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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 June 30, 2023

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** (Address of principal executive offices)	**06511** (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 August 10, 2023 was 29,267,197.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). The words "anticipate," "believe," "can," "continue," "could," "designed," "estimate," "expect," "forecast," "goal," "intend," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. All statements contained in this Quarterly Report on Form 10-Q, other than statements of historical fact, are forward-looking statements, including, without limitation, statements regarding:

- our sales strategy for IGALMI™;
- strategy relating to, anticipated benefits from, and cost savings from our Reprioritization (as defined herein);
- developments relating to our TRANQUILITY II clinical trial;
- our ability to restructure or refinance our OFA Facilities (as defined herein) and extend our cash runway;
- our plans relating to clinical trials for our product candidates;
- 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, including 505(b)(2) regulatory approval, for our product candidates;
- the rate and degree of market acceptance, clinical utility, number of prescribers and formulary wins of IGALMI and any product candidates for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy, including the potential benefits from any advertising campaigns;
- our participation in, and any potential benefits from, events, conferences, presentations and conventions;
- our intellectual property position and strategy;
- our estimates regarding expenses, future revenue, capital requirements and need for additional financing;
- potential investments in, or other strategic options for, our subsidiary, OnkosXcel Therapeutics, LLC ("OnkosXcel");
- developments relating to our competitors and our industry;
- the impact of government laws and regulations; and
- our relationship with BioXcel LLC.

These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, 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. These and other important factors discussed under the caption "Risk Factors" in our other filings with the Securities and Exchange Commission ("SEC") could cause actual results to differ materially from those indicated by the forward-looking statements made in this filing. Given these uncertainties, you should not rely on these forward-looking statements as predictions of future events. While we may elect to update forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change.

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.

We may use our website as a distribution channel for material information about the Company. Financial and other important information regarding the Company is routinely posted on and accessible through the Investors & Media section of its website at *www.bioxceltherapeutics.com*. In addition, you may automatically receive email alerts and

other information about the Company when you enroll your email address by visiting the "Email Alerts" option under the News / Events menu of the Investors & Media section of our website at *www.bioxceltherapeutics.com.*

SUMMARY RISK FACTORS

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

- We have a limited operating history and have not generated substantial product revenues to date, 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.
- Our Reprioritization and related reduction in force may not achieve our intended outcome.
- 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 significant indebtedness and other contractual obligations that could impair our liquidity, restrict our ability to do business and thereby harm our business, results of operations and financial condition. We may not have sufficient cash flow from operations to satisfy our obligations under our financing facilities.
- We have identified conditions and events that raise substantial doubt about our ability to continue as a going concern.
- Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease. These developments also subject us to additional risks and uncertainties, including regulatory, stockholder or other actions, loss of investor confidence and negative impacts on the trading price of our common stock.
- We have limited experience in drug discovery and drug development.
- In the near term, we are dependent on the success of IGALMI, and four of our product candidates, BXCL501, BXCL502, BXCL701 and BXCL702. If we are unable to complete the clinical development of or obtain marketing approval for our product candidates or successfully commercialize IGALMI or our product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.
- Interim "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.
- The regulatory approval processes of the United States ("U.S.") Food and Drug Administration ("FDA"), and comparable foreign authorities are lengthy, time consuming, expensive and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.
- Clinical trials are expensive, difficult to design, difficult to conduct and involve an uncertain outcome.
- We depend on enrollment of patients in our clinical trials 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.

- 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.

- The discovery and development of product candidates based on EvolverAI, BioXcel LLC's proprietary pharmaceutical discovery and development engine, as well as our own AI platform is novel and unproven, and we do not know whether we will be able to develop any products of commercial value. Furthermore, EvolverAI and our own AI platform could be disrupted due to a rapidly evolving artificial intelligence ("AI") environment requiring us to in parallel develop an internal alternate AI engine.

- 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.

- Regulators may limit our ability to develop or implement our proprietary AI algorithms and/or may eliminate or restrict the confidentiality of our proprietary technology, which could have an adverse effect on our business, results of operations, and financial condition.

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

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

- Although we obtained FDA approval for IGALMI, our products and 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. Our business could be adversely affected if BioXcel LLC is unable to provide such services or there is a disruption in its provision of such services.

- 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.

- We are substantially dependent on third parties for the manufacture of our clinical supplies of our product candidates, and our commercial supplies of IGALMI, and we intend to rely on third parties to produce commercial supplies of any other approved product candidate.

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

- A securities class action has been filed against the Company, which could result in significant costs and/or liabilities, and, as a public Company, we continue to be at risk of securities class action litigation.

- We face risks associated with the increased scrutiny relating to environmental, social and governance matters.

- It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection.

TRADEMARKS, TRADE NAMES AND SERVICE MARKS

This Quarterly Report includes our trademarks, trade names and service marks, including, without limitation, "IGALMI" and our logo, which are our property and are protected under applicable intellectual property laws. Solely for convenience, trademarks, trade names and service marks may appear in this Quarterly Report without the ®, ™ and ℠ symbols, but such references are not intended to indicate, in any way, that we or the applicable owner forgo or will not assert, to the fullest extent permitted under applicable law, our rights or the rights of any applicable licensors to these trademarks, trade names and service marks. We do not intend our use or display of other parties' trademarks, trade names or service marks to imply, and such use or display should not be construed to imply a relationship with, or endorsement or sponsorship of us by, these other parties.

INDUSTRY AND OTHER DATA

Unless otherwise indicated, information contained in this Quarterly Report concerning our industry and the markets in which we operate, including our general expectations, market position and market opportunity, is based on our management's estimates and research, as well as industry and general publications and research, surveys and studies conducted by third parties. We believe the information from these third-party publications, research, surveys and studies included in this Quarterly Report is reliable. Management's estimates are derived from publicly available information, their knowledge of our industry and their assumptions based on such information and knowledge, which we believe to be reasonable. This data involves a number of assumptions and limitations which are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described in this Quarterly Report under "Forward-Looking Statements" and Part II, Item 1A "Risk Factors." These and other factors could cause our future performance to differ materially from our assumptions and estimates.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

BIOXCEL THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(amounts in thousands, except per share amounts)

	June 30, 2023 (unaudited)	December 31, 2022
ASSETS		
Current assets		
Cash and cash equivalents	$ 127,545	$ 193,725
Accounts receivable, net	431	248
Inventory	1,925	1,985
Prepaid expenses	3,698	3,067
Other current assets	4,636	3,843
Total current assets	$ 138,235	$ 202,868
Property and equipment, net	941	1,084
Operating lease right-of-use assets	834	976
Other assets	87	925
Total assets	$ 140,097	$ 205,853
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 11,203	$ 10,228
Accrued expenses	19,128	18,669
Due to related parties	854	422
Accrued interest	3,106	3,175
Other current liabilities	453	404
Total current liabilities	$ 34,744	$ 32,898
Long-term portion of operating lease liabilities	617	786
Derivative liabilities	2,026	2,343
Long-term debt	96,846	93,051
Total liabilities	$ 134,233	$ 129,078
Commitments and contingencies (Note 15)		
Stockholders' equity		
Preferred stock, $0.001 par value, 10,000 shares authorized; no shares issued and outstanding as of June 30, 2023 and December 31, 2022	$ —	$ —
Common stock, $0.001 par value, 100,000 shares authorized as of June 30, 2023 and December 31, 2022; 29,267 and 28,147 shares issued and outstanding as of June 30, 2023 and December 31, 2022, respectively	29	28
Additional paid-in-capital	523,691	488,292
Accumulated deficit	(517,856)	(411,545)
Total stockholders' equity	$ 5,864	$ 76,775
Total liabilities and stockholders' equity	$ 140,097	$ 205,853

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

BIOXCEL THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

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

	Three Months Ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Revenues				
Product revenue, net	$ 457	$ —	$ 663	$ —
Operating expenses				
Cost of goods sold	$ 26	$ —	$ 34	$ —
Research and development	26,973	17,906	54,773	36,593
Selling, general and administrative	25,872	18,382	49,467	31,175
Total operating expenses	$ 52,871	$ 36,288	$ 104,274	$ 67,768
Loss from operations	$ (52,414)	$ (36,288)	$ (103,611)	$ (67,768)
Other expense (income)				
Interest expense	3,259	1,586	6,627	1,593
Interest income	(1,621)	(204)	(3,636)	(219)
Other income, net	(537)	—	(291)	—
Net loss	$ (53,515)	$ (37,670)	$ (106,311)	$ (69,142)
Basic and diluted net loss per share attributable to common stockholders	$ (1.83)	$ (1.35)	$ (3.68)	$ (2.47)
Weighted average shares outstanding - basic and diluted	29,187	27,989	28,903	27,985

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

BIOXCEL THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

(amounts in thousands)
(unaudited)

	Common stock		Additional paid-in-capital	Accumulated deficit	Total
	Shares	Amount			
Balance as of December 31, 2021	27,980	$ 28	$ 467,427	$ (245,788)	$ 221,667
Stock-based compensation	—	—	3,825	—	3,825
Net loss	—	—	—	(31,472)	(31,472)
Balance as of March 31, 2022	27,980	$ 28	$ 471,252	$ (277,260)	$ 194,020
Stock-based compensation	—	—	4,482	—	4,482
Issuance of stock purchase warrants	—	—	3,245	—	3,245
Exercise of stock options	38	—	185	—	185
Net loss	—	—	—	(37,670)	(37,670)
Balance as of June 30, 2022	28,018	$ 28	$ 479,164	$ (314,930)	$ 164,262

	Common stock		Additional paid-in-capital	Accumulated deficit	Total
	Shares	Amount			
Balance as of December 31, 2022	28,147	$ 28	$ 488,292	$ (411,545)	$ 76,775
Issuance of common shares, net of offering costs	756	1	23,917	—	23,918
Stock-based compensation	—	—	4,877	—	4,877
Exercise of stock options	173	—	258	—	258
Vesting of restricted stock units, net of employee tax obligations	24	—	(27)	—	(27)
Net loss	—	—	—	(52,796)	(52,796)
Balance as of March 31, 2023	29,100	$ 29	$ 517,317	$ (464,341)	$ 53,005
Stock-based compensation	—	—	6,124	—	6,124
Exercise of stock options	136	—	250	—	250
Vesting of restricted stock units, net of employee tax obligations	31	—	—	—	—
Net loss	—	—	—	(53,515)	(53,515)
Balance as of June 30, 2023	29,267	$ 29	$ 523,691	$ (517,856)	$ 5,864

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

BIOXCEL THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(amounts in thousands)
(unaudited)

	Six months ended June 30,	
	2023	2022
OPERATING CASH FLOW ACTIVITIES:		
Net loss	$ (106,311)	$ (69,142)
Reconciliation of net loss to net cash used in operating activities		
Depreciation	161	161
Accretion of debt discount and amortization of financing costs	689	321
Change in fair value of derivative liabilities	(317)	—
Stock-based compensation expense	11,001	8,307
Payable-in-kind interest on Credit Agreement	803	—
Loss on disposal of equipment	2	—
Operating lease right-of-use assets	142	133
Changes in operating assets and liabilities		
Accounts receivable	(183)	—
Inventory	60	(1,395)
Prepaid expenses, other current assets and other assets	(527)	(7,072)
Accounts payable, accrued expenses, due to related parties, and other current liabilities	1,843	3,315
Accrued interest	2,234	—
Operating lease liabilities	(156)	(144)
Net cash used in operating activities	$ (90,559)	$ (65,516)
INVESTING CASH FLOW ACTIVITIES:		
Purchases of equipment and leasehold improvements	$ (20)	$ (139)
Net cash used in investing activities	$ (20)	$ (139)
FINANCING CASH FLOW ACTIVITIES:		
Proceeds from long-term debt	$ —	$ 68,600
Debt issuance costs	—	(2,646)
Proceeds from issuance of common stock	24,657	—
Offering costs for common stock issuance	(739)	—
Payment of employee tax obligations related to vested restricted stock units	(27)	—
Exercise of stock options	508	185
Net cash provided by financing activities	$ 24,399	$ 66,139
Net (decrease) increase in cash and cash equivalents	$ (66,180)	$ 484
Cash and cash equivalents, beginning of the period	193,725	232,968
Cash and cash equivalents, end of the period	$ 127,545	$ 233,452
Supplemental cash flow information:		
Deferred initial public offering costs in accounts payable	$ 59	$ —

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

BIOXCEL THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

Note 1. Nature of the Business

BioXcel Therapeutics, Inc. ("BTI" or the "Company") is a biopharmaceutical company utilizing artificial intelligence ("AI") approaches to develop transformative medicines in neuroscience and immuno-oncology. The Company is focused on utilizing cutting-edge technology and innovative research to develop high-value therapeutics aimed at transforming patients' lives. BTI employs unique AI platforms designed to reduce therapeutic development costs and potentially accelerate timelines. The Company's approach leverages existing approved drugs and/or clinically evaluated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI management believes this differentiated approach has the potential to reduce the expense and time associated with drug development in diseases with substantial unmet medical needs.

As used in these condensed consolidated financial statements, unless otherwise specified or the context otherwise requires, the term "BioXcel LLC" refers to the Company's former parent and current significant stockholder and its predecessor, BioXcel Corporation. "OnkosXcel" refers to BTI's wholly owned subsidiary for its advanced immuno-oncology assets, OnkosXcel Therapeutics, LLC.

On April 6, 2022, BTI announced that the U.S. FDA approved IGALMI (dexmedetomidine or "Dex") sublingual film for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. IGALMI is approved to be self-administrated by patients under the supervision of a healthcare provider. On July 6, 2022, BTI announced that IGALMI, was commercially available in doses of 120 and 180 micrograms through the Company's third-party logistics provider and was available for order through wholesalers.

The Company's most advanced clinical development program is BXCL501, an investigational proprietary, orally dissolving film formulation of Dex for the treatment of agitation associated with psychiatric and neurological disorders.

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

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

Note 2. Basis of Presentation

The accompanying unaudited interim condensed consolidated financial statements do not include all the information and notes required by Generally Accepted Accounting Principles ("GAAP") in the U.S. The accompanying year-end balance sheet was derived from audited financial statements but does not include all disclosures required by U.S. GAAP. The unaudited interim condensed consolidated 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 June 30, 2023, the results of its operations for the three and six months ended June 30, 2023 and 2022 and its cash flows for the six months ended June 30, 2023 and 2022. The results for the three and six months ended June 30, 2023 are not necessarily indicative of results to be expected for the year ending December 31, 2023, any other interim periods or any future year or period. The accompanying unaudited interim condensed consolidated financial statements of the Company should be read in conjunction with the audited financial statements and notes thereto included in the

Company's Annual Report on Form 10-K for the year ended December 31, 2022 filed with the Securities and Exchange Commission on March 16, 2023.

The accompanying condensed consolidated financial statements include the accounts for the Company and all entities where BTI has a controlling financial interest after elimination of all intercompany accounts and transactions and have been prepared in conformity with U.S. GAAP.

As of June 30, 2023, the Company had cash and cash equivalents of $127,545 and an accumulated deficit of $517,856. BTI has incurred substantial net losses and negative cash flows from operating activities in nearly every fiscal period since inception and expects this trend to continue for the foreseeable future. The Company recognized net losses of $53,515 and $37,670 for the three months ended June 30, 2023 and 2022, respectively, and $106,311 and $69,142 for the six months ended June 30, 2023 and 2022, respectively, and had net cash used in operating activities of $90,559 and $65,516 for the six months ended June 30, 2023 and 2022, respectively.

Under ASC Topic 205-40, Presentation of Financial Statements - Going Concern, management is required at each reporting period to evaluate whether there are conditions and events, considered in the aggregate, that raise substantial doubt about an entity's ability to continue as a going concern within one year after the date that the financial statements are issued.

The Company's history of significant losses, its negative cash flows from operations, potential near-term, increased covenant-driven payments under its OFA Facilities (as defined in Note 8, *Debt and Credit Facilities*), its limited liquidity resources currently on hand, and its dependence on its ability to obtain additional financing to fund its operations after the current resources are exhausted, about which there can be no certainty, have resulted in management's assessment that there is substantial doubt about the Company's ability to continue as a going concern for a period of at least 12 months from the issuance date of the financial statements included in this Quarterly Report on Form 10-Q.

The accompanying condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business, and do not include any adjustments that may result from the outcome of this uncertainty.

