatment.

We continue to explore the use of a wearable device to measure early signs of agitation in subsets of patients who are part of BXCL501 clinical trials. Data is obtained by the wearable device, collected on other electronic devices such as phones, and transmitted to our service vendor for analysis. Feasibility studies demonstrated that patients were able to wear these devices and that these devices were able to transmit data to our service vendor. Along with our service vendor, we are establishing methods to use these data to construct algorithms designed to predict when a patient is becoming agitated. The goal of this program is to establish a predictive algorithm that allows providers to dose 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. There are additional neuropsychiatric disorders where agitation is a symptom that requires treatment.

We recently identified and initiated the development of a second neuropsychiatric drug candidate, BXCL502. 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 be a potent and selective antagonist of a G-protein coupled receptor ("GPCR") that affects serotonergic signaling in the brain. Our preclinical data and machine learning results suggests BXCL502 has potential to treat agitation and stress related neuropsychiatric symptoms in dementia. In previously published third party clinical trial data, daily administration of the API of BXCL502 demonstrated improvement in agitation using a well-established, clinically validated symptom scale. Formulation and clinical development planning are currently underway with BXCL502.

Commercial and Launch Readiness

We continue to advance our preparations for commercial and launch readiness if our NDA for BXCL501 is approved by the FDA. We are optimizing the design and recruitment of our sales force and market access and pricing strategy for the potential commercial launch of BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders I & II. This work will be a crucial foundation to launching additional potential follow-on indications, paving the way for our expanding neuroscience business.

During the third quarter of 2021, we fully launched our unbranded disease education campaign to promote awareness around the treatment of agitation in schizophrenia and bipolar disorders. In addition, we fully deployed our Medical Science Liaison and Medical Managed Care teams who continued to actively engage with healthcare professionals and payors to provide key insights to support our commercial strategy.

BXCL701 Immuno-Oncology Program

BXCL701 is a potential first-in-class, oral, small-molecule immunomodulator designed to stimulate the innate and acquired immune systems by inhibiting DPP 8/9 (dipeptidyl peptidases). DPP 8/9 behave as "checkpoints" of the innate immune system. 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 other checkpoint inhibitors and/or immune activating agents, may be able to convert immuno-resistant tumors to immuno-sensitive tumors (“cold” to “hot” tumors).

BXCL701 Clinical Trials

BXCL701 is currently being evaluated in two combination therapy clinical trials:

In April 2020, we announced the initiation of the Phase 2 efficacy portion of the Phase 1b/2 trial evaluating BXCL701 in combination with KEYTRUDA® (pembrolizumab, a PD-1 inhibitor) for small-cell neuroendocrine carcinoma (“SCNC”) / neuro endocrine prostate cancer (“NEPC”). The Phase 1b safety assessment of BXCL701 identified a split dose totaling 0.6 mg per day as the recommended dose when used in combination with KEYTRUDA.

In addition to the efficacy cohort in SCNC patients, in August 2020, we opened a separate cohort for adenocarcinoma, a subtype of metastatic castration resistant prostate cancer (“mCRPC”) patients who have failed taxane-based chemotherapy and up to two lines of second-generation androgen pathway blockers. The Phase 1b portion of this trial was presented at the Society for Immunotherapy of Cancer’s 35th Anniversary Annual Meeting in 2020, and an efficacy update was recently presented at the American Society of Clinical Oncology Genitourinary Symposium in February 2021.

In October 2021, we announced the SCNC / NEPC cohort of the ongoing Phase 1b/2 trial of BXCL701 and KEYTRUDA® in aggressive forms of prostate cancer met the protocol specified threshold to move to stage two. We expect to report updated data from the Phase 2 portion of this trial in SCNC or NEPC and adenocarcinoma or mCRPC cohorts in Q1 2022.

In May 2021, we announced the adenocarcinoma cohort of the ongoing Phase 1b/2 trial of BXCL701 and KEYTRUDA® in aggressive forms of prostate cancer met the protocol specified efficacy threshold to move to stage two. Data from the Phase 2 portion was presented at the European Society for Medical Oncology conference in September 2021. The trial is now continuing towards full enrollment.

The MD Anderson-led Phase 2 open-label basket trial is designed to evaluate the response rate of orally administered BXCL701, combined with KEYTRUDA, in two arms: Arm A is enrolling checkpoint naïve patients (where checkpoint therapy is indicated: “hot tumors”); and Arm B is enrolling patients who have progressed following checkpoint therapy alone. As of August 2020, the efficacy threshold had been met for both arms, allowing the trial to advance to completion. Preliminary data was presented at the June 2021 American Society of Clinical Oncology meeting. In the first half of 2022, we expect to present additional efficacy data from the trial.

Other Immuno-oncology Indications

In addition to CRPC and checkpoint inhibitor (“CPI”) 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 immuno-suppressive microenvironment is driven by the potential molecular and cellular targets of BXCL701 and where the single agent activity of approved immune checkpoint inhibitors is limited. In January 2021, we announced that BXCL701 was granted Orphan Drug Designation for the treatment of soft tissue sarcoma.

In addition, we believe BXCL701, if successfully developed and approved, may provide a platform for combination with immunotherapy modalities that go beyond currently approved immune checkpoint agents targeting

the PD-1/PD-L1 axis. Following our proof-of-concept trials, we plan to conduct clinical trials covering a broad range of additional combinations with other immunotherapy agents, including:

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

Recent Research Findings

On November 4, 2021, the *Journal of Immunotherapy of Cancer* reported data findings suggesting BXCL701 may enhance immunotherapy efficacy in 'cold' tumor types such as pancreatic cancer. These findings also highlighted the potential importance of natural killer (NK) cells along with T cells in regulating pancreatic cancer tumor growth.

Other Product Candidates

Neuroscience Program

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.

Immuno-oncology Program

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 has permitted us to select a potential pipeline of candidates based on our ability to alter the tumor micro-environment and the potential to address relevant unmet medical needs for various tumor types.

Finally, we continually leverage the artificial intelligence platform 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 NEPC and CRPC.

Intellectual Property

Our policy is to protect and enhance the proprietary technologies, inventions, and improvements that are commercially important to our business by filing patent applications in the United States and other jurisdictions related to our proprietary technology, inventions, improvements, and product candidates. We also rely on trademarks, trade secrets, and know-how relating to our proprietary technologies and product candidates, continuing innovation, and in-licensing technology and products. This reliance is expected to develop, 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 October 18, 2021, our patent portfolio included 5 Patent Cooperation Treaty (PCT) applications, 11 U.S. utility applications, 1 issued U.S. utility patent, 13 U.S. provisional patent applications, 77 pending non-U.S. applications, 8 allowed or granted non-U.S. patents, 1 design patent application, which is a U.S. design application, and 34 allowed or registered design patents. 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. We plan to list the U.S. patent in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, or Orange Book. We have filed applications in the core patent family protecting BXCL501 in the United States, 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 United States, Europe and Japan directed to methods of treating insomnia using sublingual Dex. We expect that patents issuing from these applications, if any, will expire no earlier than 2035. We also have applications filed in 15 regions, including the United States, Europe, Japan, and China, directed to methods of treating agitation. We expect that patents issuing from these applications, if any, will expire no earlier than 2037. We have 1 U.S. application and 1 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 United States 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 United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. Depending upon the timing, duration, and specifics of FDA approval of our product candidates, a 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, referred to as 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 IND, and the submission date of a NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension, and the application for extension must be made prior to patent expiration. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration date, depending on the expected length of clinical trials and other factors involved in the submission of the relevant NDA.

The patent positions of companies such as ours are generally uncertain and involve complex legal and factual questions. No consistent policy regarding the scope of claims allowable in patents in the field of method of use patents or reformulation patents has emerged in the United States. Relevant patent laws and their interpretation outside of the United States are also uncertain. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our technology or product candidates and enforce the patent rights that we license, and also could affect the value of such intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell, or importing products that infringe our intellectual property will depend in part on our success in obtaining and enforcing patent claims that cover our technology, inventions, and improvements. With

respect to both licensed and company owned intellectual property, we cannot guarantee that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications we may file in the future, nor can we be sure that any patents that may be granted to us in the future will be commercially useful in protecting our products, the methods of use, or the manufacture of those products. Patent and other intellectual property rights in the pharmaceutical and biotechnology space are evolving and involve many risks and uncertainties. For example, third parties may have blocking patents that could be used to prevent us from commercializing our product candidates and practicing our proprietary technology, and the issued patents that we in-license and those that may issue in the future may be challenged, invalidated, or circumvented, which could limit our ability to stop competitors from marketing related products or could limit the term of patent protection that otherwise may exist for our product candidates. In addition, the scope of the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies 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 Generally Accepted Accounting Principles in the United States of America ("GAAP"). All amounts are presented in thousands.

Components of Our Results of Operations

Revenues

We have not recognized any revenue since inception.

Operating Costs and Expenses

Research and Development

Our research and development expenses reflect costs incurred for the research and development of our clinical and pre-clinical product candidates. 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 pre-clinical and clinical trial materials and lab supplies, and depreciation and other expenses.

We expense research and development costs to operations as incurred.

Our research and development costs by program for the three and nine months ended September 30, 2021 and 2020 were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Direct external costs				
BXCL501	$ 2,652	$ 9,114	$ 12,613	$ 30,524
BXCL701	3,312	1,948	8,520	4,917
Other research and development programs	564	181	1,231	538
Total direct external costs	6,528	11,243	22,364	35,979
Internal personnel costs	4,660	4,393	16,080	9,067
Sub-total direct costs	11,188	15,636	38,444	45,046
Indirect costs and overhead	772	681	2,101	1,549
Research and development tax credit	(27)	—	(362)	—
Total research and development expenses	$ 11,933	$ 16,317	$ 40,183	$ 46,595

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 and insurance charges.

We expect that our general and administrative expenses will increase to enable us to support our operations as we continue to 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 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 in this Quarterly Report on Form 10-Q.

Summary of Results of Operations

Comparison of the Three Months Ended September 30, 2021 and 2020

Revenues

We have not recognized any revenues since inception.

Research and Development Expense

Research and development expenses for the three months ended September 30, 2021 and 2020 were $11,933 and $16,317, respectively. Research and development expenses for the three months ended September 30, 2021 and 2020 were comprised as follows:

	Three Months Ended September 30,			
	2021	2020	Change	% Change
Personnel and related costs	$ 3,710	$ 2,190	$ 1,520	69 %
Non-cash stock-based compensation	951	2,203	(1,252)	(57)%
Professional fees	2,856	2,269	587	26 %
Clinical trials expense	3,483	8,479	(4,996)	(59)%
Chemical, manufacturing and controls cost ("CMC")	353	859	(506)	(59)%
Travel and other costs	607	317	290	91 %
Research and development tax credit	(27)	—	(27)	(100)%
Total research and development expenses	$ 11,933	$ 16,317	$ (4,384)	(27)%

The decrease of $4,384 for the three months ended September 30, 2021 is primarily attributable to:

- Decreased non-cash stock-based compensation due to the reversal of expense related to forfeitures during the period.
- Decreased clinical trial expense primarily related to decreased costs related to our SERENITY I and II, TRANQUILTY, RELEASE and BXCL501 bioavailability clinical trials, partially offset by increased costs related to our BXCL701 prostate cancer clinical trial.
- Decreased CMC costs largely related to decreased BXCL501 manufacturing costs.

These decreases were partially offset by:

- Increased personnel costs related to our efforts to enlarge our clinical team as we expand our clinical programs and in preparation of the potential commercial launch of BXCL501 in the U.S.
- Increased professional fees primarily due to increased fees related to toxicology studies and consulting fees related to BXCL501.
- Increased travel and other costs to support the growing clinical team as we expand our clinical programs.

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 September 30, 2021 and 2020 were $14,879 and $8,451, respectively. General and administrative expenses for the three months ended September 30, 2021 and 2020 were comprised as follows:

	Three Months Ended September 30,			
	2021	2020	Change	% Change
Personnel and related costs	$ 2,702	$ 1,022	$ 1,680	164 %
Non-cash stock-based compensation	3,935	3,065	870	28 %
Professional fees	2,575	1,361	1,214	89 %
Commercial	4,299	963	3,336	346 %
Insurance	560	442	118	27 %
Travel and other costs	808	1,598	(790)	(49)%
Total general and administrative expenses	$ 14,879	$ 8,451	$ 6,428	76 %

The increase of $6,428 for the three months ended September 30, 2021 is primarily attributable to:

- Increased personnel costs due to our continuing efforts to expand our teams in preparation of the potential commercial launch of BXCL501 in the U.S.
- Increased non-cash stock-based compensation primarily resulting from an increase in personnel.
- Increased professional fees due to the expanding growth of our operations and was primarily related to increased corporate legal fees and increased consulting costs related to our preparation for the potential commercial launch of BXCL501 in the U.S.
- Increased commercial related fees primarily related to our unbranded agitation awareness campaigns, planned BXCL501 marketing campaigns and overall preparation for the potential commercial launch of BXCL501 in the U.S.
- Increased insurance costs primarily related to an increase in Director and Officer liability premiums.

These increases were partially offset by:

- Decreased travel and other costs. An e-mail-based wire fraud incident in September 2020, which led to the misappropriation of approximately $1,927. Of this amount, $774 was subsequently recovered. As such, $1,153 was included in travel and other costs in 2020. This was partially offset by an increase in travel and other overhead expense to support the growing company and preparation for the potential commercial launch of BXCL501 in the US.

Comparison of the Nine Months Ended September 30, 2021 and 2020

Revenues

We have not recognized any revenues since inception.

Research and Development Expense

Research and development expenses for the nine months ended September 30, 2021 and 2020 were $40,183 and $46,595, respectively. Research and development expenses for the nine months ended September 30, 2021 and 2020 were comprised as follows:

	Nine Months Ended September 30,			
	2021	2020	Change	% Change
Personnel and related costs	$ 10,924	$ 5,480	$ 5,444	99 %
Non-cash stock-based compensation	5,155	3,587	1,568	44 %
Professional fees	9,413	5,443	3,970	73 %
Clinical trials expense	10,591	28,379	(17,788)	(63)%
Chemical, manufacturing and controls cost ("CMC")	3,046	2,859	187	7 %
Travel and other costs	1,416	847	569	67 %
Research and development tax credit	(362)	—	(362)	(100)%
Total research and development expenses	$ 40,183	$ 46,595	$ (6,412)	(14)%

The decrease of $6,412 for the nine months ended September 30, 2021 is primarily attributable to:

- Decreased clinical trial expense primarily related to decreased costs related to our SERENITY I and II, TRANQUILTY, RELEASE and BXCL501 bioavailability clinical trials, partially offset by increased costs related to our BXCL701 prostate cancer clinical trial.