This going concern evaluation takes into consideration the potential mitigating effect of management's Reprioritization (as defined in Note 16 Subsequent Events) plans that have not been fully implemented as of the date the financial statements are issued. When substantial doubt exists, management evaluates whether the mitigating effect of its plans sufficiently alleviates the substantial doubt about the Company's ability to continue as a going concern. The mitigating effect of management's plans, however, is only considered if both (i) it is probable that the plans will be effectively implemented within one year after the date that the financial statements are issued and (ii) it is probable that the plans, when implemented, will mitigate the relevant conditions or events that raise substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued. Generally, to be considered probable of being effectively implemented, the plans need to be approved by the Company's Board of Directors. The Company's Reprioritization was approved by the Board of Directors on August 8, 2023, however, such plans will not mitigate the entity's ability to continue as a going concern within one year after the date that the financial statements are issued.

The accompanying condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business and does not include any adjustments that may result from the outcome of this uncertainty. The going concern analysis does not consider possible amendments to or restructuring of the OFA Facilities or other potential sources of debt or equity capital.

Successful completion of the Company's development programs and, ultimately, the attainment of profitable operations are dependent upon future events, including obtaining adequate financing to support the Company's cost structure and operating plan. Management's plans to improve the Company's liquidity and reduce its operating expenses and capital requirements include, among other things, pursuing one or more of the following steps to raise additional capital, none of which can be guaranteed or are entirely within the Company's control:

- raise funding through the sale of the Company's equity securities;
- raise funding through third-party investments in or other strategic options for OnkosXcel;
- raise funding through debt financing and/or restructuring of its existing OFA Facilities;
- establish collaborations with potential partners to advance the Company's product pipeline;
- establish collaborations with potential marketing partners;
- reduce overhead and headcount to focus on core priorities; and/or
- any combination of the foregoing.

If the Company is unable to raise capital when needed or on acceptable terms, refinance or restructure its existing OFA Facilities or if it is unable to procure collaboration arrangements to advance its programs, the Company would be forced to discontinue some of its operations or develop and implement a plan, beyond its Reprioritization initiatives, to further extend payables, reduce overhead, scale back or cease some or all of its revised operating plan until sufficient additional capital is raised to support further operations.

Note 3. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in accordance with U.S. GAAP requires management to make estimates and assumptions that affect amounts reported in the condensed consolidated financial statements and notes thereto. Although these estimates are based on the Company's knowledge of current events and actions it may undertake in the future, actual results may ultimately materially differ from these estimates.

Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. As of June 30, 2023 and December 31, 2022, cash equivalents were comprised primarily of money market funds. Cash and cash equivalents held at financial institutions may at times exceed federally insured amounts. BTI management believes it mitigates such risk by investing in or through major financial institutions.

Accounts Receivable, Net

Accounts receivable arise from sales of IGALMI and represent amounts due from distributors. Payment terms generally range from 30 to 75 days from the date of the sale transaction, and accordingly, do not involve a significant financing component. Receivables from product sales are recorded net of allowances which generally include distribution fees, prompt payment discounts, chargebacks, and credit losses. Allowances for distribution fees, prompt payment discounts and chargebacks are based on contractual terms. The Company estimated the current expected credit losses of its accounts receivable by assessing the risk of loss and available relevant information about collectability, existing contractual payment terms, actual payment patterns of its customers, individual customer circumstances, and reasonable and supportable forecast of economic conditions expected to exist throughout the contractual life of the receivable. Based on its assessment, as of June 30, 2023, the Company determined that an allowance for credit losses was not required.

Concentrations of Credit Risk

The Company sells IGALMI through a drop-ship program under which orders from hospitals and similar health care institutions are processed through wholesalers, but shipments of the product are sent directly to the individual hospitals and similar health care institutions. BTI also contracts directly with certain hospitals, and intermediaries such as group purchasing organizations ("GPOs"). All trade accounts receivables are due from the distributor that fulfills orders on behalf of the Company.

Inventory

Inventory is stated at the lower of cost or net realizable value. Cost of inventory is determined on a first-in, first-out basis.

BTI capitalizes inventory costs associated with the Company's products prior to regulatory approval, when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized; otherwise, such costs are expensed as Research and development expense in the Condensed Consolidated Statements of Operations.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period and writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, will be recorded within Cost of goods sold in the Condensed Consolidated Statements of Operations. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected, write-downs of inventory may be required.

Deferred Initial Public Offering Costs

Deferred initial public offering costs of $2,570 as of June 30, 2023, consist of legal, accounting, underwriting fees and other costs incurred through the balance sheet date that are directly related to the Company's proposed initial public offering of OnkosXcel and are included in Other current assets in the Condensed Consolidated Balance Sheets. The deferred initial public offering costs will reduce the proceeds of the proposed initial public offering of OnkosXcel. Should the proposed initial public offering prove to be unsuccessful, these deferred costs, as well as any additional expenses incurred, will be recorded as an expense in the Condensed Consolidated Statements of Operations.

Property and Equipment

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

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

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

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 from its use and disposition. 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

The Company determines 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 the Condensed Consolidated Balance Sheets.

ROU assets represent BTI's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease ROU assets and lease liabilities are recognized at commencement date based on the present value of lease payments over the lease term. The Company

uses the implicit rate when readily determinable. As BTI's leases do not provide an implicit rate, it used an incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments. The operating lease ROU asset also includes any prepaid lease payments made and excludes lease incentives. The Company's leases may include options to extend the lease; such options are included in determining the lease term when it is reasonably certain that BTI will exercise that option. Lease expense is recognized on a straight-line basis over the lease term.

Debt and Detachable Warrants

Detachable warrants are evaluated for classification as either equity instruments, derivative liabilities, or liabilities depending on the specific terms of the warrant agreement. The portion of the proceeds allocated to the warrants are accounted for as paid-in capital and a debt discount. The remaining proceeds, as further reduced by discounts created by the bifurcation of any embedded derivatives, are allocated to the debt. Detachable warrants classified as derivative liabilities are accounted for as indicated under "*Derivative Assets and Liabilities*" section of this Note and as a debt discount. The Company accounts for debt as liabilities measured at amortized cost and amortizes the resulting debt discount from the allocation of proceeds to interest expense using the effective interest method over the expected term of the debt instrument. The Company considers whether there are any embedded features in debt instruments that require bifurcation and separately accounts for them as derivative financial instruments.

The Company entered into financing arrangements, the terms of which involve significant assumptions and estimates, including future net product sales, in determining interest expense, amortization period of the debt discount, as well as the classification between current and long-term portions. In estimating future net product sales, the Company assesses prevailing market conditions using various external market data against the Company's anticipated sales and planned commercial activities. Consequently, the Company imputes interest on the carrying value of the debt and records interest expense using an imputed effective interest rate. The Company reassesses the expected payments during each reporting period and accounts for any changes through an adjustment to the effective interest rate on a prospective basis, with a corresponding impact to the classification of the Company's current and long-term portions of the debt.

Derivative Assets and Liabilities

Derivative assets and liabilities are recorded on the Company`s Condensed Consolidated Balance Sheets at their fair value on the date of issuance and are revalued on each balance sheet date until such instruments are settled or expire, with changes in the fair value between reporting periods recorded as other income or expense within Other income, net in the Condensed Consolidated Statements of Operations.

The Company does not use derivative instruments for speculative purposes or to hedge exposures to cash flow or market risks. Certain financing facilities entered into by the Company include freestanding financial instruments and/or embedded features that require separate accounting as derivative assets and/or liabilities.

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.

Revenue Recognition

The Company's revenues consist of product sales of IGALMI.

BTI recognizes revenue when its customers obtain control of promised goods or services, in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. To determine revenue recognition, BTI management performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price, including variable consideration, if any; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies a performance obligation. Arrangements that include rights to additional goods or services that are exercisable at a customer's discretion are generally considered options. The Company assesses if these options provide a material right to the customer and if so, they are considered performance obligations.

The exercise of a material right may be accounted for as a contract modification or as a continuation of the contract for accounting purposes.

The Company assesses whether the goods or services promised within each contract are distinct to identify those that are performance obligations. This assessment involves subjective determinations and requires management to make judgments about the individual promised goods or services and whether such goods and services are separable from the other aspects of the contractual relationship. Promised goods and services are considered distinct provided that: (i) the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer and (ii) the Company's promise to transfer the good or service to the customer is separately identifiable from other promises in the contract.

The Company allocates the transaction price (the amount of consideration it expects to be entitled to from a customer in exchange for the promised goods or services) to each performance obligation and recognizes the associated revenue when (or as) each performance obligation is satisfied. The Company's estimate of the transaction price for each contract includes all variable consideration to which the Company expects to be entitled.

BTI distributes IGALMI in the U.S. through arrangements with a distributor, wholesalers, and GPOs. The distributor and wholesalers help process and fulfill orders from hospitals on the Company's behalf. The Company believes the hospitals are its customers.

The Company recognizes product revenues, net of consideration payable to customers, as well as variable consideration related to certain allowances and accruals that are determined using either the expected value or most likely amount method, depending on the type of the variable consideration, in its condensed consolidated financial statements at the point in time when control transfers to the customer, which is typically when the product has been delivered to the customer's location. The amount included in the transaction price is constrained to the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. The Company's only performance obligation identified for IGALMI is to deliver the quantity of product ordered to the location specified by the customer's order. The Company records shipping and handling costs associated with delivery of product to its customers within Selling, general and administrative expenses on its Condensed Consolidated Statements of Operations. Under the Company's current product sales arrangements, BTI does not have contract assets (unbilled receivables), as it generally invoices its customer at the time of revenue recognition.

BTI sells IGALMI at wholesale acquisition cost and calculates product revenue net of variable consideration and consideration payable to third parties associated with distribution of product. The Company records reserves, based on contractual terms, for the following components of consideration related to product sold during the reporting period. Calculating these amounts involves estimates and judgments, and the Company reviews these estimates quarterly and records any material adjustments in the period they are identified, which affects net product revenue and earnings in the period such variances occur.

Trade Discounts and Allowances

The Company provides the distributor and wholesalers with discounts for prompt payment and pays fees to the distributor, wholesalers and GPOs related to distribution of the product. BTI expects the relevant third parties to earn these discounts and fees, and therefore it deducts such amounts from gross product revenue and accounts receivable at the time it recognizes the related revenue.

Government Rebates

IGALMI is eligible for purchase by, or qualifies for reimbursement from, Medicaid and other U.S. government programs that are eligible for rebates on the price they pay for the product. To determine the appropriate amount to reserve for these rebates, BTI applies the applicable government discount to these sales, and estimates the portion of total rebates that it anticipates will be claimed. The Company deducts certain government rebates from gross product

revenue and accounts receivable at the time it recognizes the related revenue; other government rebates are recognized as an accrued liability at the time BTI recognizes the related revenue.

Chargebacks

BTI provides product discounts to hospitals associated with certain GPOs. The Company estimates the chargebacks that it expects to be obligated to provide based upon the terms of the applicable arrangements. BTI deducts such amounts from gross product revenue and accounts receivable at the time it recognizes the related revenue.

Product Returns

The Company provides contractual return rights to its customers including the right to return product within six months of product expiration and up to 12 months after product expiration, as well as for incorrect shipments, and damaged or defective product, which the Company expects to be rare. Management expects product returns to be minimal, thus BTI recognizes a nominal allowance for product returns at the time of each sale. In the future, if any of these factors and/or the history of product returns changes, the Company will adjust the allowance for product returns.

BTI classifies all fees paid to the distributor, other than those discussed above and those related to warehouse operations, as Selling, general and administrative expenses on its Condensed Consolidated Statements of Operations. Fees paid to the distributor for warehouse operations are classified as Cost of goods sold on BTI's Condensed Consolidated Statements of Operations.

Cost of Goods Sold

Cost of goods sold includes the cost of producing and distributing inventories that are related to product revenues during the respective period. Cost of goods sold may also include costs related to excess or obsolete inventory adjustment charges, as well as costs related to warehouse operations paid to distributors.

Stock-Based Compensation

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

The Company's stock-based awards are valued at fair value on the date of grant and that fair value is recognized as an expense in the Condensed Consolidated Statements of Operations over the requisite service period using the accelerated attribution method. The estimated fair value of RSUs is based on the Company's closing stock price on the grant date or third-party valuation if related to a subsidiary. The estimated fair value of stock-option and profit unit awards was determined using the Black-Scholes pricing model on the date of grant.

The Black-Scholes pricing model is affected by the Company's stock price, as well as assumptions regarding variables including, but not limited to, the strike price of the instrument, the risk-free rate, the expected stock price volatility over the term of the awards and expected term of the award. 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, non-cash stock-based compensation, facilities, supplies, external services, clinical study, manufacturing costs related to clinical trials and other expenses that are directly related to the Company's research and development activities. At the end of the reporting period, the Company compares payments made to third-party service providers to the estimated progress toward completion of the research or development objectives. Depending on the timing of payments to the service providers and the progress that the Company estimates has been made for the program as a result of the level of service provided, the Company may

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

Most of the Company's service providers invoice BTI monthly in arrears for services performed. The Company estimates its accrued expenses as of each balance sheet date in the condensed consolidated financial statements based on facts and circumstances known to management at that time. BTI management periodically confirms the accuracy of the Company's estimates with the service providers and makes adjustments if necessary.

Although management does not expect its estimates to be materially different from amounts actually incurred, management's 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 BTI 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 in Selling, general and administrative expenses in the Condensed Consolidated Statements of Operations and are expensed as incurred since recoverability of such expenditures is uncertain.

Fair Value of Financial Instruments

The Company measures certain financial assets and liabilities at fair value, which is defined 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. The Company applies 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). Fair value measurements must be classified and disclosed in one of the following 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, as well as considering counterparty credit risk in its assessment of fair value.

Earnings (Loss) Per Share

Earnings (loss) per share ("EPS") is calculated by dividing net income or loss attributable to common stockholders by the weighted average number of shares of common stock that were outstanding. Diluted EPS is calculated by adjusting the weighted average number of shares of common stock that were outstanding for the dilutive effect of common stock equivalents. In periods in which a net loss is recorded, no effect is given to potentially dilutive securities, since the effect would be antidilutive.

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, the Company's 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 June 2016, the Financial Accounting Standards Board ("FASB") issued ASU No. 2016-13, *Financial Instruments-Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*, and subsequent amendments to the initial guidance (collectively, "Topic 326"). Topic 326 requires measurement and recognition of expected credit losses for financial assets held. Topic 326 was to be effective for reporting periods beginning after December 15, 2019, with early adoption permitted. In November 2019, the FASB issued ASU No. 2019-10, *Financial Instruments - Credit Losses (Topic 326), Derivatives and Hedging (Topic 815), and Leases (Topic 842) - Effective Dates*, which deferred the effective dates of Topic 326 for the Company, until fiscal year 2023. The adoption of Topic 326 on January 1, 2023 did not have a material impact on the Company's condensed consolidated financial statements.

Note 4. Inventory

Inventory consists of the following:

	June 30, 2023	December 31, 2022
Raw materials	$ 682	$ 682
Work-in-process	—	708
Finished goods	1,243	595
Total inventory	$ 1,925	$ 1,985

There were no write-downs of inventory for the three and six months ended June 30, 2023 and 2022**.**

Note 5. Property and Equipment, Net

Property and equipment, net consists of the following:

	June 30, 2023	December 31, 2022
Computers and equipment	$ 202	$ 213
Furniture	575	575
Leasehold improvements	1,200	1,181
Total property and equipment	$ 1,977	$ 1,969
Accumulated depreciation	(1,036)	(885)
Total property and equipment, net	$ 941	$ 1,084

Depreciation expense was $81 and $84 for the three months ended June 30, 2023 and 2022, respectively, and $161 and $161 for the six months ended June 30, 2023 and 2022, respectively.

Note 6. Accrued Expenses

Accrued expenses consist of the following:

	June 30, 2023	December 31, 2022
Accrued research and development expenses	$ 9,754	$ 8,659
Accrued compensation and benefits	4,039	6,370
Accrued professional fees	4,601	2,738
Accrued taxes	144	82
Other accrued expenses	590	820
Total accrued expenses	$ 19,128	$ 18,669

Note 7. 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 thereafter (the “Services Agreement”), pursuant to which BioXcel LLC has agreed to provide the Company with certain services through its subsidiaries in India and the U.S., as agreed upon by the parties. These services are primarily for drug discovery, chemical, manufacturing and controls and administrative support.

Service charges recorded under the Services Agreement for the three and six months ended June 30, 2023 and 2022 were as follows:

	Three Months Ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Research and development	$ 414	$ 299	$ 629	$ 605
Selling, general and administrative	60	58	86	129
Total	$ 474	$ 357	$ 715	$ 734

As of June 30, 2023, $747 of service charges related to the Services Agreement are included in Due to related parties in the Company’s Condensed Consolidated Balance Sheets.