This decrease was partially offset by:

- Increased personnel costs related to our efforts to enlarge our clinical team as we expand our clinical programs and in preparation of the potential commercial launch of BXCL501 in the U.S.
- Increased non-cash stock-based compensation primarily resulting from an increase in personnel.
- Increased professional fees primarily due to increased fees related to toxicology studies as well as increased regulatory and consulting fees related to BXCL501.
- Increased travel and other costs to support the growing clinical team as we expand our clinical programs.

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 nine months ended September 30, 2021 and 2020 were $40,621 and $14,605, respectively. General and administrative expenses for the nine months ended September 30, 2021 and 2020 were comprised as follows:

	Nine Months Ended September 30,			
	2021	2020	Change	% Change
Personnel and related costs	$ 7,297	$ 2,233	$ 5,064	227 %
Non-cash stock-based compensation	12,064	4,413	7,651	173 %
Professional fees	8,196	3,652	4,544	124 %
Commercial	9,865	1,077	8,788	816 %
Insurance	1,578	1,170	408	35 %
Travel and other costs	1,621	2,060	(439)	(21)%
Total general and administrative expenses	$ 40,621	$ 14,605	$ 26,016	178 %

The increase of $26,016 for the nine months ended September 30, 2021 is primarily attributable to:

- Increased personnel costs due to our continuing efforts to expand our teams in preparation of the potential commercial launch of BXCL501 in the U.S.
- Increased non-cash stock-based compensation primarily resulting from an increase in personnel.
- Increased professional fees due to the expanding growth of our operations and increased corporate and intellectual property legal fees and increased consulting costs related to our preparation for the potential commercial launch of BXCL501 in the U.S.
- Increased commercial related fees primarily related to our unbranded agitation awareness campaigns, planned BXCL501 marketing campaigns and overall preparation for the potential commercial launch of BXCL501 in the U.S.
- Increased insurance costs primarily related to an increase in Director and Officer liability premiums.

These increases were partially offset by:

- Decreased travel and other costs. An e-mail-based wire fraud incident in September 2020, which led to the misappropriation of approximately $1,927. Of this amount, $774 was subsequently recovered. As such, $1,153 was included in travel and other costs in 2020. This was partially offset by an increase in travel and other overhead expense to support the growing company and preparation for the potential commercial launch of BXCL501 in the US.

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 September 30, 2021, we had cash and cash equivalents of $252,912, working capital of $242,333 and stockholders' equity of $245,556. We incurred losses of approximately $26,811 and $24,753 for the three months ended September 30, 2021 and 2020, respectively, and losses of $80,806 and $61,083 for the nine months ended September 30, 2021 and 2020, 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 September 30, 2021 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 outbreak of COVID-19, or other pandemic, epidemic or outbreak of an infectious disease may materially and adversely impact our business, including our preclinical studies and clinical trials." in 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 by BioXcel LLC, and from proceeds from the sale of equity securities. Through September 30, 2021, we have received approximately $459,856 in aggregate gross proceeds from stock issuances including our initial public offering, private placements of our common stock, and registered offerings of our common stock and Open Market Sale Agreements ("ATM Program"). 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 June 2021, we sold in a registered offering 3,155 shares of its common stock at a public offering price of $31.70 per share. We received proceeds of $96,937, net of issuance costs of $3,076.

In May 2021, we entered into an Open Market Sale Agreement (the "Sale Agreement") with Jefferies LLC ("Jeffries") pursuant to which the Company could offer and sell shares if its common stock, par value $0.001 per share (the "Common Stock"), having an aggregate offering price of up to $100,000 (the "Shares"), from time to time, through an "at the market offering" program under which Jefferies will act as sale agent. We sold 124 shares under the Sale Agreement in June 2021. As of September 30, 2021, we received proceeds of $4,056, net of issuance costs of $500.

In July 2020, we sold in a registered offering 4,000 shares of our common stock at a public offering price of $50.00 per share. We received proceeds of $186,974, net of issuance costs of $13,026 (with $34 recorded as a true up in the fourth quarter of 2020).

In February 2020, we sold in a registered offering 2,300 shares of our common stock at a public offering price of $32.00 per share. We received proceeds of $68,811, net of issuance costs of $4,789.

Cash Flows

(in thousands)	Nine Months Ended September 30, 2021	2020
Cash provided by (used in) in thousands:		
Operating activities	$ (62,221)	$ (46,078)
Investing activities	(433)	(46)
Financing activities	102,447	247,126

Operating Activities

Net cash used in operating activities for the nine months ended September 30, 2021 was $62,221, which was primarily attributable to our net loss of $80,806, partially offset by $17,219 in non-cash stock-based compensation and $221 of depreciation and amortization and a $2,807 increase in accounts payable, accrued expenses and other liabilities.

Net cash used in operating activities for the nine months ended September 30, 2020 was $46,078 which was primarily attributable to our net loss of $61,083, partially offset by $8,000 in non-cash stock-based compensation and $143 of depreciation and amortization and a $7,830 increase in accounts payable, accrued expenses and other liabilities.

Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2021 was $433 and was attributable to the purchase of equipment and leasehold improvements.

Net cash used in investing activities for the nine months ended September 30, 2020 was $46 and was attributable to the purchase of equipment and leasehold improvements.

Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2021 was $102,447 and was attributable to $96,937 in net proceeds from the issuance of common stock in our June 2021 public offering, $4,056 in net proceeds from the sale of common stock under our Sale Agreement in June 2020 and $1,454 in proceeds from the exercise of stock options.

Net cash provided by financing activities for the nine months ended September 30, 2020 was $247,126 and was primarily attributable to $255,819 in net proceeds from the issuance of common stock in our February 2020 and July 2020 public offerings and $331 in proceeds from the exercise of stock options. This amount was partially offset by $9,024 used for the purchase and cancellation of 300 shares of common stock owned by BioXcel LLC.

Operating Capital and Capital Expenditure Requirements

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

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

- continue our clinical development of BXCL501 and BXCL701;
- conduct additional research and development with our product candidates;
- seek to identify, acquire, 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 efforts;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;

- ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any product candidates for which we may obtain regulatory approval; and
- continue to operate as a public company.

We believe that our existing cash and cash equivalents as of September 30, 2021 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. We expect that we will need to obtain substantial additional funding in order to fund our 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 BXCL501, BXCL701 or other product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to BXCL501, BXCL701 or other product candidates that we otherwise would seek to develop or commercialize ourselves.

Off-Balance Sheet Arrangements

As of September 30, 2021, we did not have any off-balance sheet arrangements as defined under SEC rules.

Contractual Obligations

There have been no material changes to our contractual obligations as disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020.

Critical Accounting Policies

Our critical accounting policies and estimates are described in "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies " in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020. We have reviewed and determined that those critical accounting policies and estimates remain our critical accounting policies and estimates as of and for the nine months ended September 30, 2021. No changes were made to our critical accounting policies during the period presented.

Recent 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.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk. As of September 30, 2021, we had $252,912 of cash and cash equivalents. Our cash and cash equivalents is 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 nine months ended September 30, 2021 and 2020, respectively.

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

Capital Market Risk. We currently have no product revenues and depend on funds raised through other sources. One source of funding is through 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 September 30, 2021. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the rules and forms of the Securities and Exchange Commission ("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 September 30, 2021, 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 other 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 September 30, 2021 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, 2020. The occurrence of any of the events or circumstances described below or other adverse events could have a material adverse effect on our business, results of operations and financial condition and could cause the trading price of our common stock to decline. Additional risks or uncertainties not presently known to us or that we currently deem immaterial may also harm our business.

Risks Related to Financial Position and Need for Additional Capital

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

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

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

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

Since our inception, we have incurred significant operating losses. Our net loss was $80.8 million and $61.1 million for the nine months ended September 30, 2021 and 2020 respectively. As of September 30, 2021, we had stockholders' equity of $245.6 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. None of our product candidates have been approved for marketing in the United States, or in any other jurisdiction, and may never receive such approval. It could be several years, if ever, before we have a commercialized product that generates significant revenues. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

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

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing clinical trials of our product candidates, developing commercial scale manufacturing processes, obtaining

marketing approval, manufacturing, marketing and selling any current and future product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate sufficient revenue to achieve profitability.

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

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

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

We anticipate that our expenses will increase substantially if and as we continue to develop and conduct clinical trials with respect to BXCL501, BXCL701, BXCL502 and our other product candidates; seek to identify and develop additional product candidates; acquire or in-license other product candidates or technologies; seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any; require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization; maintain, expand and protect our intellectual property portfolio; hire and retain additional personnel, such as clinical, quality control and scientific personnel; add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and add equipment and physical infrastructure to support our research and development programs.

We expect that our cash and cash equivalents as of September 30, 2021 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 in order to 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 candidate or any future product candidates that we may develop for one or more indications, we may not be able to enter into a collaboration for any of our product candidates for such indications on suitable terms, on a timely basis or at all. In any event, the net proceeds of our 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 BXCL501, BXCL701, BXCL502 and our other product candidates;
- our ability to enter into and the terms and timing of any collaborations, licensing agreements or other arrangements;
- the costs, timing and outcome of seeking regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- our headcount growth and associated costs as we expand our research and development as well as establish a commercial infrastructure;
- revenue received from commercial sales, if any, of our current and future product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the number of future product candidates that we pursue and their development requirements;
- changes in regulatory policies or laws that may affect our operations;
- changes in physician acceptance or medical society recommendations that may affect commercial efforts;
- the costs of acquiring potential new product candidates or technology; and
- the costs of operating as a public company.

Risks Related to the Discovery and Development of Product Candidates

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

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

In the near term, we are dependent on the success of BXCL501, BXCL701 and BXCL502. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize BXCL501, BXCL701, BXCL502 and our other product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.

We currently do not have any products that have received regulatory approval and may never be able to develop marketable product candidates. We are investing a significant portion of our efforts and financial resources in the development of BXCL501 and BXCL701, as well as our other product candidates, including BXCL502. Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop, obtain marketing approval for and successfully commercialize product candidates in one or more disease indications.

The success of BXCL501, BXCL701, BXCL502 and our other product candidates will depend on several factors, including the following:

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

Many of these factors are beyond our control, including the results of clinical trials, the time required for the FDA or any comparable foreign regulatory authorities to review any regulatory submissions we may make, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive marketing approval for and successfully commercialize BXCL501, BXCL701, BXCL502 and our other product candidates, on our own or with any future collaborator, or experience delays as a result of any of these factors or otherwise, our business could be substantially harmed.

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

From time to time, we may publicly disclose top-line or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change, 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 have not obtained regulatory approval for any product candidate and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

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

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an New Drug Application, or NDA, or other submission or to obtain regulatory approval in the United States or elsewhere; the FDA or comparable foreign regulatory authorities may disagree that our changes to branded reference drugs meet the criteria for the 505(b)(2) regulatory pathway or foreign regulatory pathways;

- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

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

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

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

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

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

Before obtaining marketing approval from the FDA or other comparable foreign regulatory authorities for the sale of our product candidates, we must complete preclinical development and extensive clinical trials to demonstrate the safety and efficacy of our product candidates. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. Although we are planning for certain clinical trials relating to BXCL501, BXCL701, BXCL502 and our other product candidates, there can be no assurance that the FDA will accept our proposed trial designs. We may experience delays in our clinical trials and we do not know whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays related to:

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

- reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- diversion of healthcare resources to combat epidemics, such as the COVID-19 pandemic;
- obtaining institutional review board, or IRB, approval at each site, or independent ethics committee, or IEC, approval at any sites outside the United States;
- dependence on the needs and timing of third party collaborators;
- changes to clinical trial protocols;
- recruiting suitable patients to participate in a trial in a timely manner and in sufficient numbers;
- clinical sites deviating from trial protocol or dropping out of a trial;
- addressing patient safety concerns that arise during the course of a trial;
- having patients complete a trial or return for post-treatment follow-up;
- imposition of a clinical hold by regulatory authorities, including as a result of unforeseen safety issues or side effects or failure of trial sites to adhere to regulatory requirements;
- the occurrence of serious adverse events in trials of the same class of agents conducted by other companies or institutions;
- subjects choosing an alternative treatment for the indications for which we are developing our product candidates, or participating in competing trials;
- adding a sufficient number of clinical trial sites;
- manufacturing sufficient quantities of a product candidate for use in clinical trials;
- lack of adequate funding to continue the clinical trial;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practice, or cGMP, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process that may be necessary or desired;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, good clinical practice, or GCP, or other regulatory requirements; or
- third-party contractors not performing data collection or analysis in a timely or accurate manner; or third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications.

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 has placed on the ICU. 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. We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs (or IECs) of the

institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and, while we have agreements governing their committed activities, we have limited influence over their actual performance.