Note 8. Debt and Credit Facilities

Debt, net of unamortized discounts and financing costs, consists of the following:

	June 30, 2023	December 31, 2022
Revenue Interest Financing Agreement ("RIFA")	$ 30,000	$ 30,000
RIFA accrued interest	4,064	2,041
RIFA payments	(45)	(10)
RIFA debt liability	$ 34,019	$ 32,031
Estimated Portion of RIFA debt liability to be paid within one-year	(1,086)	(1,401)
RIFA long-term debt liability	$ 32,933	$ 30,630
Credit Agreement and Guaranty	70,000	70,000
Payable-in-kind interest on Credit Agreement and Guaranty	1,610	807
Total long-term debt liability	$ 104,543	$ 101,437
Unamortized debt discounts and issuance costs	(7,697)	(8,386)
Total long-term debt	$ 96,846	$ 93,051

On April 19, 2022 (the “Effective Date”), the Company entered into two financing agreements: (i) a Credit Agreement and Guaranty (the “Credit Agreement”) by and among the Company, as the borrower, certain subsidiaries of the Company from time to time party thereto as subsidiary guarantors, the lenders party thereto (the “Lenders”), and Oaktree Fund Administration LLC (“OFA”) as administrative agent, and (ii) a Revenue Interest Financing Agreement (as amended from time to time, the “RIFA”; and together with the Credit Agreement, the “OFA Facilities”) by and among the Company, the purchasers party thereto (the “Purchasers”) and OFA as administrative agent. Under the OFA Facilities, the Lenders and the Purchasers agreed to, in aggregate between the two OFA Facilities, provide up to $260,000 in gross funding to support the Company’s commercial activities of IGALMI sublingual film. In addition, the OFA Facilities are intended to support the expansion of clinical development efforts of BXCL501, which includes a Phase 3 program for the acute treatment of agitation in patients with Alzheimer’s disease, and for general corporate purposes. The Lenders and Purchasers are comprised of affiliates of Oaktree Capital Management, L.P. and Qatar Investment Authority.

A summary of the OFA Facilities is provided below.

Credit Agreement

The Credit Agreement provides up to $135,000 in senior secured term loans, of which the initial Tranche A of $70,000 was funded on April 28, 2022, and the remaining tranches may be borrowed at the Company’s option prior to December 31, 2024, subject to satisfaction of certain conditions, including regulatory and financial milestones. Tranche B of the Credit Agreement is $35,000 and is available upon satisfaction of certain conditions, including receipt of certain regulatory and financial milestones. Tranche C of the Credit Agreement is $30,000 and is available upon satisfaction of certain conditions, including specified minimum net sales of the Company attributable to sales of BXCL501 for a trailing consecutive 12-month period. As of June 30, 2023, $65,000 remained available under the Credit Agreement, subject to achievement of the specified conditions and milestones.

The loans under the Credit Agreement do not amortize and mature on the fifth anniversary of the Effective Date; provided that the Company may, at its option, extend the maturity date to the sixth anniversary if, prior to December 31, 2024, the Company receives and satisfies certain conditions including receipt of certain regulatory and financial milestones. Borrowings under the Credit Agreement are issued at a 200-basis point original issue discount and bear interest at a fixed annual rate of 10.25%, payable quarterly. Of such interest, 225-basis points per annum is, at the Company’s option, payable in kind by capitalizing and adding such interest to the outstanding principal amount of loans from the first payment date on which such interest is owed through, and including, the third anniversary of such payment date, unless, with respect to any payment date, the Company elects to pay all or a portion of such interest in cash. The Company is required to pay a ticking fee equal to 0.75% per annum on the undrawn amount of the commitments, payable quarterly commencing 120 days after the funding of the Tranche A term loan through the termination of the commitments, which is expensed as incurred and recognized as Interest expense in the Condensed Consolidated Statements of Operations. The Company may voluntarily prepay the Credit Agreement at any time subject to a prepayment fee.

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

The Credit Agreement contains customary representations and warranties and customary affirmative and negative covenants, including, among other things, restrictions on indebtedness, liens, investments, mergers, dispositions, prepayment of other indebtedness, and dividends and other distributions, subject to certain exceptions, including specific exceptions with respect to product commercialization and development activities. The Company must also comply with certain financial covenants, including (i) maintenance of cash or permitted cash equivalent investments in accounts controlled by OFA for the Lenders, of at least (a) $15,000 from the Effective Date until the date on which the second tranche of loans are funded (the “Step-Up Date”) and (b) $20,000 from and after the Step-Up Date, provided, in the case of (a) and (b), that following any Permitted BXCL701 Release Event (as defined below), such amount will

increase by $12,500, and following such time as unaffiliated third parties hold ownership of at least 30% of the equity interests in the BXCL701 Subsidiaries (as defined below), such amount will increase by an additional $5,000 (provided, that such amount will in no event exceed 50% of the aggregate amount of loans outstanding at any time); and (ii) a minimum revenue test, measured quarterly beginning with the Company's fiscal quarter ending on December 31, 2023 (such six-month period the "Revenue Covenant Measurement Period"), that requires it and its subsidiaries' consolidated net revenue for the six consecutive month period ending on the last day of each such fiscal quarter to not be less than a minimum revenue amount specified in the Credit Agreement (such testing date, the "Revenue Covenant Measurement Testing Date" and the covenant described in this clause (ii) the "Revenue Covenant"). The Company's failure to comply with the financial covenants will result in an event of default, subject to certain cure rights with respect to the Revenue Covenant. If, as of a Revenue Covenant Measurement Testing Date, the Company's revenue for the applicable Revenue Covenant Measurement Period is less than the minimum revenue amount specified for the applicable period then required under the Revenue Covenant, the Company would have a right to cure such shortfall for a total of three fiscal periods by making a revenue cure payment (which would be treated as prepayments of the loans subject to a prepayment fee) to the Lenders in an amount equal to the difference between such minimum required revenue amount and the Company's actual revenues for such Revenue Covenant Measurement Period, such payment to not be less than $1,000. If paid, the Company will be deemed to have complied with the Revenue Covenant as of such Revenue Covenant Measurement Testing Date. Any such payment will be applied to the prepayment of the loans under the Credit Agreement. For the Revenue Covenant Measurement Testing Dates ending December 31, 2023, March 31, 2024 and June 30, 2024, shortfalls under the Revenue Covenant, as described above, could result in a revenue cure payment of up to $7,657, $10,636, and $14,313, respectively, plus aggregate prepayment fees of up to $1,500.

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

The Credit Agreement contains events of default that are customary for financings of this type relating to, among other things, payment defaults, breach of covenants, breach of representations and warranties, cross default to material indebtedness, bankruptcy-related defaults, judgment defaults, breach of the financial covenants described above, and the occurrence of certain change of control events.

In certain circumstances, events of default are subject to customary cure periods. The Credit Agreement also contains certain regulatory-related events of defaults, which do not have cure periods. Following an event of default and any applicable cure period, the Lenders will have the right upon notice to terminate any undrawn commitments and may accelerate all amounts outstanding under the Credit Agreement, in addition to other remedies available to them as the Company's secured creditors.

Revenue Interest Financing Agreement

The RIFA provides up to $120,000 in financing in exchange for a capped revenue interest on net sales of IGALMI, and other future BXCL501 products, if any, that receive regulatory approval for sale. The initial Tranche A of $30,000 was funded on July 8, 2022, and the remaining tranches may be borrowed at the Company's option prior to December 31, 2024, subject to satisfaction of certain conditions, including certain regulatory, patent, and financial milestones. The effective interest rate on the RIFA as of June 30, 2023 and December 31, 2022, was approximately 11% and 14%, respectively.

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

Any time after the initial funding of the RIFA, BTI has the right (the "BTI Call Option"), but not the obligation, to buy out the Purchasers' interests in the revenue interest payments at an agreed upon repurchase price. The BTI Call Option can be exercised in year one, two, three and thereafter at a multiple of the Purchasers invested capital of 1.225x,1.375x, 1.525x and 2.25x, respectively. The Purchasers will not be entitled to any Tail Royalty if the BTI Call Option is exercised before the third anniversary of the Effective Date.

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

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

Tranche B and C of the RIFA are each $45,000 and are available upon satisfaction of certain conditions, including receipt of certain regulatory and patent-related milestones and specified minimum net sales of BXCL501 during any consecutive 12-month period. As of June 30, 2023, $90,000 remained available under the RIFA, subject to achievement of the specified conditions and milestones.

The Company does not currently expect to satisfy the conditions necessary to draw Tranches B or C.

Warrants and Equity Investment Right

In connection with the Credit Agreement, on the Effective Date, the Company granted warrants to the Lenders to purchase up to 278 shares of its common stock (the "BTI Warrants") at an exercise price of $20.04 per share. The BTI Warrants will expire on April 19, 2029, are freely transferable and may be net exercised at the holder's election. In addition, pursuant to the Credit Agreement, the Lenders have the right to purchase shares of the Company's common stock after the Effective Date, so long as borrowings under the Credit Agreement are outstanding, for a purchase price of $5,000 at a price per share equal to a 10% premium to the volume-weighted average price of the common stock over the 30 trading days prior to the Lenders' election to proceed with such equity investment (the "Equity Investment Right"). BTI entered into a registration rights agreement with the Lenders and filed a registration statement on Form S-3 to register the shares issuable upon exercise of the BTI Warrants and, if issued, the shares related to the Equity Investment Right, for resale. The maximum shares of BTI common stock issuable under the BTI Warrants and Lenders' Equity Investment Right is 5,593.

As part of the Credit Agreement, OnkosXcel granted warrants to the Lenders to purchase 175 individual limited liability company units (which number of units is not in thousands; referred to herein as the "OnkosXcel Warrants"). The strike price of the OnkosXcel Warrants is formulaic based on the value of OnkosXcel at the time of exercise and can only be exercised upon occurrence of an equity related liquidity event for OnkosXcel of at least $20,000. The exercise price per unit of the OnkosXcel Warrants will be set upon the earlier of the closing of the next sale (or series of related sales) by OnkosXcel of equity securities of OnkosXcel with aggregate proceeds of not less than $20,000 to unrelated third parties (the "Next Equity Financing") at an exercise price per unit equal to a 10% premium over the price per unit of the equity securities sold by OnkosXcel in such Next Equity Financing or, in the event of a sale of OnkosXcel prior to the Next Equity Financing or an initial public offering constituting the Next Equity Financing, the lesser of (x) 75% of the fair value of the consideration to be paid for a unit upon the consummation of such transaction and (y) 150% of the valuation applicable to the initial profits units issued by OnkosXcel after the closing of the Credit Agreement. The OnkosXcel Warrants are transferable with approval from BTI, which cannot be unreasonably withheld, expire on April 19, 2029, and may be net exercised at the holder's election.

Maturities of debt, excluding the impacts of any mandatory payments pursuant to the Revenue Covenant or to meet minimum royalty levels, are expected to be as follows:

	June 30, 2023
2023	$ 139
2024	$ 2,288
2025	$ 4,956
2026	$ 8,647
2027	$ 86,168
Thereafter	$ 3,431

Interest expense was as follows:

	Three Months Ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Interest expense	$ 2,903	$ 1,265	$ 5,938	$ 1,272
Accretion of debt discount and amortization of financing costs	356	321	689	321
Total interest expense	$ 3,259	$ 1,586	$ 6,627	$ 1,593

Note 9. Derivative Financial Instruments

BTI identified certain freestanding financial instruments and/or embedded features that require separate accounting from the borrowings under the OFA Facilities. This includes the OnkosXcel Warrants and Equity Investment Right held by the Lenders, along with certain put/call options. The OnkosXcel Warrants and Equity Investment Right do not meet certain scope exceptions under U.S. GAAP, primarily because the exercise prices and number of shares of the Company's common stock issuable under the instruments are variable, and the instruments meet the definition of a derivative instrument. Therefore, these instruments are recorded as Derivative liabilities in the Condensed Consolidated Balance Sheets. The respective derivative liabilities were recorded at fair value on the date of issuance and are revalued on each balance sheet date until such instruments are settled or expire, with changes in the fair value between reporting periods recorded within Other income, net in the Company's Condensed Consolidated Statements of Operations.

Note 10. Common Stock Financing Activities

In May 2021, the Company entered into an Open Market Sale Agreement (the "Sale Agreement") with Jefferies LLC ("Jefferies") pursuant to which the Company could offer and sell shares of its common stock, having an aggregate offering price of up to $100,000, from time to time, through an "at the market offering" program under which Jefferies

will act as sale agent. The Company sold 756 shares under the Sale Agreement with Jefferies in the first six months of 2023 for net proceeds of $23,918, net of offering costs of $739. There were no sales under the Sale Agreement in 2022.

Note 11. 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, 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 and (ii) 233 shares of the Company's common stock, which represents the number of shares that remained available for issuance under the 2017 Plan immediately prior to the approval of the 2020 Plan by the Company's stockholders. Any shares of common stock which, immediately prior to the approval of the 2020 Plan by the Company's stockholders, were subject to awards granted under the 2017 Plan that are forfeited or lapse unexercised and are not issued under the 2017 Plan will increase the number of shares of common stock available for grant under the 2020 Plan. In addition, the number of shares available for issuance under the 2020 Plan will increase on the first day of each calendar year, beginning January 1, 2021 and ending on and including January 1, 2030, by a number of shares equal to the lesser of (A) 4% of the aggregate number of shares of the Company's common stock outstanding on the final day of the immediately preceding calendar year and (B) such smaller number of shares of common stock as determined by the Board of Directors. The shares available for issuance under the 2020 Plan increased by 1,126 shares and 1,119 shares on January 1, 2023 and 2022, respectively.

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

As of June 30, 2023, there were 561 shares available to be granted under the 2020 Plan.

Restricted stock units

The table below summarizes activity relating to BTI RSUs.

	Number of shares
Outstanding as of January 1, 2023	119
Granted	133
Vested	(55)
Outstanding as of June 30, 2023	197

During the six months ended June 30, 2023, the Company granted 133 time-based RSUs to certain employees. All of the RSUs vest over four years, with 25% vesting at the one-year anniversary of the grant date and the balance vesting ratably over the remaining 12 quarters of the vesting period. The average grant date fair value per share for the RSUs was $19.62. Unrecognized stock-based compensation expense related to these awards was approximately $2,419 as of June 30, 2023.

During the six months ended June 30, 2022, the Company granted 122 (119, net of forfeitures) time-based RSUs to certain employees and consultants. The majority of RSUs granted to employees vest over four years, with 25% vesting at the one-year anniversary of the grant date and the balance vesting ratably over the remaining 12 quarters of the vesting period. There were 25 RSUs granted to employees in May 2022 which fully vested at the one-year anniversary

of the grant date. RSUs granted to a third-party consultant vest 50% on each of the first and second anniversaries of the grant date. The average grant date fair value per share for the RSUs during 2022 was $13.97. Unrecognized stock-based compensation expense related to these awards was approximately $939 as of June 30, 2023.

OnkosXcel profit sharing units

The table below summarizes activity relating to profits interests (the "profit sharing units" or "PSUs") associated with OnkosXcel.

	Number of units	Weighted average price per unit (in whole dollars)
Outstanding as of January 1, 2023	1,310	$ 5,506
Granted	30	$ 10,176
Outstanding as of June 30, 2023	1,340	
Vested units as of June 30, 2023	456	$ 5,516

The Company granted 1,340 individual (not in thousands) time-based PSUs related to OnkosXcel to certain employees and consultants of the Company in consideration for services provided to OnkosXcel. The PSUs represent indirect equity interests in OnkosXcel. All PSUs, other than those granted to certain executive employees of the Company, vest ratably over 48 months. PSUs granted to certain executive employees of the Company, vest ratably over 24 months.

The fair values of PSUs granted during 2023 of $8 per unit were estimated at the date of grant using a Black-Scholes option pricing model and assumptions below.

	2023 grant profit share unit valuation inputs
Expected volatility	97.4 %
Risk-free rate of interest	3.6 %
Expected dividend yield	— %
Expected term	5.8 years

Unrecognized stock-based compensation expense related to these awards was $3,833 at June 30, 2023.

OnkosXcel restricted stock units

The table below summarizes activity relating to restricted stock units associated with OnkosXcel (the "OnkosXcel RSUs").

	Number of units
Outstanding as of January 1, 2023	—
Granted	225
Outstanding as of June 30, 2023	225

During the six months ended June 30, 2023, the Company granted 225 individual (not in thousands) OnkosXcel RSUs to certain employees. 125 of the OnkosXcel RSUs vest upon the earlier to occur of (a) 180 days after an initial public offering of OnkosXcel, or (b) a change in control of OnkosXcel. The remaining OnkosXcel RSUs vest over four years, with 25% vesting at the one-year anniversary of the grant date and the balance vesting ratably over the remaining 12 quarters of the vesting period. The weighted average grant date fair value per unit for the OnkosXcel RSUs was approximately $10. Unrecognized stock-based compensation expense related to these awards was approximately $1,648 as of June 30, 2023.