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

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

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

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

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

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by

the FDA or other comparable foreign authorities. The clinical evaluation of BXCL501, BXCL701, BXCL502 and our other product candidates in patients, in many cases, is still in the early stages 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 for the treatment of emergent Neuroendocrine Prostate Cancer, one patient experienced acidosis with a fatal outcome. Although the clinical investigator could not determine that the fatality was related to treatment with BXCL701, it is possible that BXCL701 could be tied to unacceptable side effects in the future. In such an event, we, the FDA, the IRBs (or IECs) at the institutions in which our studies are conducted, or the DSMB could suspend or terminate our clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. For example, the FDA placed Point Therapeutics, Inc.'s IND for BXCL701 on clinical hold following an increase in observed mortality in patients receiving BXCL701 in a Phase 3 trial in patients with non-small cell lung cancer. Though we believe that this result was caused by, among other things, an imbalance in the disease severity of patients enrolled in the active arm of the clinical trial, there is no guarantee that excess mortality will not be observed in future clinical studies. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the clinical trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

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

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

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

BioXcel 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 EvolverAI to create a pipeline of neuroscience and immuno-oncology product candidates for patients whose diseases have not been adequately addressed to date by other approaches and to design and conduct efficient clinical trials with a higher likelihood of success. While we believe that applying EvolverAI to create medicines for defined patient populations may potentially enable drug research and clinical development that is more efficient than conventional drug research and development, our approach is both novel and unproven. Because our approach is both novel and unproven, the cost and time needed to develop our product candidates is difficult to predict,

and our efforts may not result in the discovery and development of commercially viable medicines. We may also be incorrect about the effects of our product candidates on the diseases of our defined patient populations, which may limit the utility of our approach or the perception of the utility of our approach. Furthermore, our estimates of our defined patient populations available for study and treatment may be lower than expected, which could adversely affect our ability to conduct clinical trials and may also adversely affect the size of any market for medicines we may successfully commercialize. Our approach may not result in time savings, higher success rates or reduced costs as we expect it to, and if not, we may not attract collaborators or develop new drugs as quickly or cost effectively as expected and therefore we may not be able to commercialize our approach as originally expected.

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

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

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

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

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

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

We intend to seek FDA approval through the 505(b)(2) regulatory pathway for certain of our product candidates, including BXCL501. The Hatch-Waxman Act added Section 505(b)(2) to the Federal Food, Drug and Cosmetic Act, or 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. 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 our product candidates. 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. 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 (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 (2017/746 or 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 will, however, only become applicable in May 2022.

The regulation of companion diagnostics will be subject to further requirements as of the entry into force of the IVDR which 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 authorities or the EMA.

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

Even if we obtain regulatory approval for BXCL501, BXCL701, BXCL502 or any product candidate, we will still face extensive and ongoing regulatory requirements and obligations and any product candidates, if approved, may face future development and regulatory difficulties.

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

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

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

- restrictions on manufacturing such products;

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

Further, the FDA's policies may change, and additional government regulations may be enacted that could impose extensive and ongoing regulatory requirements and obligations on any product candidate for which we obtain marketing approval. For instance, the EU has adopted Regulation (EU) No 536/2014 (Clinical Trials Regulation, or CTR) in April 2014, which is expected to come into application in 2022. The CTR will be directly applicable in all the EU member states, repealing the current Clinical Trials Directive. Conduct of all clinical trials performed in the European Union will continue to be bound by currently applicable provisions until the new CTR becomes applicable. The extent to which ongoing clinical trials will be governed by the CTR will depend on when the CTR becomes applicable and on the duration of the individual clinical trial. If a clinical trial continues for more than three years from the day on which the CTR becomes applicable the CTR will at that time begin to apply to the clinical trial. The CTR harmonizes the assessment and supervision processes for clinical trials throughout the EU via a Clinical Trials Information System, which will notably contain a centralized EU portal and database. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

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

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

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 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 ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA 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, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the COVID-19 pandemic, on March 10, 2020 the FDA announced its intention to postpone most inspections of foreign manufacturing facilities and products, and on March 18, 2020 the FDA temporarily postponed routine surveillance inspections of domestic manufacturing facilities. Subsequently, on July 10, 2020, the FDA announced its intention to resume certain on-site inspections of domestic manufacturing facilities subject to a risk-based prioritization system. The FDA intends to use this risk-based assessment system to identify the categories of regulatory activity that can occur within a given geographic area, ranging from mission critical inspections to resumption of all regulatory activities. 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. According to the guidance, the FDA intends to request such remote interactive evaluations in situations where an in-person inspection would not be prioritized, deemed mission-critical, or where direct inspection is otherwise limited by travel restrictions, but where the FDA determines that remote evaluation would be appropriate. Regulatory authorities outside the United States may adopt 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 be subject to extensive regulations outside the United States and may not obtain marketing approvals for products in Europe and other jurisdictions.

In addition to regulations in the United States, should we or our collaborators pursue marketing approvals for BXCL501, BXCL701, 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 BXCL501, BXCL701, BXCL502 and our other product candidates in Europe and other jurisdictions outside the United States with collaborative partners. The time and process required to obtain regulatory approvals and reimbursement in Europe and other jurisdictions may be different from those in the

United States regulatory and approval in one jurisdiction does not ensure approvals in any other jurisdiction; however, negative regulatory decisions in any jurisdiction may have a negative impact on the regulatory process in other jurisdictions.

Following a national referendum and enactment of legislation by the government of the United Kingdom, or the UK, the United Kingdom withdrew from the European Union, or Brexit, on January 31, 2020 and entered into a transition period during which it was essentially treated as a member state of the EU and the regulatory regime remained the same across the United Kingdom and the European Union. Since January 1, 2021, the United Kingdom operates under a distinct regulatory regime. EU pharmaceutical laws now only apply to the UK in respect of Northern Ireland (as laid out in the Protocol on Ireland and Northern Ireland, including but not limited to marketing authorization applications). EU laws which have been transposed into UK law through secondary legislation continue to be applicable as "retained EU law". As there is no general power to amend this "retained EU law" the UK government has introduced a new Medicines and Medical Devices Bill which seeks to address regulatory gaps through implementing regulations and delegated powers covering the fields of human medicines, clinical trials of human medicines, veterinary medicines and medical devices. The purpose of the bill is to enable the existing UK regulatory frameworks to be updated. Although regulatory authorities in the UK have indicated in the bill that new UK rules will closely align with EU laws, detailed proposals are yet to be published. The bill has not been formally enacted and had its final reading in the House of Lords on January 21, 2021. As the House of Lords proposed amendments to the draft legislation, the bill will go back to the other house of parliament for debate. There is no set time period for consideration of amendments and the bill will only proceed to be enacted as law once the draft bill has been agreed by both houses and royal assent has been obtained. The draft bill currently contemplates that the provisions will come into effect immediately upon enactment or otherwise within two months thereafter, with the exception of certain provisions on enforcement and disclosure, which are subject to further regulation Significant political and economic uncertainty therefore remains about how much the relationship between the UK and EU will differ as a result of the UK's withdrawal. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

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

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

- the federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug for which payment may be made under a federal health care program, such as Medicare or Medicaid. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it to have committed a violation;

- false claims laws prohibit anyone from knowingly and willfully presenting or causing to be presented for payment to third-party payers, including government payers, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. Cases have been brought under false claims laws alleging that off-label promotion of pharmaceutical products or the provision of kickbacks has resulted in the submission of false claims to governmental health care programs. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute

constitutes a false or fraudulent claim for purposes of the false claims laws. Further, private individuals have the ability to bring actions on behalf of the government under the federal False Claims Act;

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

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

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

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

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; 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 healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and imprisonment, any of which could adversely affect our ability to market our products and adversely impact our financial results.

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

Our inability to retain sufficient clinical trial liability insurance at an acceptable cost to protect against potential liability claims could prevent or inhibit our ability to conduct clinical trials for product candidates we develop. We may be unable to obtain appropriate levels of such insurance. Even if we do secure clinical trial liability insurance for our

programs, we may not be able to achieve sufficient levels of such insurance. Any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. We expect we will supplement our clinical trial coverage with product liability coverage in connection with the commercial launch of BXCL501, BXCL701, BXCL502 or other product candidates we develop in the future; however, we may be unable to obtain such increased coverage on acceptable terms or at all. If we are found liable in a clinical trial lawsuit or a product liability lawsuit in the future, we will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Risks Related to Commercialization of Our Product Candidates

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

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

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

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

We have been granted Orphan Drug Designation for BXCL701 for the treatment of pancreatic cancer, melanoma, 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 United States and Europe, may designate drugs intended for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the United States provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances the applicable exclusivity period is ten years in Europe. The European exclusivity period can be reduced to six years if, 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 indications or for our other product candidates. There can be no assurances that we will be able to obtain such designations.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain or maintain orphan drug exclusivity for that product candidate. We may not be the first to obtain marketing approval of any product candidate for which we have obtained orphan drug designation for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products, and it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity expires. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties may be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care or the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process, nor does it prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation.

If we are unable to develop satisfactory sales and marketing capabilities, we may not succeed in commercializing BXCL501, BXCL701, BXCL502 or any other product candidate.

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

We operate in a highly competitive and rapidly changing industry.

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

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

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

Established biopharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving FDA approval for or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

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

Even if we obtain regulatory approvals to commercialize BXCL501, BXCL701, BXCL502 or our other product candidates, our product candidates may not be accepted by physicians or the medical community in general.

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

- our demonstration of the clinical efficacy and safety of BXCL501, BXCL701, BXCL502 and our other product candidates;
- timing of market approval and commercial launch of BXCL501, BXCL701, BXCL502 and our other product candidates;
- the clinical indication(s) for which BXCL501, BXCL701, BXCL502 and our other product candidates are approved;
- product label and package insert requirements;
- advantages and disadvantages of our product candidates compared to existing therapies;
- continued interest in and growth of the market for anti-cancer or anti-agitation drugs;
- strength of sales, marketing, and distribution support;
- product pricing in absolute terms and relative to alternative treatments;
- future changes in health care laws, regulations, and medical policies; and
- availability of reimbursement codes and coverage in select jurisdictions, and future changes to reimbursement policies of government and third-party payors.

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

Other third-party payors are increasingly challenging the prices charged for medical products and services. It will be time consuming and expensive for us to go through the process of seeking coverage and reimbursement from

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

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

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

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

New laws, regulations and judicial decisions, or new interpretations of existing laws, regulations and decisions, that relate to healthcare availability, methods of delivery or payment for products and services, or sales, marketing or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons, including policies advanced by the current executive administration in the United States, new healthcare legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the United States and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

For example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the ACA, has substantially changed the way healthcare is financed by both government health plans and private insurers, and significantly impacts the pharmaceutical industry. The ACA 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. For example, the Tax Cuts and Jobs Act, or the Tax Act, was enacted, which, among other things, removed penalties for not complying with the ACA's individual mandate to carry health insurance. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and

inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. The U.S. Supreme Court held that Texas and other plaintiff states do not have standing to challenge the constitutionality of the Affordable Care Act's individual mandate. It is also unclear how other efforts, if any, to challenge, repeal or replace the ACA will impact the law, or our business or financial condition.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. These changes include the Budget Control Act of 2011, which resulted in aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030, with the exception of a temporary suspension from May 1, 2020 through March 31, 2021, unless additional Congressional action is taken, as well as the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

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

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

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 November 5, 2021, BioXcel LLC owned approximately 31.0% of the economic interest and voting power of our outstanding common stock. 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 with BioXcel LLC, as amended and restated, or the Services Agreement, and the Amended and Restated Asset Contribution Agreement, or the Contribution Agreement, that we have entered into with BioXcel LLC have not been negotiated on behalf of BioXcel LLC by persons consisting solely of disinterested BioXcel 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 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. The Company has negotiated the Services Agreement with BioXcel LLC pursuant to which BioXcel LLC shall perform product identification and related services for us utilizing EvolverAI. Under the Services Agreement, the Company has an option, exercisable until March 12, 2023, 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 milestones shall not exceed $10 million in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestones shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30 million in the aggregate. BioXcel LLC shall continue to make such product identification and related services available to us until at least September 30, 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 option for first negotiation shall be valid for a period of five years from the date of our IPO. If our rights and access to BioXcel LLC's collaborative services and to EvolverAI were to become limited, terminated, or if we were otherwise precluded from conducting research and development using EvolverAI, or if BioXcel 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, as well as a director, officer and stockholder of BioXcel LLC. Additionally, as of November 5, 2021, each of Dr. Mehta and Dr. Nandabalan, through their beneficial ownership of BioXcel LLC, beneficially owned approximately 32% and 32%, respectively, 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;
- 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.

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, biotech, biopharmaceutical, diagnostic, medical device and contract research and manufacturing clients. Some of the technologies supporting the industries they serve are changing rapidly and we must continue to adapt to these changes in a timely and effective manner at an acceptable cost. They also must continue to deliver data to its clients in forms that are easy to use while simultaneously providing clear answers to complex questions. There can be no guarantee that we or BioXcel 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 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 we intend to rely on third parties to produce commercial supplies of any approved product candidate. Therefore, our development of our products could be stopped or delayed, and our commercialization of any future product could be stopped or delayed or made less profitable if third party manufacturers fail to obtain approval of the FDA or comparable regulatory authorities or fail to provide us with drug product in sufficient quantities or at acceptable prices.

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

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

We and/or our third-party manufacturers may be adversely affected by developments outside of our control, and these developments may delay or prevent further manufacturing of our products. Adverse developments may include labor disputes, resource constraints, shipment delays, inventory shortages, lot failures, unexpected sources of contamination, lawsuits related to our manufacturing techniques, equipment used during manufacturing, or composition of matter, unstable political environments, acts of terrorism, war, natural disasters, and other natural and man-made disasters. If BioXcel LLC, we or our third-party manufacturers were to encounter any of the above difficulties, or otherwise fail to comply with contractual obligations, our ability to provide any product for clinical trial or commercial purposes would be jeopardized. This may increase the costs associated with completing our clinical trials and commercial production. Further, production disruptions may cause us to terminate ongoing clinical trials and/or commence new clinical trials at additional expense. We may also have to take inventory write-offs and incur other charges and expenses for products that fail to meet specifications or pass safety inspections. Moreover, as a result of the COVID-19 pandemic, third-party manufacturers may 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, may be unable to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing approved products, if any.

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

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

Our strategy for the development and commercialization of our proprietary product candidates may include the formation of collaborative arrangements with third parties. Collaborators have significant discretion in determining the efforts and resources they apply and may not perform their obligations as expected. Potential third party collaborators include biopharmaceutical, pharmaceutical and biotechnology companies, academic institutions and other entities. Third-party collaborators may assist us in:

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

If we are not able to establish 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, or EEA and comparable foreign regulatory authorities for all of our products in clinical development.

Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our on-going clinical, nonclinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

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

Risks Related to Our Business and Industry

The outbreak of the novel coronavirus disease, COVID-19, or other pandemic, epidemic or outbreak of an infectious disease may materially and adversely impact our business, including our preclinical studies and clinical trials.