Stock options

A summary of the Company's stock option activity for the six months ended June 30, 2023 is presented below.

	Number of shares		Weighted average price per share
Outstanding as of January 1, 2023	4,882	$	17.23
Granted	1,072	$	19.62
Forfeited	(34)	$	17.13
Cancelled	(7)	$	22.33
Exercised	(309)	$	1.65
Outstanding as of June 30, 2023	5,604	$	18.54
Options vested and exercisable as of June 30, 2023	3,214	$	16.43

As of June 30, 2023, the intrinsic value of options outstanding was $9,215. 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 six months ended June 30, 2023 was $5,928. The total intrinsic value of stock options exercisable as of June 30, 2023 was $7,903.

The weighted average grant date fair value per share of options granted during the six months ended June 30, 2023 was $15.74.

The weighted average grant date fair value per share of options vested as of June 30, 2023 was $12.19.

The weighted average remaining contractual life is 5.8 years for options exercisable as of June 30, 2023. The weighted average remaining contractual life was 7.2 years for options outstanding as of June 30, 2023.

Stock-Based Compensation

The fair value of BTI stock options granted during the six months ended June 30, 2023 and 2022 was estimated using the Black-Scholes pricing model with the following assumptions:

	Six months ended June 30, 2023			Six months ended June 30, 2022		
Expected term	5.5 years	-	6.1 years	6.1 years	-	6.1 years
Expected stock price volatility	96.6 %	-	97.9 %	92.7 %	-	95.6 %
Risk-free rate of interest	3.5 %	-	4.2 %	2.4 %	-	3.0 %
Expected dividend yield	0.0 %	-	0.0 %	0.0 %	-	0.0 %

In 2023, the Company began using the historical volatility of its common stock to estimate volatility. Prior to 2023, volatility was estimated using a combination of the historical volatility of publicly traded peer companies and that of the Company's common stock. The expected term of the awards is estimated based on the simplified method, which

calculates the expected term based upon the midpoint of the life 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 zero percent as the Company has no history of paying dividends nor does management expect to pay dividends over the contractual terms of these options. The risk-free interest rates are determined by reference to the U.S. Treasury yield curve in effect at the time of grant, with maturities approximating the expected term of the stock options. The fair value of the underlying common stock is generally determined as the closing price of the Company's common stock on The Nasdaq Capital Market on the grant date, with consideration of whether there is material nonpublic information that could impact that estimated fair value when it is released.

The Company recognized stock-based compensation expense related to awards issued under the 2017 Plan and the 2020 Plan, as well as the OnkosXcel RSUs and PSUs, of $6,124 and $4,482 for the three months ended June 30, 2023 and 2022, respectively, and $11,001 and $8,307 for the six months ended June 30, 2023 and 2022, respectively, which were comprised as follows:

	Three Months Ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Research and development	$ 1,898	$ 1,235	$ 3,195	$ 2,210
Selling, general and administrative	4,226	3,247	7,806	6,097
Total	$ 6,124	$ 4,482	$ 11,001	$ 8,307

Unrecognized compensation expense related to unvested BTI stock option awards as of June 30, 2023, was $25,301 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.

2020 Employee Stock Purchase Plan

The Company's 2020 Employee Stock Purchase Plan (the "ESPP") was also approved and became effective at the Company's 2020 annual meeting of stockholders on May 20, 2020. The ESPP is designed to assist eligible employees of the Company with the opportunity to purchase the Company's common stock at a discount through accumulated payroll deductions during successive offering periods. The aggregate number of shares that may be issued pursuant to rights granted under the ESPP is 100 shares of common stock. In addition, the number of shares available for issuance under the ESPP will increase on the first day of each calendar year, beginning on January 1, 2021 and ending on and including January 1, 2030, by a number of shares of common stock equal to the lesser of (a) 1% of the shares outstanding on the final day of the immediately preceding calendar year and (b) such smaller number of shares as determined by the Board of Directors. The number of shares that may be issued or transferred pursuant to rights granted under the component of the ESPP that is intended to qualify for favorable U.S. federal tax treatment under Section 423 of the Internal Revenue Code (the "Section 423 Component") shall not exceed 500 shares. The purchase price will be determined by the administrator of the ESPP and, for purposes of the Section 423 Component, shall not be less than 85% of the fair value of a share on the first trading day or on the last trading day of the applicable offering period, whichever is lower. The shares available for issuance under the ESPP increased by 281 shares and 280 shares on January 1, 2023 and 2022, respectively. To date, no shares have been sold under the ESPP. There were 905 shares available for issuance as of June 30, 2023.

Note 12. Leases

BTI leases office space for its corporate headquarters at 555 Long Wharf Drive, New Haven, Connecticut (the "HQ Lease") under an operating lease that expires in February 2026. The Company has an option to renew the HQ Lease for one additional five-year term. Payments under the HQ Lease are fixed.

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

The future minimum annual lease payments under operating leases, as of June 30, 2023, are as follows:

Year ending December 31,	Amount
Remainder of 2023	$ 187
2024	381
2025	391
2026	65
2027	—
Thereafter	—
Total lease payments	$ 1,024
Imputed interest	(75)
Total lease liability	$ 949
Less current portion of lease liability	(332)
Long-term portion of operating lease liability	$ 617

The current portion of the Company's operating lease liability of $332, as of June 30, 2023, is included in Other current liabilities on the Condensed Consolidated Balance Sheets.

Lease expense was $98 and $107 for the three months ended June 30, 2023 and 2022, respectively, and $197 and $204 for the six months ended June 30, 2023 and 2022, respectively.

Lease renewal options are not included in the ROU asset or lease liability.

Note 13. Fair Value Measurements

The Company groups its assets and liabilities measured at fair value in three levels based on the nature of the inputs and assumptions used to determine fair value. Refer to Note 3, *Summary of Significant Accounting Policies*, for additional information on the accounting policies related to fair value.

The carrying amounts of cash and cash equivalents, accounts receivable, net, and accounts payable approximate fair value due to the short-term nature of these instruments. As of June 30, 2023 and December 31, 2022, the Company had $124,654 and $191,022, respectively, primarily in money market funds that hold U.S. government cash equivalent instruments (included in cash and cash equivalents) which were valued based on Level 1 inputs. There were no transfers between levels within the hierarchy during the six months ended June 30, 2023 and the year ended December 31, 2022.

Derivative liabilities measured at fair value on a recurring basis are summarized below.

	Six months ended June 30, 2023				
	Fair Value	Level 1	Level 2	Level 3	Total
Derivative liability - Equity Investment Right	$ 1,188	$ —	$ —	$ 1,188	$ 1,188
Derivative liability - OnkosXcel Warrants	838	—	—	838	838
Total derivative liabilities	$ 2,026	$ —	$ —	$ 2,026	$ 2,026

Derivative liabilities are comprised of the OnkosXcel Warrants and Equity Investment Right held by the Lenders. The fair value of the derivative liabilities was determined using Monte Carlo simulation models for the Equity Investment Right, and Binomial Option Pricing and Distribution models for the OnkosXcel Warrants.

The following table presents changes in Level 3 liabilities measured at fair value for the six months ended June 30, 2023. Both observable and unobservable inputs were used to determine the fair value of positions that the Company has classified within the Level 3 category.

	Derivative liabilities
Balance - December 31, 2022	$ 2,343
Change in fair value	(317)
Balance - June 30, 2023	$ 2,026

The change in fair value of the derivative liabilities was reported in the Condensed Consolidated Statements of Operations as Other income, net, for the three and six months ended June 30, 2023.

Inputs used to calculate the estimated fair value of the Equity Investment Right at June 30, 2023 were as follows:

	Equity Investment Right
Strike price relative to volume weighted 30-day average	110.0 %
Volatility (annual)	97.3 %
Probability of exercise	88.4 %
Time period	3.8 years
Estimated premium to 30-day average	25.0 %
Discount rate	5.0 %

In estimating the fair value of the derivative liability related to the OnkosXcel Warrants, inputs included third-party fair value estimates of OnkosXcel limited liability company units along with the volatility of those units (which was set at 100% based on the historical volatility of the Company's stock), and the timing and probability of the relevant capital transactions occurring.

The estimated fair value of the Credit Agreement and RIFA as of June 30, 2023, were $55,897 and $28,794, respectively. Both observable and unobservable inputs were used to determine the fair value of long-term debt, which was classified within the Level 3 category.

The fair value of the BTI warrants issued in 2022, which is a non-recurring fair value, were determined as of the date of issuance using a Black-Scholes pricing model and the fair value of $3,245 was recorded as a component of stockholders' equity in Additional-paid-in-capital in the Condensed Consolidated Balance Sheets, with the offset recorded as a discount on the amounts funded under the OFA Facilities. This non-recurring measurement is classified as Level 3. The inputs used were a strike price of $20.04, the Company's stock price of $14.93, volatility of 95%, term of 7 years and risk-free rate of 2.95%.

Note 14. Net Loss Per Share

Basic and diluted net loss per share are as follows:

	Three Months Ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Net loss (numerator)	$ (53,515)	$ (37,670)	$ (106,311)	$ (69,142)
Weighted average shares (denominator)	29,187	27,989	28,903	27,985
Basic and diluted net loss per share	$ (1.83)	$ (1.35)	$ (3.68)	$ (2.47)

Potentially dilutive securities outstanding consists of stock options and RSUs. The Company had common stock equivalents outstanding as of June 30, 2023 and 2022 of 5,801 and 4,916 shares, respectively.

Note 15. 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. Other than the below, the Company is not currently subject to any matters where it believes there is a reasonable possibility that a material loss may be incurred.

On July 7, 2023, plaintiff Katelyn Martin filed a class action complaint against the Company and certain executives in the United States District Court for the District of Connecticut, captioned *Martin v. BioXcel Therapeutics, et al.*, 3:23-cv-00915 (D. Conn). The complaints generally allege violations of Sections 10(b) and 20A of the Securities and Exchange Act of 1934 (the "Exchange Act") and SEC Rule 10b-5 promulgated thereunder, based on certain public statements related to the development of BXCL501, TRANQUILITY II and TRANQUILITY III between December 15, 2021 and June 28, 2023. Pursuant to the Private Securities Litigation Reform Act, other investors may seek to serve as lead plaintiff and pursue these claims on behalf of a putative class of investors. The allegations and claims at issue in matter may be amended or supplemented in the future, including when a lead plaintiff is appointed by the Court. At this time, the Company does not believe the claims have merit, but the potential costs and liabilities associated with this litigation are uncertain.

In April 2022, the Company signed a commercial supply agreement that requires minimum annual payments for the first three years of the agreement that in aggregate total $10,000 for the three-year period.

Note 16. Subsequent Events

Costs Associated with Reprioritization Activities

On August 8, 2023, the Company's Board of Directors approved a broad-based strategic reprioritization (the "Reprioritization"). The Company has determined to take actions to reduce certain operational and workforce expenses that are no longer deemed core to ongoing operations in order to extend its cash runway and drive innovation and growth in high potential clinical development and value creating opportunities. These actions will include a shift in commercial strategy for IGALMI™ in the institutional setting, a reduction of in-hospital commercialization expenses, a suspension of programs no longer determined to be core to ongoing operations, and a prioritization of at-home treatment setting opportunities for BXCL501.

As part of this strategy, the Company's Board of Directors approved a reduction of approximately 50% of the Company's current workforce. The Company began notifying impacted employees of the Reprioritization on August 14, 2023. Annual operating expenses are expected to be reduced by approximately $80,000, and the Reprioritization initiatives are expected to extend the Company's cash runway into mid-2024. The Reprioritization is expected to be complete by the end of the third quarter of 2023. The Company is also in active negotiations with its strategic lenders to amend its existing financing agreements in order to extend its cash runway.

As a result of the Reprioritization, the Company estimates that it will incur approximately $7,000 to $8,000 in costs, consisting of severance and benefit payments, notice pay, and related expenses, all of which are expected to be paid in cash. The estimated costs that the Company expects to incur and the expected timing to complete the Reprioritization are subject to a number of assumptions, and actual results may differ. The Company may also incur other cash or non-cash charges or cash expenditures not currently contemplated due to events that may occur as a result of, or in association with, the Reprioritization and/or associated with the investigations and audits related to our TRANQUILITY II Phase 3 trial.

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 unaudited interim condensed consolidated financial statements and related notes appearing elsewhere in this report and the audited financial statements and related notes contained in our Annual Report on Form 10-K for the year ended December 31, 2022. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those discussed below and in the forward-looking statements. Factors that could cause or contribute to these differences include, without limitation, those discussed in this Management's Discussion and Analysis of Financial Condition and Results of Operations, those listed under "Summary Risk Factors," and those discussed in the section titled "Risk Factors" included in Part II, Item 1A. of this report. All dollar amounts in the below Management's Discussion and Analysis of Financial Condition and Results of Operations are presented in U.S. dollars, and all dollar and share amounts are presented in thousands, unless otherwise noted or the context otherwise provides.

Overview

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

Our most advanced neuroscience asset is BXCL501. In indications other than those approved by the United States ("U.S.") Food and Drug Administration ("FDA") as IGALMI™, BXCL501 is an investigational, proprietary, orally dissolving film formulation of dexmedetomidine (or "Dex") in development for the treatment of agitation associated with psychiatric and neurological disorders. Our most advanced immuno-oncology asset, BXCL701, is an investigational oral innate immune activator currently being developed as a potential therapy for the treatment of aggressive forms of prostate cancer, pancreatic cancer, and other solid and liquid tumors.

On April 6, 2022, we announced that the FDA approved IGALMI™ (dexmedetomidine or "Dex" and developed as BXCL501) sublingual film for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. IGALMI is approved to be self-administrated by patients under the supervision of a health care provider. On July 6, 2022, we announced that IGALMI was commercially available in doses of 120 and 180 microgram ("mcg") through the Company's third-party logistics provider and was available for order through wholesalers.

We are continuing to develop BXCL501 for the acute treatment of agitation associated with bipolar disorders or schizophrenia in the at-home setting and for the acute treatment of agitation associated with mild to moderate dementia due to probable Alzheimer's disease in the at-home setting or in assisted living facilities ("ALFs"). As described further below, we have recently deprioritized certain other indications of BXCL501, including as a potential adjunctive treatment for major depressive disorder ("MDD"), as well as our BXCL701 program.

In our SERENITY program, we are evaluating BXCL501 for use in the at-home setting for agitation associated with bipolar disorders or schizophrenia in our SERENITY III trial. We completed Part 1 of the SERENITY III trial and announced topline results on May 25, 2023. We initiated Part 2 of the trial in the second half of 2023, which is currently evaluating the safety of a 60 mcg dose with an optional second 60 mcg dose of BXCL501 in the at-home setting, and we plan to seek FDA feedback regarding a proposed amendment to the protocol to evaluate an 80 mcg dose of BXCL501 in this setting.

For our TRANQUILITY program, we have conducted clinical studies evaluating BXCL501 for the acute treatment of agitation associated with mild to moderate dementia in patients with probable Alzheimer's disease, who reside in ALFs and residential care settings and who required minimal assistance with activities of daily living. On June 29, 2023, we announced positive topline data from our TRANQUILITY II trial, as well as information regarding certain

investigator misconduct and noncompliance at one of the clinical trial sites. We were conducting the TRANQUILITY III clinical trial evaluating the potential for BXCL501 to treat acute agitation in patients with moderate to severe dementia associated with probable Alzheimer's disease living in nursing homes and who require moderate to full assistance with activities of daily living. Enrollment in TRANQUILITY III has been paused due to the much higher-than-expected background frequency of episodes of agitation experienced by the first several patients enrolled in the study.

Strategic Reprioritization

On August 8, 2023, the Company's Board of Directors approved a broad-based strategic reprioritization (the "Reprioritization"). The Company has determined to take actions to reduce certain operational and workforce expenses that are no longer deemed core to ongoing operations in order to extend its cash runway and drive innovation and growth in high potential clinical development and value creating opportunities. These actions include a shift in commercial strategy for IGALMI™ in the institutional setting as described under "IGALMI Commercial Progress" below, a reduction of in-hospital commercialization expenses, a de-prioritization of programs no longer determined to be core to ongoing operations as described under "Our Neuroscience Clinical Programs" and "Our Immuno-Oncology Clinical Programs", and a prioritization on at-home treatment setting opportunities for BXCL501. As part of this strategy, the Company's Board of Directors approved a reduction of approximately 50% of the Company's current workforce. The Company began notifying impacted employees of the Reprioritization on August 14, 2023. Annual operating expenses are expected to be reduced by approximately $80 million, and the Reprioritization initiatives are expected to extend the Company's cash runway into mid-2024. The Reprioritization is expected to be complete by the end of the third quarter of 2023.