In 2020, the novel coronavirus disease, COVID-19, was declared a pandemic and spread across the globe, including throughout the United States and Europe. The pandemic and government measures taken in response have also 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 also implemented protocols as required under the State of Connecticut's Reopen program and have limited travel. We have taken steps to protect our workforce and have instituted strict work rules to protect our employees. Beginning late in the first quarter of 2021, and in line with State of Connecticut guidelines, we opened our office on a voluntary basis up to full capacity. In May 2021, the State of Connecticut lifted all business and office restrictions in the State. We have instituted a return to the office policy and will continue to evaluate that policy over the next several months.

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 federal or state 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 outbreak 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 United States 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 United States 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 in order to manage our operations and clinical trials, establish independent manufacturing, continue our research and development

activities, and commercialize our product candidate. Our management and scientific personnel, systems and facilities currently in place may not be adequate to support our future growth.

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

- manage our clinical trials effectively, including our planned clinical trials of BXCL501, BXCL701, BXCL502 and our other product candidates;
- manage our internal development efforts effectively while carrying out our contractual obligations to licensors, contractors and other third parties;
- continue to improve our operational, financial and management controls and reporting systems and procedures; and
- attract and retain sufficient numbers of talented employees.

We may utilize the services of third-party vendors to perform tasks including pre-clinical and clinical trial management, statistics and analysis, regulatory affairs, medical advisory, market research, formulation development, chemistry, manufacturing and control activities, other drug development functions, legal, auditing, financial advisory, and investor relations. Our growth strategy may also entail expanding our group of contractors or consultants to implement these and other tasks going forward. Because we rely on numerous consultants, to outsource many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for our product candidate or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may be unable to successfully implement the tasks necessary to further develop and commercialize our product candidate and, accordingly, may not achieve our research, development and commercialization goals.

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

Our success depends largely upon the continued services of our key executive officers, Vimal Mehta, our Chief Executive Officer, President 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 potential commercialization of BXCL501. 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 in the past experienced, and we expect to continue to experience in the future, difficulty in hiring and retaining highly skilled personnel with appropriate qualifications. The pool of qualified personnel with experience working with the pharma market is limited overall. In addition, many of the companies with which we compete for experienced personnel have greater resources than we have.

In addition, in making employment decisions, particularly in the biotechnology and high-technology industries, job candidates often consider the value of the stock options or other equity instruments they are to receive in connection

with their employment. Volatility in the price of our stock might, therefore, adversely affect our ability to attract or retain highly skilled personnel. Furthermore, the requirement to expense stock options and other equity instruments might discourage us from granting the size or type of stock option or equity awards that job candidates require to join our company. If we fail to attract new personnel or fail to retain and motivate our current personnel, our business and future growth prospects could be severely harmed.

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

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

In addition, we do not have any experience in acquiring other businesses. If we acquire additional businesses, we may not be able to integrate the acquired personnel, operations and technologies successfully, or effectively manage the combined business following the acquisition. We also may not achieve the anticipated benefits from the acquired business due to a number of factors, including:

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

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

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

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

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with any regulations applicable to us, to provide accurate information to regulatory

authorities, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations, or to report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Conduct 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 to manufacture BXCL501 and BXCL701 and conduct clinical trials, and similar events as those described in the prior paragraph relating to their business systems, equipment and facilities could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidate could be delayed or altogether terminated.

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 cyber-attacks or cyber-intrusions over the Internet, malware, computer viruses, email spoofing, attachments to e-mails, persons inside our organization, 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. For example, in September 2020, we were the victim of an email-based wire fraud that involved two electronic communications impersonating one of our vendors, resulting in our sending wires totaling $1.9 million to accounts controlled by the impersonator. 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. 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 artificial intelligence and machine learning may be subject to laws and evolving regulations regarding the use of artificial intelligence/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 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. Further, the 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, or 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 United States, and the efficacy and longevity of current transfer mechanisms between the EU and the United States remains uncertain. For example, in 2016, the EU and United States agreed to a transfer framework for data transferred from the EU to the United States, called the Privacy Shield, but the Privacy Shield was invalidated in July 2020 by the Court of Justice of the European Union, or CJEU. While the CJEU upheld the adequacy of the standard contractual clauses (a standard form of contract approved by the European Commission as an adequate personal data transfer mechanism, and potential alternative to the Privacy Shield), it made clear that reliance on them alone may not necessarily be sufficient in all circumstances. These and other recent developments are likely to require us to review and amend the legal mechanisms by which we make and/or receive personal data transfers to and in the United States. 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 to comply with the GDPR and also the United Kingdom GDPR, or 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 £17.5 million or 4% of global turnover. The European Commission has 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 additional product candidates and technologies. These investments will not constitute a significant portion of our business. However, our internal research capabilities are limited and we may be dependent upon pharmaceutical and biotechnology companies, academic scientists and other researchers to sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify, select and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. We have limited resources to identify and execute the acquisition or in-licensing of third party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated

benefits of such efforts. We may not be able to acquire the rights to additional product candidates on terms that we find acceptable, or at all.

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

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

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

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

As of December 31, 2020, we had federal operating loss carryforwards, or NOLs, of approximately $40.7 million. Our NOLs arising before January 1, 2018 are subject to expiration and will begin to expire in 2037. As of December 31, 2020, we also had federal and state research and development and other tax credit carryforwards, or credits of approximately $5.0 million available to reduce future tax liabilities. The federal and state credits expire at various dates through 2037. These NOLs and credits could expire unused and be unavailable to offset future taxable income or income tax liabilities. In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change NOLs or credits to offset future taxable income or income tax liabilities. For these purposes, an ownership change generally occurs where the aggregate change in stock ownership of one or more stockholders or groups of stockholders owning at least 5% of a corporation's stock exceeds 50 percentage points over a three-year period. Future changes in our stock ownership, including as a result of our public offerings of common stock in 2021 and 2020, many of which are outside of our control, could result in an ownership change. Our NOLs or credits may also be impaired under state law. Accordingly, even if we attain profitability, we may not be able to utilize a material portion of our NOLs or credits. Furthermore, under the Tax Cuts and Jobs Act of 2017 and modified by the CARES Act signed on March 27, 2020, although the treatment of NOLs arising on or before December 31, 2017 has generally not changed, NOLs arising on or after January 1, 2018 and beyond may only be used to offset 80% of taxable income for tax years beginning after December 31, 2020. This change may require us to pay federal income taxes in future years despite generating a loss for federal income tax purposes in prior years.

Risks Related to Our Intellectual Property

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

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our current and future product candidates, the processes used to manufacture them and the methods for using them, as well as successfully defending these patents against third-party challenges. We are the owner of record of patents and patent applications pending in the United States and in certain foreign jurisdictions. Patents issue from non-provisional applications, which are typically filed from provisional patent applications or from Patent Cooperation Treaty (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 October 18, 2021, we had five allowed or issued foreign patents relevant to our BXCL701 program and one issued U.S. patent and two issued foreign patents relevant to our BXCL501 program. We cannot be certain that any future patents will issue with claims that cover our product candidates. Our ability to stop third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

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

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

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

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

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

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

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

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

In addition, disputes may arise regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our diligence obligations under the license agreement and what activities satisfy those obligations;
- if a third-party expresses interest in an area under a license that we are not pursuing, under the terms of certain of our license agreements, we may be required to sublicense rights in that area to a third party, and that sublicense could harm our business; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us.