As a result of the Reprioritization, the Company estimates that it will incur approximately $7 million to $8 million in aggregate costs, consisting of severance and benefit payments, notice pay, and related expenses, all of which are expected to be paid in cash. The estimated costs that the Company expects to incur and the expected timing to complete the Reprioritization are subject to a number of assumptions, and actual results may differ. The Company may also incur other cash or non-cash charges or cash expenditures not currently contemplated due to events that may occur as a result of, or in association with, the Reprioritization.

IGALMI Commercial Progress

Commercialization of IGALMI continued to build on its momentum through the second quarter of 2023, climbing to over 185 formulary approvals and unlocking up to $80 million in market potential. As of June 30, 2023, there are more than 650 additional formulary reviews scheduled, representing up to an additional $275 million in bipolar and schizophrenia agitation market opportunity. We believe this market opportunity remains available to us under our new sales strategy.

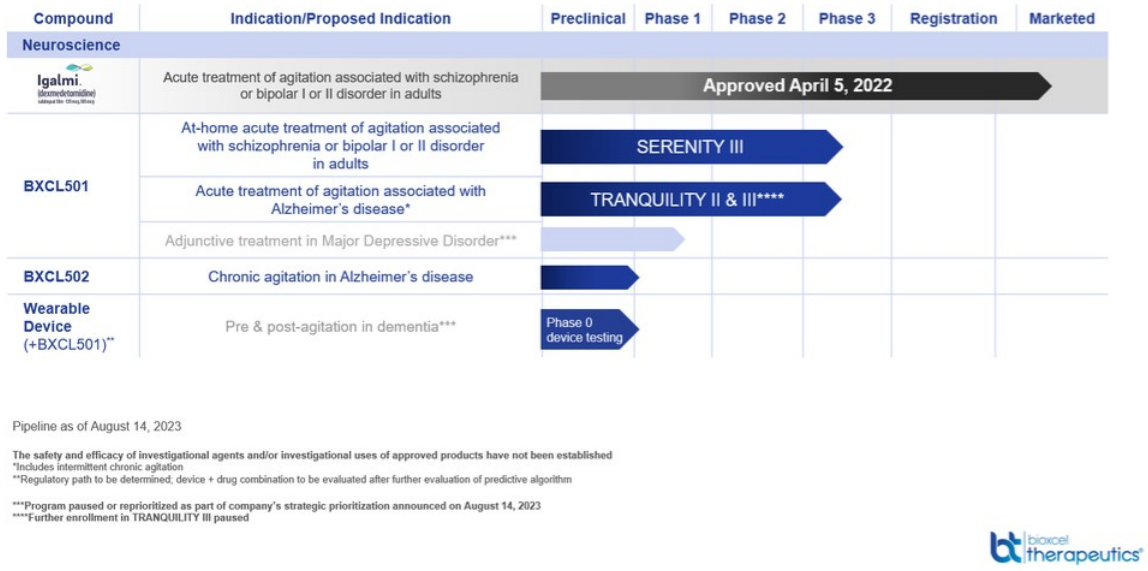
Recent substantial purchases through the contracting process resulted in the doubling of second quarter 2023 revenues sequentially, a process which the Company will emphasize moving forward.

- The commercial realignment is designed to reduce the direct sales footprint and marketing efforts and pivot focus to market access with contracting by existing customers and large systems (Integrated Delivery Networks or IDNs).

- The Company plans to continue to support hospitals purchasing IGALMI and those with positive formulary status through trade, distribution, and medical support.

Our Neuroscience Clinical Programs

The following is a summary of the status of our major neuroscience clinical development programs as of the date of this Quarterly Report on Form 10-Q. With our Reprioritization announcement on August 14, 2023, some of our trials and programs have been paused or deprioritized as noted below.



Neuroscience Program

BXCL501 Development

In indications other than those approved by the FDA as IGALMI, BXCL501 remains an investigational, proprietary, orally dissolving film formulation of Dex, a selective alpha-2 receptor agonist, targeting symptoms from stress-related behaviors such as agitation. BXCL501 is our most advanced neuroscience clinical program, being evaluated for at-home acute treatment of agitation related to bipolar disorders or schizophrenia and for the acute treatment of agitation related to mild to moderate Alzheimer's disease in ALFs or in the at-home setting. As noted above, the Company has recently implemented a shift in commercial strategy for IGALMI™ in the institutional setting, a reduction of in-hospital commercialization expenses, a suspension of programs no longer deemed core to the Company's business, and a shift to focus on the development of BXCL501 for use in the at-home and the assisted living facility setting in the treatment of agitation in schizophrenia, bipolar disorders, and in ADA.

As a selective adrenergic agent with a sublingual or buccal route of administration, BXCL501 is designed to be easily administered and, compared with medications that may take days or weeks, has shown a relatively rapid onset of action in multiple clinical trials, including those studying patients with schizophrenia, bipolar disorders, and Alzheimer's disease. We believe results from these studies suggest that BXCL501 has the potential to reduce agitation without producing excessive sedation. We also believe BXCL501 is highly differentiated from antipsychotics, which are currently used as first-line standard-of-care treatments despite often producing unwanted side effects such as excessive sedation or extra pyramidal motor effects. 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 Program: Agitation Associated with Bipolar Disorders or Schizophrenia (At-Home Use)

We are currently evaluating the potential at-home use of BXCL501 in patients with agitation associated with bipolar disorders or schizophrenia in our SERENITY III study, which consists of two parts. The first part was comparable to our pivotal SERENITY I and II studies. Using similar inclusion and exclusion criterion under a well-controlled in-patient setting, acutely agitated patients with schizophrenia or bipolar disorders were randomized to self-

administer either 60 mcg of BXCL501 or placebo, in a double-blind placebo-controlled trial SERENITY III. The primary endpoint of Part I was efficacy, as measured by the change in PEC score change from baseline at two hours post-dose. The secondary objectives of Part I were safety and tolerability.

On May 25, 2023, we reported topline results from Part 1 of the study. Although the trial did not meet its primary efficacy endpoint, we believe the efficacy results, observed with the 60 mcg dose, representing half of the lowest approved dose of IGALMI for in-patient use (120 mcg), were promising. Specifically, greater than 50% of individuals were responders, defined as those patients who achieved a 40% or greater reduction in PEC score. Further, this population responder rate was consistent and dose-proportionate to the same response rates observed in the larger SERENITY I and II trials. Although the primary efficacy endpoint as a group mean change was not statistically significant at the primary endpoint at 2 hours (p=0.077), BXCL501 statistically separated from placebo at 4 hours (p=0.049).

SERENITY III Part 2 is currently underway using a 60 mcg dose with an optional second 60 mcg dose, while pharmacokinetic and pharmacodynamic modeling suggested that use of an 80 mcg dose of BXCL501 could provide an optimal balance between safety and efficacy for at-home use. We believe the evaluation of an 80 mcg dose is further supported by our previous clinical experience with this dose during our Phase 1b trial in schizophrenia patients with agitation. We believe the totality of evidence provides support for evaluating this dose. Further, we plan to meet with FDA to discuss a protocol amendment to the ongoing SERENITY III Part 2 study to replace the current 60 mcg dosing regimen with a single 80 mcg dose. Part 2 is primarily intended to evaluate safety of BXCL501 over 12 weeks when used as needed for episodes of agitation associated with schizophrenia and bipolar at home. The primary objective is to describe the incidence of treatment-emergent adverse events. The primary endpoint of the trial is a comparison of serious adverse events and treatment-emergent adverse events as compared to placebo, and the secondary endpoints for Part 2 include a number of efficacy assessments.

As we continue to conduct our SERENITY III trial, we are also planning a meeting to seek alignment with the FDA on the data package necessary to support approval of a supplemental new drug application (“sNDA”) to expand the currently approved label for IGALMI to include at-home use of an 80 mcg dose in a non-medically supervised setting, including whether we may need to conduct a further efficacy trial to evaluate the 80 mcg dose.

TRANQUILITY Program: Agitation Associated with Alzheimer’s Disease

The TRANQUILITY I Part A study of agitation in dementia concluded with a total of four sites enrolling 46 subjects. Part B tested the 40 mcg dose versus placebo. The purpose of enrolling this additional cohort was to gather further evidence supporting dose selection and statistical powering of multicenter Phase 3 pivotal trials. All patients were able to take the film themselves and properly place it. There were no serious adverse events (“SAEs”) related to the drug, and no falls, loss of consciousness, or syncopal events reported. There were also no local tolerability issues. The adverse events (“AEs”) observed for 40mcg were consistent with those previously observed for 30 mcg, 60 mcg, and placebo doses. The incidence of individual and categorical AEs for the 40 mcg dose were lower than the 60 mcg group and similar to the 30mcg dose group.

Efficacy was measured by the change from pre-dose baseline Positive and Negative Syndrome Scale Excitatory Component (“PEC”) total score at two hours, the same primary endpoint utilized in prior pivotal trials of BXCL501. The 40 mcg dose showed statistically significant reductions in PEC total score at two hours and demonstrated statistically significant separation from placebo as early as one hour. The magnitude of change in PEC total score was greater for the 40 mcg dose than that of 30 mcg and somewhat less than the 60 mcg dose in previous cohorts. Overall, we believe the 40 mcg data support continued evaluation of both 40 mcg and 60 mcg doses in Phase 3 pivotal trials.

On December 15, 2021, after our initial Breakthrough Therapy designation meetings with the FDA, we announced the initiation of our program to evaluate BXCL501 for the acute treatment of agitation associated with dementia in Alzheimer’s patients. The program’s two studies, TRANQUILITY II and TRANQUILITY III, are designed to evaluate the safety and efficacy of BXCL501 in adults 65 years and older across the range of illness including mild, moderate, and severe dementia in ALFs and residential care settings.

On June 29, 2023, the Company announced positive topline results from TRANQUILITY II, a randomized, double-blind, placebo-controlled, parallel group trial that evaluated the safety and efficacy of BXCL501 for the acute treatment of Alzheimer's-related agitation in adults 65 years and older in ALFs and residential care settings who required minimal assistance with activities of daily living. The trial dosed 149 patients with mild to moderate dementia. Randomized patients self-administered 40 mcg or 60 mcg of BXCL501 or placebo for agitation episodes that occurred over a 12-week period. The primary endpoint was the change from pre-dose in PEC total score at 2 hours post-dose for the first treated episode of agitation. The key secondary efficacy endpoints were PEC change from pre-dose at 1-hour post-dose of study treatment for the first treated episode of agitation, and PEC change from pre-dose at 30 minutes post-dose of study treatment for the first treated episode of agitation.

The Phase 3 trial met its primary efficacy endpoint with the 60 mcg dose; a statistically significant and clinically meaningful 7.5 point reduction from baseline in Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score was observed at 2 hours versus 5.4 with placebo (p=0.0112). The 60 mcg dose also met the first key secondary endpoint of reducing agitation symptoms at 1 hour during the first episode of agitation (p=0.0185) but did not meet the other key secondary endpoint of change from baseline in PEC score at 30 minutes.

Efficacy for this dose was supported by several secondary measures, including CGI-Improvement and Agitation-Calmness Evaluation Scale. Most patients (76%) responded to the first 60 mcg dose and were determined to be "Very Much" or "Much Improved" (CGI-I of 1 or 2, respectively) compared to 50% with placebo. The primary endpoint was not met for the 40 mcg dose, with a 5.7 point reduction from baseline in PEC score.

On June 29, 2023, we also announced that we had learned that an investigator in this study who enrolled approximately 40% of the patients engaged in misconduct, and we are in the process of conducting an investigation into certain data integrity issues related to the TRANQUILITY II Phase 3 clinical trial and a number of independent third parties have been retained to conduct audits of the data from that investigator's clinical trial site to assess their integrity, reliability, and usability, as discussed further in risk factor below entitled: "Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease."

We have also been conducting the TRANQUILITY III trial to evaluate the safety and efficacy of BXCL501 in patients residing predominantly in nursing homes with moderate to severe dementia who require moderate or greater assistance with activities of daily living. We have elected to halt additional enrollment in this Phase 3 trial in patients with agitation associated with moderate to severe dementia with probable Alzheimer's disease in nursing homes; enrolled patients are continuing the 12-week treatment period. The initial enrolled patients were observed to have more frequent episodes of agitation than originally anticipated, suggesting that agitation may present chronically in this population. Due to the chronic nature of agitation episodes observed in this population thus far, we believe that continued evaluation of BXCL501 in this population would require a different development program targeting more frequent or chronic use. We have chosen to focus our development efforts on the urgent need for episodic treatment in the ALF and at-home setting, consistent with the Breakthrough Therapy designation FDA has granted to BXCL501 for acute treatment of agitation associated with dementia.

We have requested a meeting with the FDA in an effort to reach alignment regarding the data needed to submit an sNDA seeking approval for BXCL501 for the acute treatment of agitation in mild to moderate dementia due to probable Alzheimer's disease. Specifically, we have requested feedback from the FDA as to whether the current data package consisting of TRANQUILITY I and II (subject to completion of the data audit), along with the clinical pharmacology and toxicology programs previously discussed with the FDA, may be sufficient to support an sNDA submission for the use of BXCL501 to treat agitation in patients with mild to moderate dementia due to probable Alzheimer's disease in either the at-home or ALF setting, or if not, what additional data would be required.

We are also evaluating the safety, tolerability, and pharmacokinetic characterization of single and multiple doses of BXCL501 in elderly subjects 65 years of age and older in a Phase 1 study. Quetiapine, an anti-psychotic drug widely prescribed in elderly patients, has been included as an active comparator. This study is also exploring the cognitive effects of daily dosing of BXCL501 and quetiapine. All dosing cohorts have been completed, including cohorts receiving 40 mcg BXCL501 (or placebo) once daily for seven days, and a cohort receiving 60 mcg BXCL501 (or

placebo) or quetiapine once daily for seven days. We expect that the results of this study will be available in the second half of 2023.

Adjunctive treatment in Major Depressive Disorder ("MDD")

We were previously evaluating BXCL501 as an adjunctive treatment for MDD. The initial clinical study in this program was a double-blind, placebo-controlled, multiple ascending dose ("MAD") trial to evaluate the safety and tolerability of daily doses of BXCL501 in healthy volunteers.

On May 16, 2023, we reported positive topline results from a MAD study. The MAD study enrolled 125 healthy adult volunteers across seven different cohorts in a 2:1 randomization to BXCL501 or placebo film. Healthy volunteers were dosed for 7 consecutive days. Both safety and pharmacokinetics were assessed. The study included 7 cohorts. Four distinct cohorts received 30 mcg, 60 mcg, 80 mcg, or 120 mcg doses of BXCL501 or placebo once daily. Two additional dosing cohorts received twice-daily (BID) BXCL501 at either 30 mcg in the morning and 60 mcg in the evening, or 40 mcg in the morning and 80 mcg in the evening. The final escalation cohort evaluated BXCL501 at 60 mcg in the morning and 80 mcg in the evening in combination with 30 mg of duloxetine BID. BXCL501 was generally well tolerated across all dosing cohorts. Based upon pre-specified stopping criteria, a maximum tolerated dose was not reached. All adverse events were reported as mild or moderate.

As part of its strategic Reprioritization, the Company is pausing its plan to develop a Phase 2 human proof-of-concept (POC) trial design to investigate BXCL501 as an adjunctive treatment and its potential accelerant effect in combination with first-line selective serotonin reuptake inhibitors (SSRIs) or serotonin-norepinephrine reuptake inhibitors.

Pediatric Study

In June 2021, we initiated a global clinical trial designed to evaluate the safety and efficacy of BXCL501 in the acute treatment of agitation associated with pediatric schizophrenia and bipolar disorders, in part to fulfill pediatric study requirements agreed to with the FDA in connection with IGALMI's approval. The trial protocol has been reviewed by the FDA, as well as by the European Medicines Agency, to fulfill potential commitments to study the effects of BXCL501 in pediatric patients ages 13 to 17 with schizophrenia and ages 10 to 17 with bipolar disorders. Enrollment of patients with schizophrenia, schizoaffective disorder, bipolar I, and bipolar II disorder is ongoing in this multisite, double-blind, placebo-controlled parallel group trial. Approximately 43% of the 150 total subjects have been enrolled in the U.S. and several European sites are planned to initiate enrollment in the second half of 2023. Similar to our registration trials in schizophrenia and bipolar disorder (SERENITY I and II), the primary endpoint is the change from baseline PEC total score at two hours. This program remains active following the Reprioritization.

Additional Neuroscience Opportunities

BXCL501 Pipeline Opportunities for Franchise Expansion

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.

Government-Supported Investigator-Initiated Trial Programs

The Company has been awarded key opportunities for the development of BXCL501 in post-traumatic stress disorder ("PTSD"), alcohol use disorder ("AUD"), and opioid use disorder ("OUD"). These are being funded through Cooperative Agreements with the U.S. Department of Defense Congressionally Directed Medical Research Program and National Institute on Drug Abuse. Clinical and regulatory responsibilities are led by clinical researchers and regulatory staff at the Veterans Affairs Connecticut Healthcare System, Yale University Medical School, RTI International, Columbia University New York State Psychiatric Institute, and the National Institute on Drug Abuse. The Company has retained all rights to commercialization of BXCL501 in all potential indications evaluated in clinical trials supported by the U.S. government.

Alcohol Use Disorder with Comorbid Post-traumatic Stress Disorder Program

In December 2020, the Veterans Affairs Connecticut Healthcare System and Yale University Medical School were awarded a grant by the U.S. Department of Defense's Congressionally Directed Medical Research Program with the overall objective to evaluate BXCL501 in patients who suffer from AUD with comorbid PTSD. The Company provided BXCL501 for the inpatient Alcohol Interaction Study, which has been completed. We understand that Yale University Medical School will be submitting an Investigational New Drug ("IND") application to evaluate the effects of up to 80 mcg of BXCL501 per day for 28 days on alcohol consumption, PTSD symptoms, cognitive function, memory, sleep, and mood in patients diagnosed with mild, moderate, or severe AUD and who meet Criterion A for comorbid PTSD. This program is being funded by the Pharmacotherapies for Alcohol and Substance Use Disorders Alliance (funded through a Cooperative Agreement between the U.S. Department of Defense Congressionally Directed Medical Research Program and RTI International). We believe the results from this study will be used to inform a Phase 3 study intended to commence with support by the Pharmacotherapies for Alcohol and Substance Use Disorders Alliance.

Opioid Use Disorder Program

As announced on August 1, 2022, the National Institute on Drug Abuse awarded a grant to Columbia University to fund clinical testing of BXCL501 as a potential treatment for opioid withdrawal in patients diagnosed with OUD. The 160-patient, three-site, four-arm study is a randomized, double-blind, double-dummy inpatient study comparing BXCL501 (180 mcg and 240 mcg BID), lofexidine (as a positive control), and placebo. The goal is to evaluate the safety and efficacy of BXCL501 relative to lofexidine and placebo in subjects with OUD. We understand that all three sites have recruited, enrolled, and dosed patients diagnosed with OUD who are physically dependent on opioids, including prescription opioids. The Company is currently supplying the drug product for the conduct of this study. The study will be used to select a recommended dose of BXCL501 to compare to lofexidine in a later Phase 3 study expected to commence with support from the National Institute on Drug Abuse.

Algorithms for Wearable Technologies

The Company completed a healthy volunteer study designed to train an algorithm to detect a hyper-aroused state. Hyperarousal often precedes agitated behaviors. In hyper-aroused healthy volunteers, robust signals were measured using wearable technology (phones and watches). As part of its strategic Reprioritization, the Company is deprioritizing its plan to develop wearable technology.

BXCL502 Development

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

Other Product Candidates Leveraging the AI Platform

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 increased levels of healthcare burden. We are also using AI approaches and machine learning to identify new candidates for rare neurological diseases and to reinnovate late stage drug candidates, such as BXCL503 and BXCL504.

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 (similar to BXCL501). We are also developing an AI-based research and development platform to help identify potential product candidates and indications across a range of treatment areas.

OnkosXcel Therapeutics, LLC

On April 19, 2022, we announced the formation of a wholly owned, clinical-stage subsidiary, OnkosXcel Therapeutics, LLC ("OnkosXcel"), to develop potentially transformative medicines in oncology. OnkosXcel uses proprietary AI capabilities to drive the capital-efficient development of innovative anti-cancer therapeutics. On March 14, 2023 we announced that OnkosXcel had confidentially submitted a draft registration statement on Form S-1 with the SEC relating to the proposed initial public offering of its common stock following its conversion into a corporation. The Company is continuing to evaluate strategic options for OnkosXcel. With the Company's Reprioritization announcement on August 14, 2023, further work on the immuno-oncology programs described below have generally been paused, except as noted below.

Our approach to drug discovery leverages the application and methodology of EvolverAI, a proprietary AI-based research and development platform utilized in the successful development of IGALMI, with the aim of efficiently identifying and developing immuno-oncology product candidates. We believe that BXCL701 reflects the potential of this discovery approach in immuno-oncology. BXCL701 is an investigational, oral innate immune activator that demonstrated a 25% composite response rate in a Phase 2a clinical trial to treat patients with small cell neuroendocrine ("SCNC") phenotype metastatic castration-resistant prostate cancer ("mCRPC"). We intend to finalize a registrational trial design in mCRPC patients with SCNC phenotype following planned meetings with the FDA in the second half of 2023, however, the start of such trial is paused following the Company's Reprioritization.

mCRPC is often characterized as a "cold" tumor, which is a tumor with an immunosuppressive tumor microenvironment ("TME") and poor immune cell infiltration. Currently approved checkpoint inhibitors ("CPIs") which target programmed cell death 1 ("PD-1"), or cytotoxic T-lymphocyte-associated protein 4, have failed to demonstrate meaningful single-agent activity against such difficult-to-treat tumor types, including mCRPC. BXCL701 is designed to promote an immune-induced inflammatory response in the TME primarily via inhibition of dipeptidyl peptidases ("DPP") 8 and 9, which we believe can provide for enhanced CPI therapeutic utility. We believe that BXCL701 can potentially provide significant benefits for approximately 20% of the estimated 288,300 men who will be diagnosed with prostate cancer in the U.S. in 2023 who are expected to progress to the more aggressive mCRPC form of the disease, including approximately 20%, or 11,500, of those patients who will develop the SCNC phenotype, for which there are currently limited treatment options.

Our Immuno-Oncology Clinical Programs

Below is a summary of the status of our immuno-oncology clinical development programs as of the date of this Quarterly Report on Form 10-Q. We believe our product candidates, if successfully developed and approved, have the potential to become compelling treatment options for their respective indications. With the Company's Reprioritization announcement on August 14, 2023, further work on its immuno-oncology program has been paused, other than as noted below.

Leveraging the insights enabled by the application and methodology of EvolverAI, a proprietary AI-based platform used to identify novel therapeutic uses for approved therapeutics and product candidates in clinical evaluation, and our internal industry expertise, we are pursuing two proprietary discovery programs to advance our goal of developing anti-cancer therapeutics. The first program, which encompasses BXCL701 across a range of indications, is based on the application of innate immune modulation technology. This program has been constructed to embrace key distinguishing characteristics of the innate immune system and we believe it is supported by our development efforts. This approach has driven the development of BXCL701, which we are currently evaluating in a Phase 2 clinical trial as a potential treatment for mCRPC with SCNC phenotype. Fundamental to the innate immune modulation program is BXCL701's potential to:

- Convert cold tumors into ones sensitive to CPIs;
- Enhance hot tumors' response rate and depth of response to CPIs; and
- Restore CPE sensitivity to tumors that had previously been responsive.

BXCL701 Innate Immune Activator

BXCL701 is an oral small molecule inhibitor of a class of enzymes called DPPs, specifically DPP8/9 and DPP4. Inhibition of DPP8/9 initiates the activation of the inflammasome and ultimately the activation of the innate immune system. Key characteristics of BXCL701 include:

- Orally bioavailable, potentially sole inhibitor of both DPP8/9 and DPP4, key regulators of the inflammasome directed innate immune response, currently in clinical development for cancer.
- Novel proposed mechanism of action may complement CPI activity, enabling therapeutic access to immunologically cold tumors as well as other difficult-to-treat cancers, including relapsed or refractory tumor types.
- Phase 2 clinical proof-of-concept achieved in treating mCRPC patients with either SCNC or adenocarcinoma phenotype.

Initial focus on mCRPC with SCNC phenotype is designed to provide for a more efficient clinical development pathway than current industry standards.

Immuno-Oncology Clinical Trials

BXCL701 as a Potential Treatment for mCRPC

We believe BXCL701 may have utility in stimulating increased activation, proliferation, and infiltration of tumor cells by immune effector cells, enabling its potential use in combination with currently approved CPIs to treat cold tumors, such as mCRPC. We elected to pursue mCRPC as an indication for BXCL701 due to its enrichment for DPP mutations, which are especially prevalent in tumors with SCNC phenotype.

BXCL701 is being evaluated in a Phase 1b/2a clinical proof-of-concept trial that we are sponsoring to investigate its potential efficacy when used in combination with pembrolizumab. Enrollment in this trial is complete.

The Phase 2a portion of the trial was segregated into two 28-patient trial cohorts, one cohort consisting of mCRPC patients with SCNC phenotype and a second cohort consisting of mCRPC patients with adenocarcinoma phenotype. Initially, we focused on mCRPC with SCNC phenotype as the primary patient population for BXCL701, since DPP9 is amplified in approximately 17% of treatment-emergent mCRPC with SCNC phenotype, compared to 5% or less in the broader prostate cancer population. However, we also observed responses in mCRPC patients with adenocarcinoma phenotype who were microsatellite stable in the Phase 1b portion of the trial. On this basis, we widened our Phase 2a trial to include relapsed mCRPC patients with either SCNC or adenocarcinoma phenotype. Both cohorts employed a Simon two-stage trial design of 15 trial participants followed by 13 additional patients. The primary endpoint of the Phase 2a portion of this trial was a composite response rate, determined as either a RECIST 1.1 response (defined as a reduction in RECIST score of 30% or more), and/or a reduction in prostate specific antigen (“PSA”) level of 50% or more, and/or a conversion in circulating tumor cells (“CTCs”) from 5 or more CTCs/7.5 milliliter (“ml”) to less than 5 CTCs/7.5 ml. Secondary endpoints included duration of response, progression-free survival, changes in circulating cytokines, and certain disease-specific biomarkers.

Final Phase 2a results for the SCNC cohort were presented at the 2023 Genitourinary Cancers Symposium of the American Society of Clinical Oncology. BXCL701 in combination with pembrolizumab demonstrated a 25% (seven out of 28 evaluable patients) composite response rate in mCRPC patients with SCNC phenotype, for whom there is no standard of care. As of December 19, 2022, the median duration of response for the seven composite responders was six-plus months (range 1.3 to 17.4 months). Five of these responders were RECIST 1.1 responders (four confirmed responses and one unconfirmed response) with decreases in tumor size ranging from 42% to 67% and a median duration of response of six-plus months (range 1.3 to 17.4 months). The sixth responder was a CTC and PSA_{50} responder, with a PSA decrease of 73%. The seventh responder was a PSA_{50} responder, with a PSA decrease of 50%.

We received initial comments from the FDA on our proposed clinical development plan, and we plan to meet with the FDA in the second half of 2023 to agree to a potential pivotal trial design. However, the start of such trial is paused following the Company's Reprioritization.

BXCL701 as a Potential Treatment for Small Cell Lung Cancer

The American Cancer Society estimates that in 2023, approximately 35,751 cases of small cell lung cancer ("SCLC") will be diagnosed in the U.S. Approximately 60 to 70% of these patients present with extensive disease, and first-line therapy for a majority of these patients involves the combination of a CPI with platinum-based chemotherapy or etoposide.

We are encouraged by the therapeutic potential of BXCL701 for SCLC given the activity it has demonstrated in the ongoing SCNC clinical trial, and we plan to initiate clinical trials targeting this indication. We are finalizing the protocol for a Phase 1b/2 trial design to be a dose-escalation safety lead-in to establish a recommended Phase 2 dose ("RP2D"). However, the start of such trial is paused following the Company's Reprioritization.

BXCL701 as a Potential Treatment for Other Cancers

In addition to its potential use in combination with CPIs to treat mCRPC, an immunologically cold tumor, we are developing BXCL701 as a therapeutic for pancreatic cancer, and other solid tumors with greater, or "non-cold," immunological activity that are nonetheless regarded as difficult-to-treat, and hematological malignancies. We believe the synergistic potential of BXCL701 and CPIs, when administered in combination, could increase cancer cell susceptibility to an enhanced immune response, potentially increasing the clinical benefit of CPIs, whose single-agent efficacy in treating these tumor types is generally viewed to be limited to between 13% and 30% of cancer patients and whose duration of response to treatment is often short. As such, we envision the potential therapeutic benefit of BXCL701 increasing the sensitivity of cold tumors to CPI therapy, enabling the potential treatment of a range of cancers including pancreatic cancer, breast cancer, colorectal cancer, and ovarian cancer, as well as enhancing the depth of response to CPIs in other cancers. In addition, based on the preclinical observation that BXCL701 showed direct cytotoxic activity against certain leukemic cells, we have initiated clinical development targeting relapsed or refractory acute myeloid leukemia ("AML").

Pancreatic Cancer

The American Cancer Society estimates that in 2023, approximately 64,050 cases of pancreatic cancer will be diagnosed in the U.S. We are supporting a Phase 2 Investigator Sponsored Trial ("IST") sponsored by Georgetown Lombardi Comprehensive Cancer Center designed to evaluate the use of BXCL701 along with pembrolizumab to treat pancreatic cancer. Few therapeutic options are available for patients with this indication, which has a five-year survival rate of less than 10%, among the lowest of all cancers. Pancreatic cancer has among the highest levels of overexpression and amplification of DPPs. Preclinical models demonstrated synergy between DPP inhibition with BXCL701 and anti-PD-1 antibody in the pancreatic cancer tumor microenvironment. Based on these preclinical observations, Georgetown Lombardi intends to assess the safety of BXCL701 when administered in combination with pembrolizumab, as well as estimate the 18-week progression-free survival rate. This trial has begun and remains ongoing.

Relapsed or Refractory AML

The American Cancer Society estimates that in 2023, approximately 20,380 new cases of AML will be diagnosed in the U.S. We are supporting a Phase 1b IST sponsored by Dana-Farber Cancer Institute, designed to evaluate the use of BXCL701, along with the current standard of care to treat relapsed or refractory AML. We believe that pyroptosis triggered by BXCL701 may provide potent single agent cytotoxicity directed toward AML. We also believe that DPP9 copy number may provide an actionable biomarker, as high copy number has been observed to correlate with BXCL701 toxicity in human AML cell lines. DPP8/9 inhibition has been shown to be cytotoxic to THP-1 cells, monocytic cancer cells cultured from a patient with AML, but not other cell lines, suggesting a specific vulnerability of AML to these inhibitors that we believe can be exploited for therapeutic benefit. Based on these preclinical observations, Dana-Farber has initiated a Phase 1b trial to determine the maximum tolerated dose or the RP2D, and to assess the safety of

BXCL701 as a single agent. This trial began in the first quarter of 2023. Subject to successful completion of this Phase 1b trial, we anticipate that Dana-Farber will conduct additional studies to determine BXCL701's objective response rate in AML in combination with the standard of care.

Other Potential Anti-Cancer Programs

We collaborated with the University of Texas MD Anderson Cancer Center in a Phase 2a IST to evaluate the potential efficacy of BXCL701 administered in combination with pembrolizumab in patients with advanced solid cancer. The design of this open label trial included two cohorts and incorporated a two-stage configuration, which allowed for an expansion of patient enrollment to a total of 17 patients in each cohort if a RECIST 1.1 complete response or partial response was observed in at least one of the initial nine patients. The first cohort enrolled patients who previously had not received CPI therapy, with a second cohort consisting of patients who were either refractory to CPI therapy or had relapsed while on CPI therapy, meaning that no further response to CPI treatment was anticipated among patients in the second cohort. Trial participants received 200 mg of pembrolizumab on day one of a 21-day cycle, with 0.2 mg BXCL701 administered twice-daily (“BID”), on days one through seven, the dose increasing to 0.3 mg BID on days eight through 14. Evaluable trial participants were required to receive a minimum of two treatment cycles. A preliminary assessment conducted after completion of the first stage of the study of BXCL701 dosed in combination with a CPI, noted responses in two patients, one in each of the CPI naïve and the CPI refractory/relapsed cohorts. Responses included a partial response in a patient with endometrial carcinoma who was CPI-naïve, microsatellite stable, and PD-L1 negative (CPS <1), and a partial response in a CPI-refractory patient with uveal melanoma. These preliminary results were presented at the 2021 American Society of Clinical Oncology annual meeting. Patient enrollment in this trial was completed in the third quarter of 2022.

We believe BXCL701 may have potential application in breast cancer, as its use in combination with monoclonal antibody therapy generated encouraging in vivo data in a preclinical disease model where enhanced antibody-dependent cellular cytotoxicity was observed.

The FDA has granted BXCL701 orphan drug designation for the treatment of AML, Stage IIb to IV melanoma, pancreatic cancer, and soft tissue sarcoma. As we consider BXCL701’s therapeutic potential for additional indications that represent unmet medical needs, we intend to apply for additional orphan drug designations for BXCL701.