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

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

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

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

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

Our product candidates 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 Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, apply. In accordance with the Hatch-Waxman Act, such NDAs may be required to include certifications, known as paragraph IV certifications, that certify that any patents listed in the Patent and Exclusivity Information Addendum of the FDA's publication, Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book, with respect to any product referenced in the 505(b)(2) application, are invalid, unenforceable or will not be infringed by the manufacture, use or sale of the product that is the subject of the 505(b)(2) NDA.

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

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

If we choose to commence a proceeding or litigation to prevent another party from infringing our patents, that party will have the right to ask the examiner or court to rule that our patents are invalid or should not be enforced against them. There is a risk that the examiner or court will decide that our patents are not valid and that we do not have the right to stop the other party from using the related inventions. There is also the risk that, even if the validity of our

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

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

Our success will depend in part on our ability to operate without infringing, misappropriating or otherwise violating the proprietary rights of third parties. There is considerable patent and other intellectual property litigation in the pharmaceutical and biotechnology industries. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our products, or the manufacture or use of our product candidates, including interference proceedings, post grant review, inter partes review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions. The legal threshold for initiating litigation or contested proceedings is low, so that even lawsuits or proceedings with a low probability of success might be initiated and require significant resources to defend. The costs of these lawsuits could affect our results of operations and divert the attention of managerial and scientific personnel. Some of these third parties may be better capitalized and have more resources than us. There is a risk that a court would decide that we are infringing the third party's patents and would order us to stop the activities covered by the patents. In that event, we may not have a viable way around the patent and may need to halt commercialization of the relevant product candidate. In addition, there is a risk that a court will order us to pay the other party damages for having violated the other party's patents. In addition, we may be obligated to indemnify our licensors and collaborators against certain intellectual property infringement claims brought by third parties, which could require us to expend additional resources. The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform.

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

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

- some patent applications in the United States may be maintained in secrecy until the patents are issued;
- patent applications in the United States are typically not published until 18 months after the priority date; and

- publications in the scientific literature often lag behind actual discoveries.

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

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

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

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

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

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we could lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

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

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

Our drug re-innovation approach involves the filing of patent applications covering new methods of use and/or new formulations of previously known, studied and/or marketed drugs. Although the protection afforded by our patent 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 significantly affected.

We may elect to sue a third party, or otherwise make a claim, alleging infringement or other violation of patents, trademarks, trade dress, copyrights, trade secrets, domain names or other intellectual property rights that we either own or license from BioXcel 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 United States; thus, we may not be able to protect our intellectual property and third parties may be able to market competitive products that may use some or all of our intellectual property.

Changes to patent law, including the Leahy-Smith America Invents Act, AIA or Leahy-Smith Act, of 2011 and the Patent Reform Act of 2009 and other future article of legislation, may substantially change the regulations and procedures surrounding patent applications, issuance of patents, and prosecution of patents. We can give no assurances that our patents and those of our licensor, BioXcel 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 U.S. Patent and Trademark Office, courts and foreign government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Risks Related to Owning our Common Stock

The price of our common stock may fluctuate substantially.

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

- sale of our common stock by our stockholders, executives, and directors;
- volatility and limitations in trading volumes of our shares of common stock;
- 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 November 5, 2021, 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, or the 1940 Act, applicable restrictions could make it impractical for us to continue our business as contemplated and could have a material adverse effect on our business, financial condition and results of operations.

Under Sections 3(a)(1)(A) and (C) of the 1940 Act, a company generally will be deemed to be an “investment company” for purposes of the 1940 Act if (1) it is, or holds itself out as being, engaged primarily, or proposes to engage primarily, in the business of investing, reinvesting or trading in securities or (2) it engages, or proposes to engage, in the business of investing, reinvesting, owning, holding or trading in securities and it owns or proposes to acquire

investment securities having a value exceeding 40% of the value of its total assets (exclusive of U.S. government securities and cash items) on an unconsolidated basis. We do not believe that we are an "investment company," as such term is defined in either of those sections of the 1940 Act.

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

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, Section 107 of the JOBS Act also provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are not electing to delay such adoption of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an "emerging growth company." We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of our initial public offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the 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 more than $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 more than $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, and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide selected financial data, supplemental financial information or risk factors.

We have elected to take advantage of certain of the reduced reporting obligations. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be reduced or more volatile.

We may be at risk of securities class action litigation.

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

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

Our amended and restated certificate of incorporation and our amended and restated bylaws and Delaware law could make it more difficult for a third party to acquire us, even if closing such a transaction would be beneficial to our stockholders. We are authorized to issue up to 10,000,000 shares of preferred stock. This preferred stock may be issued in one or more series, the terms of which may be determined at the time of issuance by our board of directors without further action by stockholders. The terms of any series of preferred stock may include voting rights (including the right to vote as a series on particular matters), preferences as to dividend, liquidation, conversion and redemption rights and sinking fund provisions. No preferred stock is currently outstanding. The issuance of any preferred stock could materially adversely affect the rights of the holders of our common stock, and therefore, reduce the value of our common stock and the Notes. In particular, specific rights granted to future holders of preferred stock could be used to restrict our ability to merge with, or sell our assets to, a third party and thereby preserve control by the present management.

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

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

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

As a publicly traded company we have incurred significant additional legal, accounting and other expenses that we did not incur as a privately held subsidiary of BioXcel LLC. The obligations of being a public company in the United States 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 of 2002, or the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, and the listing requirements of the stock exchange on which our securities are listed. These rules require the establishment and maintenance of effective disclosure and financial controls and procedures, internal control over financial reporting and changes in corporate governance practices, among many other complex rules that are often difficult to implement, monitor and maintain compliance with. Moreover, despite 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 management and

other personnel will need to devote a substantial amount of time to ensure that we comply with all of these requirements and to keep pace with new regulations, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems.

General Risk Factors

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

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

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 of 2002 related to accounting controls and procedures in the future, or, if we discover material weaknesses and other deficiencies in our internal control and accounting procedures, our stock price could decline significantly and raising capital could be more difficult.

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

During the quarter ended September 30, 2020, we experienced an email-based wire fraud that resulted in the misappropriation of certain funds. As a result of the foregoing, we identified a material weakness due to our internal controls having not been adequately designed to prevent or timely detect unauthorized cash disbursements. If we are unable to remediate any such material weakness, if we are unable to implement and maintain effective internal control over financial reporting and effective disclosure controls and procedures, or if we fail to satisfy other requirements as a public company, including the requirements of Section 404 of the Sarbanes-Oxley Act, we may be unable to accurately report our financial results or report them within the timeframes required by the SEC.

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†	Separation Agreement and General Release between Reina Benabou and BioXcel Therapeutics, Inc., dated July 28, 2021.	10-Q	001-38410	10.1	8/10/2021	
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.
† Indicates a management contract or compensatory plan, contract or arrangement.

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: November 10, 2021

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

Dated: November 10, 2021

By:
/s/ Richard Steinhart
Richard Steinhart, Chief Financial Officer
(Principal 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 September 30, 2021 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: November 10, 2021

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 September 30, 2021 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: November 10, 2021

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 September 30, 2021, 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: November 10, 2021

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 September 30, 2021, 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: November 10, 2021

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