Biomarker Development Initiatives Intended to Complement BXCL701 Administration

We are also actively engaged in the identification and development of predictive biomarkers that we believe could be used in conjunction with BXCL701 to predict the likelihood of patient response to therapy across the range of targeted indications. Based on preliminary data from AML patients, we believe DPP9 copy number could correlate to BXCL701 response rate, with a greater likelihood of BXCL701 cytotoxicity in patients with increased DPP9 copy number. We are pursuing its use in our biomarker discovery activities as a potential companion diagnostic.

BXCL701 Lifecycle Management Considerations

We envision employing computational and medicinal chemistry approaches to advance development of a next-generation BXCL701 molecule to introduce enhanced life cycle management capabilities. We anticipate that a next-generation molecule may embrace characteristics such as the inhibition of DPP8/9, while providing an improved orally bioavailable pharmacokinetic profile, including a linear pharmacokinetic, a half-life of between 12 and 48 hours, with multiple mechanisms of elimination that are clearly understood and are not burdened with potential drug-drug interaction liabilities. This next generation molecule would be intended to demonstrate anti-cancer activity as either a monotherapy or in combination with an approved CPI.

Intellectual Property

Our policy is to protect and enhance the proprietary technologies, inventions, and improvements that are commercially important to our business by filing patent applications in the U.S. and other jurisdictions related to our proprietary technology, inventions, improvements, and product candidates. We also rely on trademarks, trade secrets, and know-how relating to our proprietary technologies and product candidates, continuing innovation, and in-licensing technology and products. This reliance is expected to develop, maintain, and strengthen our proprietary position for

novel therapeutics and novel formulations of existing therapeutics across multiple therapeutic areas. We also plan to rely on data exclusivity, market exclusivity, and patent term extensions when available.

Patent Portfolio

We have multiple patent families filed to protect our Neuroscience program, including BXCL501. As of June 30, 2023, our neuroscience patent portfolio included four Patent Cooperation Treaty ("PCT") applications, 19 U.S. utility applications, nine U.S. provisional, five issued U.S. utility patents, 104 pending non-U.S. utility applications, 11 allowed or granted non-U.S. patents (including three in Japan), one pending U.S. design patent application, and 34 allowed or registered design patents (including two in Japan). Four U.S. utility patents, directed to our proprietary sublingual film formulation of Dex and set to expire no earlier than 2039, are now listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the "Orange Book"). In the same family, we also have a granted patent in China, two granted patents in Eurasia, and pending applications in the U.S., China, and other major markets. We expect that patents issuing in this family will expire no earlier than 2039. We have also filed applications in additional patent families that are relevant to BXCL501. We have applications pending in the U.S., Europe and Japan directed to methods of treating insomnia using sublingual Dex. We expect that patents issued from these applications, if any, will expire no earlier than 2035. We also have applications filed in 16 regions/countries, including the U.S., Europe, Japan, and China, directed to methods of treating agitation. We expect that patents issued from these applications, if any, will expire no earlier than 2042. We also have one PCT application directed to treating mania and another to treating depression. 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 immuno-oncology program, including our core patent family directed to methods of using BXCL701 with immune checkpoint inhibitors, which is granted in the U.S., Japan, Australia, Canada, Russia, China, South Africa, Mexico, New Zealand, and United Arab Emirates, and with at least one pending application in the U.S., China, Mexico, the Republic of Korea, New Zealand, Russia, Australia, Brazil, Hong Kong, and Europe. Patents issued from this family, if any, are expected to expire no earlier than 2036. We have one additional patent issued in the U.S. directed to a method of selecting patients based on a biomarker, with an expected expiration date no earlier than 2039.

Additional applications are directed to administering BXCL701 in combinations with various other molecules, biomarkers, and dosing regimens. We also have four provisional applications directed to novel formulations of BXCL701, various dosing regimens, methods of use, and combination therapies. We expect that patents issued from these applications, if any, will expire from 2039 to 2044.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the U.S., the patent term is 20 years from the earliest filing date of a non-provisional patent application. Depending upon the timing, duration, and specifics of FDA approval of our product candidates, a U.S. patent that we own or license may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984 (a.k.a., the "Hatch-Waxman Act"). The act permits a patent restoration term of up to five years as compensation for patent term lost during product development and the drug approval regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND, and the submission date of a new drug application ("NDA"), plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension, and the application for extension must be made prior to patent expiration. The U.S. Patent and Trademark Office ("USPTO"), in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration date, depending on the expected length of clinical trials and other factors involved in the submission of the relevant NDA.

The term of a patent can also be extended by Patent Term Adjustment ("PTA") established in 35 USC 154(b). The intention of the PTA is to accommodate for delays caused by the USPTO during the prosecution of a US utility or plant patent application. Under PTA, the USPTO delay is divided into three types: type A (delays after 14 months from the filing date of the application until the USPTO issues a first Office Action and delays after four months from the filing

of certain actions by the applicant until the USPTO responds to such actions); type B (delays after three years from the earliest effective filing date until a patent is granted); and type C (delays due to interferences, secrecy orders, and successful appeals). The total amount of PTA is calculated by adding the types A, B, and C delays, and then subtracting any delay that is overlapped among three types or that is attributable to the applicant.

The term of a patent can also be shortened by a terminal disclaimer. A terminal disclaimer is a statement filed by a patent owner in which the owner disclaims or dedicates to the public the terminal part of the term of a patent. Often, the terminal disclaimer is filed in cases where at least one claim of a pending application would have been obvious in light of at least one claim in an earlier-filed patent, AKA non-statutory obviousness-type double patenting rejection.

The patent positions of companies such as ours are generally uncertain and involve complex legal and factual questions. No consistent policy regarding the scope of claims allowable in patents in the field of method of use patents or reformulation patents has emerged in the U.S. Patent laws and their interpretation outside of the U.S. are also uncertain. Changes in either the patent laws or their interpretation in the U.S. and other countries may diminish our ability to protect our technology or product candidates and enforce the patent rights that we license, and also could affect the value of such intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell, or importing products that infringe our intellectual property will depend in part on our success in obtaining and enforcing patent claims that cover our technology, inventions, and improvements. With respect to both licensed and company owned intellectual property, we cannot guarantee that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications we may file in the future, nor can we be sure that any patents that may be granted to us in the future will be commercially useful in protecting our products, the methods of use, or the manufacture of those products. Patent and other intellectual property rights in the pharmaceutical and biotechnology space are evolving and involve many risks and uncertainties. For example, third parties may have blocking patents that could be used to prevent us from commercializing our product candidates and practicing our proprietary technology, and the issued patents that we in-license and those that may issue in the future may be challenged, invalidated, or circumvented, which could limit our ability to stop competitors from marketing related products or could limit the term of patent protection that otherwise may exist for our product candidates. In addition, the scope of the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies outside the scope of the rights granted under any issued patents that we own or exclusively in license. For these reasons, we may face competition with respect to our product candidates. Moreover, because of the extensive time required for development, testing, and regulatory review of a potential product, it is possible that, before any particular product candidate can be commercialized, any patent protection for such product may expire or remain in force for only a short period following commercialization, thereby reducing the commercial advantage the patent provides.

Basis of Presentation

The Company’s condensed consolidated financial statements are prepared in accordance with U.S. Generally Accepted Accounting Principles.

Components of Our Results of Operations

Product Revenues, Net

Revenues relate to sales of IGALMI and reflect limited market access since commercial launch in July 2022. The revenues are net of rebates, chargebacks, discounts, and other adjustments. During the fourth quarter of 2022, we began contracting directly with intermediaries such as GPOs.

Operating Costs and Expenses

Cost of Goods Sold

Cost of goods sold primarily relate to the costs of producing, packaging and delivering our product to customers.

Research and Development

Our research and development expenses reflect costs associated with the identification of our preclinical and clinical product candidates. Expenditures 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 and sites that conduct our non-clinical studies and clinical trials, costs of outside consultants engaged in research and development activities, travel expenses, the cost of acquiring, developing and manufacturing preclinical and clinical trial materials and lab supplies, and depreciation and other expenses. Payments to BioXcel LLC are also included in research and development expenses. Costs associated with third parties that provide non-clinical services such as toxicology, pharmacology, research and discovery, biomarker studies and similar services are included in the professional fees category of research and development expenses.

We expense research and development costs as incurred.

Our research and development costs by program for the three and six months ended June 30, 2023 and 2022 were as follows:

	Three Months Ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Direct external costs				
BXCL501	$ 15,010	$ 9,054	$ 31,478	$ 17,570
BXCL701	2,095	1,785	4,763	4,701
Other research and development programs	1,390	671	2,281	1,223
Total direct external costs	$ 18,495	$ 11,510	$ 38,522	$ 23,494
Internal personnel costs	7,289	5,564	14,043	11,416
Sub-total direct costs	$ 25,784	$ 17,074	$ 52,565	$ 34,910
Indirect costs and overhead	1,189	832	2,208	1,683
Total research and development expenses	$ 26,973	$ 17,906	$ 54,773	$ 36,593

Selling, General and Administrative

Selling, general and administrative expenses primarily consist of salaries, benefits and non-cash stock-based compensation for our sales, executive and administrative personnel. Selling, general and administrative expenses also include legal expenses to pursue patent protection of our intellectual property, professional fees for audit and tax services and insurance charges. We may also incur costs to comply with corporate governance, internal controls, investor relations and disclosures and similar requirements applicable to public companies.

With the announcement of our Strategic realignment, we expect that our selling, general and administrative expenses will decline due to the restructured commercialization of IGALMI and reduced labor costs. However, we may also experience increased fees for outside consultants, attorneys, and accountants.

Other Expense (Income)

Other expense (income) primarily consists of interest costs associated with the financing agreements the Company put in place in April 2022, changes in fair value of derivative financial instruments, and interest income earned on cash and cash equivalents that were comprised primarily of money market funds. We expect that interest expense will increase in the future, as we meet additional milestones, in connection with payments to cure the revenue covenant and as we draw down additional funds under the financing agreements.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements is set forth in Note 3 to the condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Results of Operations

Comparison of the Three Months Ended June 30, 2023 and 2022

Product Revenues, Net

Product revenues, net in the three months ended June 30, 2023 were $457, comprised of sales of IGALMI, subsequent to commercial launch in July 2022. Sales to date reflect limited market access. There were no revenues in the second quarter of 2022.

Cost of Goods Sold

Cost of goods sold for the three months ended June 30, 2023, were $26, which primarily related to the costs to produce, package and deliver IGALMI to customers. There were no cost of goods sold in the second quarter of 2022.

Research and Development Expense

Research and development expenses for the three months ended June 30, 2023 and 2022 were as follows:

	Three Months Ended June 30,			
	2023	2022	Change	% Change
Personnel and related costs	$ 5,390	$ 4,329	$ 1,061	25 %
Non-cash stock-based compensation	1,898	1,235	663	54 %
Professional fees	5,631	3,415	2,216	65 %
Clinical trials expense	10,701	5,927	4,774	81 %
Chemical, manufacturing and controls cost	2,369	2,264	105	5 %
Travel and other costs	984	736	248	34 %
Total research and development expenses	$ 26,973	$ 17,906	$ 9,067	51 %

The overall increase of $9,067 for the three months ended June 30, 2023 relative to the same period in 2022 was primarily attributable to:

- An increase in clinical trials expense associated with the SERENITY III study to evaluate BXCL501 for at-home use for the acute treatment of agitation related to schizophrenia and bipolar disorders, as well as the TRANQUILITY II study of BXCL501 for the potential treatment of agitation in patients with Alzheimer’s disease.
- Increased professional fees due to required toxicology testing for IGALMI as well as increased pharmacology and professional research fees.
- An increase in personnel costs related to our efforts to grow our clinical team as we expanded our clinical trials, particularly for evaluating BXCL501 for treatment of agitation in patients with Alzheimer’s disease and at-home use for agitation associated with bipolar disorders and schizophrenia.
- Increased stock-based compensation costs due to increased equity award grants and higher grant date fair values resulting from a higher price for the Company’s common stock.

Following IGALMI’s approval by the FDA, we capitalize costs related to commercial production of IGALMI as inventory and expense those chemical, manufacturing and controls (“CMC”) costs related to clinical trials.

Selling, General and Administrative Expense

Selling, general and administrative expenses for the three months ended June 30, 2023 and 2022 were as follows:

	Three Months Ended June 30,			
	2023	2022	Change	% Change
Personnel and related costs	$ 8,569	$ 4,529	$ 4,040	89 %
Non-cash stock-based compensation	4,226	3,247	979	30 %
Professional fees	5,771	3,690	2,081	56 %
Commercial and marketing	4,808	4,719	89	2 %
Insurance	396	603	(207)	(34)%
Travel and other costs	2,102	1,594	508	32 %
Total selling, general and administrative expenses	$ 25,872	$ 18,382	$ 7,490	41 %

The overall increase of $7,490 for the three months ended June 30, 2023, relative to the same period in 2022 was primarily attributable to:

- An increase in personnel and related costs due to our efforts to expand our functional teams, particularly in sales, to support commercialization of IGALMI in the U.S.
- Increased professional fees, primarily relating to our investigation related to our TRANQUILITY II study, the commercial launch of IGALMI in the U.S., formation of OnkosXcel, and higher corporate operating support levels. The increase in legal fees was offset primarily by reductions in consulting and recruiting fees.
- Increased stock-based compensation costs due to increased equity award grants resulting from increased personnel and higher grant date fair values resulting from a higher price for the Company's common stock.
- An increase in travel and other costs following the commercial launch of IGALMI. In addition, we experienced higher technology costs related to the addition of personnel and expansion of our operations.

Other Expense (Income)

Interest expense increased to $3,259 for the second quarter of 2023 from $1,586 in the same period in 2022 primarily due to increased average outstanding borrowings under the OFA Facilities (as defined below) that the Company put in place in April 2022. The expense was partially offset by interest income earned on cash and cash equivalents that were held primarily in short-term money market funds. Other income, net is primarily associated with changes in the fair value of derivative financial instruments for the period, which relate to instruments associated with the OFA Facilities.

Comparison of the Six Months Ended June 30, 2023 and 2022

Product Revenues, Net

Product revenues, net in the six months ended June 30, 2023 were $663, comprised of sales of IGALMI. Sales to date reflect limited market access. There were no revenues in the first half of 2022.

Cost of Goods Sold

Cost of goods sold for the six months ended June 30, 2023, were $34, which primarily related to the costs to produce, package and deliver IGALMI to customers. There were no cost of goods sold in the first half of 2022.

Research and Development Expense

Research and development expenses for the six months ended June 30, 2023 and 2022 were as follows:

	Six months ended June 30,			
	2023	2022	Change	% Change
Personnel and related costs	$ 10,848	$ 9,205	$ 1,643	18 %
Non-cash stock-based compensation	3,195	2,210	985	45 %
Professional fees	9,265	7,339	1,926	26 %
Clinical trials expense	23,720	12,242	11,478	94 %
Chemical, manufacturing and controls cost	5,840	4,204	1,636	39 %
Travel and other costs	1,905	1,393	512	37 %
Total research and development expenses	$ 54,773	$ 36,593	$ 18,180	50 %

The overall increase of $18,180 for the six months ended June 30, 2023 relative to the same period in 2022 was primarily attributable to:

- An increase in clinical trials expense due to the SERENITY III study evaluating BXCL501 for at-home use for the acute treatment of agitation related to schizophrenia and bipolar disorders, as well as the TRANQUILITY II study of BXCL501 for the potential treatment of agitation in patients with Alzheimer's disease.
- Increased professional fees due to increased pharmacology and research and discovery costs.
- An increase in personnel and related costs related to our efforts to grow our clinical team as we expanded our clinical trials, particularly evaluating BXCL501 for treatment of agitation in patients with Alzheimer's disease and at-home use for agitation associated with bipolar disorders and schizophrenia.
- Increased CMC costs associated with producing materials for our clinical trials of BXCL501 for at-home use for the acute treatment of agitation related to schizophrenia and bipolar disorders and for the treatment of agitation associated with Alzheimer's disease, and BXCL701 for the treatment of prostate and lung cancers.
- Increased stock-based compensation costs due to increased equity award grants and higher grant date fair values resulting from a higher price for the Company's common stock.

Following IGALMI's approval by the FDA, we capitalize costs related to commercial production of IGALMI as inventory and expense those CMC costs related to clinical trials.

Selling, General and Administrative Expense

Selling, general and administrative expenses for the six months ended June 30, 2023 and 2022 were as follows:

	Six months ended June 30,			
	2023	2022	Change	% Change
Personnel and related costs	$ 18,020	$ 7,735	$ 10,285	133 %
Non-cash stock-based compensation	7,807	6,097	1,710	28 %
Professional fees	8,812	7,821	991	13 %
Commercial and marketing	9,178	6,202	2,976	48 %
Insurance	951	1,173	(222)	(19)%
Travel and other costs	4,699	2,147	2,552	119 %
Total selling, general and administrative expenses	$ 49,467	$ 31,175	$ 18,292	59 %

The overall increase of $18,292 for the six months ended June 30, 2023, relative to the same period in 2022 was primarily attributable to:

- An increase in personnel and related costs due to our efforts to expand our functional teams, particularly in sales, to support commercialization of IGALMI in the U.S.
- Higher commercial and marketing expense associated with media, marketing and data and business analytics costs associated with commercialization of IGALMI.
- An increase in travel and other costs following the commercial launch of IGALMI. In addition, we experienced higher technology costs related to the addition of personnel and expansion of our operations.
- Increased stock-based compensation costs due to increased equity award grants resulting from increased personnel and higher grant date fair values resulting from a higher price for the Company's common stock.
- Increased professional fees, mainly for corporate legal fees, primarily relating to our investigation related to our TRANQUILITY II study, the commercial launch of IGALMI in the U.S., formation of OnkosXcel, and higher corporate operating support levels. The increase in legal fees was offset primarily by reductions in consulting and recruiting fees.

Other Expense (Income)

Interest expense increased to $6,627 for the first half of 2023 from $1,593 in the same period in 2022 primarily due to increased average outstanding borrowings under the OFA Facilities that the Company put in place in April 2022. The expense was partially offset by interest income earned on cash and cash equivalents that were held primarily in short-term money market funds. Other income, net is primarily associated with changes in the fair value of derivative financial instruments for the period, which relate to instruments associated with the OFA Facilities.

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. For a discussion of inflationary risks to our future revenues under the Inflation Reduction Act, see "*Healthcare reform measures could hinder or prevent our product candidates' commercial success.*" in Part II, Item 1A, "Risk Factors" elsewhere in this Quarterly Report on Form 10-Q.

Liquidity and Capital Resources

As of June 30, 2023, we had cash and cash equivalents of $127,545, working capital of $103,491 and stockholders' equity of $5,864. Net cash used in operating activities was $90,559 and $65,516 for the six months ended June 20, 2023 and 2022, respectively. We incurred losses of $53,515 and $37,670 for the three months ended June 30, 2023 and 2022, respectively and $106,311 and $69,142 for the six months ended June 30, 2023 and 2022, respectively. We have generated limited revenues to date, and we have not yet achieved profitability. We expect that our research and development and selling, general and administrative expenses will continue to increase and, as a result, we will need to generate significant product revenues to achieve profitability. Our history of significant losses, negative cash flows from operations, potential near-term, increased covenant-driven amortization payments under our OFA Facilities (as defined and described under Sources of Liquidity Below), the regulatory event of default triggers under the OFA Facilities, limited liquidity resources currently on hand, and dependence on our ability to obtain additional financing to fund our operations after the current resources are exhausted, about which there can be no certainty, have resulted in management's assessment that there is substantial doubt about our ability to continue as a going concern for a period of at least 12 months from the issuance date of the financial statements included in this Quarterly Report on Form 10-Q.

This going concern analysis takes into consideration the potential mitigating effect of management's Reprioritization plans that have not been fully implemented as of the date the financial statements are issued. However, this analysis does not consider a possible restructuring of the OFA Facilities, which is currently under discussion.

Based on our cash, cash equivalents and marketable securities of $128 million as of June 30, 2023, management believes we will be able to fund our operating expenses and capital expenditures into mid-2024.

Successful completion of the Company's development programs and, ultimately, the attainment of profitable operations are dependent upon future events, including obtaining adequate financing to support the Company's cost structure and operating plan. Management's plans to improve the Company's liquidity and reduce its operating expenses and capital requirements include, among other things, pursuing one or more of the following steps to raise additional capital, none of which can be guaranteed or are entirely within the Company's control:

- raise funding through the sale of the Company's equity securities;
- raise funding through third-party investments in or other strategic options for OnkosXcel;
- raise funding through debt financing and/or restructuring of its existing OFA Facilities;
- establish collaborations with potential partners to advance the Company's product pipeline;
- establish collaborations with potential marketing partners;
- reduce overhead and headcount to focus on core priorities, and/or
- any combination 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 when there is market uncertainty or an economic downturn. If we are unable to secure adequate additional funding as and when needed on acceptable or commercially reasonable terms, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more product candidates. In addition, there are various macro-economic trends affecting the financing markets whose impact on our liquidity and future funding requirements are uncertain as of the filing date of this Quarterly Report on Form 10-Q. 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. See "Risk Factors – Risks Related to Financial Position and Need for Additional Capital - 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" in Part II. Item 1A. of this Quarterly Report on Form 10-Q.

Sources of Liquidity

We have focused our efforts on raising capital and building the products in our pipeline, and only recently on sales for our first FDA approved product IGALMI. Since our inception, our operations have been financed primarily from proceeds from the sale of equity securities, including our initial public offering, private placements of our common stock, registered offerings of our common stock, an Open Market Sale Agreement (the "Sale Agreement") with Jefferies LLC ("Jefferies"), and borrowings under strategic financing arrangements (as described below). 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 April 2022, we entered into two financing agreements; a Credit Agreement and Guaranty (the "Credit Agreement") by and among the Company, as the borrower, certain subsidiaries of the Company from time to time party thereto as subsidiary guarantors, the lenders party thereto (the "Lenders"), and Oaktree Fund Administration LLC ("OFA") as administrative agent, and a Revenue Interest Financing Agreement (the "RIFA"; and together with the Credit Agreement, the "OFA Facilities") by and among the Company, the purchasers party thereto (the "Purchasers") and OFA as administrative agent. Pursuant to the Credit Agreement, the Lenders agreed to loan us up to $135,000 in senior secured term loans. On April 28, 2022, we borrowed the first tranche of $70,000 of loans. The remaining two tranches of the commitments under the Credit Agreement may be borrowed at our option prior to December 31, 2024 as follows:

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

- $30,000 upon satisfaction of certain conditions, including specified minimum net sales of the Company attributable to sales of BXCL501 for a trailing 12 consecutive month period.

The foregoing additional amounts were not eligible to be borrowed as of June 30, 2023.

Pursuant to the RIFA, the Purchasers agreed to provide us with up to $120,000 in financing for our near-term commercial activities of IGALMI, development and commercialization of BXCL501 and other general corporate purposes. On July 8, 2022, we drew down the first tranche of $30,000 under the RIFA. The remaining commitments under the RIFA may be drawn at our option prior to December 31, 2024, as follows:

- $45,000 upon satisfaction of certain conditions, including receipt of certain regulatory and patent related milestones and specified minimum net sales of BXCL501 during any consecutive 12-month period; and

- $45,000 upon satisfaction of certain conditions, including receipt of certain regulatory and patent related milestones and specified minimum net sales of BXCL501 during any consecutive 12-month period.

The foregoing additional amounts were not eligible to be borrowed as of June 30, 2023.

We do not anticipate that we will be able to satisfy the conditions necessary to draw these commitments.

In connection with the Credit Agreement, we granted to the Lenders certain warrants to purchase up to 278 shares of our common stock, rights to purchase up to $5,000 of our common stock and warrants to purchase up to 175 individual ownership units (i.e., not in thousands) in OnkosXcel.

See Note 8, Debt and Credit Facilities in the Notes to Condensed Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for additional information relating to the Credit Agreement and RIFA, including applicable interest rates, anticipated payment obligations and certain restrictive and financial covenants thereunder.

If, as of a Revenue Covenant Measurement Testing Date (as defined in Note 8, Debt and Credit Facilities), the Company's revenue for the applicable Revenue Covenant Measurement Period (as defined in Note 8, Debt and Credit Facilities) is less than the minimum revenue amount specified for the applicable period then required under the Revenue Covenant, (as defined in Note 8, Debt and Credit Facilities) the Company would have a right to cure such shortfall for a total of three fiscal periods by making a revenue cure payment (which would be treated as prepayments of the loans subject to a prepayment fee) to the Lenders in an amount equal to the difference between such minimum required revenue amount and the Company's actual revenues for such Revenue Covenant Measurement Period, such payment to not be less than $1,000. If paid, the Company will be deemed to have complied with the Revenue Covenant as of such Revenue Covenant Measurement Testing Date. Any such payment will be applied to the prepayment of the loans under the Credit Agreement. For the Revenue Covenant Measurement Testing Dates ending December 31, 2023, March 31, 2024 and June 30, 2024, shortfalls under the Revenue Covenant, as described above, could result in a revenue cure payment of up to $7,657, $10,636, and $14,313, respectively, plus aggregate prepayment fees of up to $1,500.

Based on current revenue projections, the Company expects that it will be required to make cure payments for failure to comply with the Revenue Covenant in 2024. Such payments could be up to an aggregate of approximately $34,106 in the next twelve months from the date of this Quarterly Report on Form 10-Q. Subsequent to the three cure periods, the Company would default on the Credit Agreement and Guaranty if it is unable to satisfy the Revenue Covenant. Given the anticipated and/or potential payment obligations that may become due within the next 12 months under the Credit Agreement, we are in active discussions with lenders to potentially restructure our financing arrangements and/or waive the Revenue Covenant. As of June 30, 2023, we were in compliance with all covenants under the Credit Agreement and the RIFA.

In May 2021, we entered into the Sale Agreement with Jefferies pursuant to which we can offer and sell shares of our common stock, having an aggregate offering price of up to $100,000, from time to time, through an "at the market offering" program under which Jefferies will act as sale agent. The Company sold 756 shares under the Sale Agreement with Jefferies in the first half of 2023 for net proceeds of $23,918, net of issuance costs of $739.

Cash Flows

	Six months ended June 30,	
	2023	2022
Cash (used in) provided by:		
Operating activities	$ (90,559)	$ (65,516)
Investing activities	$ (20)	$ (139)
Financing activities	$ 24,399	$ 66,139

Operating Activities

Net cash used in operating activities for the six months ended June 30, 2023 was $90,559 and was primarily attributable to our net loss of $106,311, which is due to our being in the early stages of commercial launch of IGALMI and our continued product development efforts, in part offset by $11,001 of non-cash stock-based compensation, a $1,843 increase in accounts payable, accrued expenses due to related parties, and other current liabilities, and a $2,234 increase in accrued interest.

Net cash used in operating activities for the six months ended June 30, 2022 was $65,516, which was primarily attributable to our net loss of $69,142, a $1,395 increase in inventory, and a $7,072 increase in prepaid expenses, other current assets and other assets, partially offset by $8,307 in non-cash stock-based compensation, as well as a $3,315 increase in accounts payable, accrued expenses due to related parties and other current liabilities.

Investing Activities

Net cash used in investing activities for the six months ended June 30, 2023 and 2022 was $20 and $139, respectively, and was primarily attributable to purchases of equipment and leasehold improvements.

Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2023, was $24,399 and was primarily attributable to net proceeds received from the sale of common stock under the Sale Agreement with Jefferies of $23,918 and the exercise of stock options.

Net cash provided by financing activities for the six months ended June 30, 2022 was $66,139 and was primarily attributable to proceeds received from the OFA Facilities.

Operating Capital and Capital Expenditure Requirements

We expect to continue to incur significant and increasing operating losses at least for the next several years as we commercialize IGALMI and as we expand our clinical trials of and seek marketing approval for BXCL501, BXCL502, BXCL701 and BXCL702, while pursuing development of additional product candidates. We expect to continue to incur net losses in the near term. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our planned clinical trials and our expenditures on other research and development activities.

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

- continue our clinical development of our product candidates;
- conduct additional research and development with our product candidates;

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

In addition, see Note 8, *Debt and Credit Facilities* in the Notes to Condensed Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for additional information relating to anticipated and potential payments in the next 12 months under the Credit Agreement and RIFA.

We believe that our existing cash and cash equivalents as of June 30, 2023, will not 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 condensed consolidated financial statements included in this Quarterly Report on Form 10-Q, including funding our ongoing research and development and commercialization efforts. We expect that we will need to obtain substantial additional funding to fund our ongoing operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted, and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends, which could adversely impact our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of our product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to our product candidates that we otherwise would seek to develop or commercialize ourselves.

Contractual Obligations and Commitments

In April 2022, the Company signed a commercial supply agreement that requires minimum annual payments for the first three years of the agreement that in aggregate total $10,000 for the three-year period and the minimum commitment for 2023 is $3,000.

In February 2022, we signed a distribution agreement with a third-party to distribute product related to BXCL501 in the U.S. The distributor will be paid defined fees for its services under the agreement, which can be terminated by either party for cause. The distribution agreement can also be terminated by us without cause, subject to payment of agreed upon termination fees.

BTI leases office space for its corporate headquarters at 555 Long Wharf Drive, New Haven, Connecticut (the “HQ Lease”). The HQ Lease expires in February 2026. The Company has an option to renew the HQ Lease for one additional five-year term. Payments under the HQ Lease are fixed. The Company has approximately $1,024 of payments remaining under the HQ Lease. For additional details, see Note 12, *Leases* in the Notes to Condensed Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for additional information relating to the Company’s leases.

In addition, we are obligated to make quarterly interest and royalty payments under our Credit Agreement and RIFA, respectively, as well as potential cure payments under the Credit Agreement. For additional details, see Note 8 *Debt and Credit Facilities* in the Notes to Condensed Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for additional information relating to the Company's debt payment obligations.

Critical Accounting Policies and Estimates

Our critical accounting policies and estimates are described in "Management's Discussion and Analysis of Financial Condition and Results of Operations-Critical Accounting Policies and Estimates" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022. We have reviewed and determined that those critical accounting policies and estimates remain our critical accounting policies and estimates as of and for the six months ended June 30, 2023. No material changes were made to our existing critical accounting policies and estimates during the period presented. Refer to Note 3, *Summary of Significant Accounting Policies* in the Notes to Condensed Consolidated Financial Statements elsewhere in this Form 10-Q.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Foreign Exchange Risk

As of June 30, 2023, we had $127,545 of cash and cash equivalents. Our cash and cash equivalents are primarily held in money market funds that hold U.S. government cash equivalent instruments. We do not participate in any foreign currency hedging activities and have limited exposure to other derivative financial instruments, primarily resulting from the terms and conditions of the OFA Facilities. We did not recognize any significant exchange rate losses during the six months ended June 30, 2023 and 2022, 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 do not contain material market 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 exceed federally insured limits. In the event of a failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we will be able to access uninsured funds in a timely manner or at all.

Interest Rate Risk

The loans under the Credit Agreement bear interest at a fixed annual rate of 10.25%, payable quarterly, and the RIFA is repaid based on a multiple of invested capital. Consequently, we do not have material floating interest rate exposure due to our indebtedness.

Capital Market Risk

We currently do not have substantial product revenues and depend on funds raised through other sources. One source of funding includes future debt or equity offerings. Our ability to raise funds in this manner depends upon, among other things, capital market forces affecting our stock price, and on the state of the capital markets generally.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2023. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the rules and forms of the 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 June 30, 2023, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the three months ended June 30, 2023 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, which could have a material adverse effect on our business, operating results, cash flows or financial condition. Please refer to Note 15, *Commitments and Contingencies* of our unaudited condensed consolidated financial statements in Part I, Item 1 of this Quarterly Report on Form 10-Q for information regarding material legal proceedings.

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, 2022. 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 not generated substantial product revenues to date, which may make it difficult to evaluate the success of our business 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, advancing the development of our product candidates, including conducting clinical and preclinical studies and establishing our commercial organization. We have only one product approved for commercial sale, and have limited experience in obtaining marketing approvals, manufacturing products on a commercial scale, and conducting sales and marketing activities necessary for successful commercialization. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully commercializing products.

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

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

Since our inception, we have incurred significant operating losses. Our net loss was $106.3 million and $69.1 million for the six months ended June 30, 2023 and 2022, respectively. As of June 30, 2023, we had stockholders' equity of approximately $5.9 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We have only one product candidate approved for marketing in the U.S., none in any other jurisdiction, and may never receive approval beyond the one product approved to date. It could be several years, if ever, before we have a commercialized product that generates significant revenues through sales of IGALMI or our product candidates, if approved. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue the development of our product candidates;
- conduct preclinical studies and clinical trials for our current product candidates and any future product candidates that we may pursue;
- continue to build our portfolio of product candidates through the acquisition or in-license of additional product candidates or technologies;

- continue to develop, maintain, expand and protect our intellectual property portfolio;
- pursue regulatory approvals for our current and future product candidates that successfully complete clinical trials;
- fully develop a sales, marketing, and distribution infrastructure to commercialize IGALMI and any other product candidates for which we may obtain marketing approval;
- hire additional clinical, commercial, regulatory, scientific and finance personnel; and
- incur additional legal, accounting and other expenses in operating as a public company.

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

Although we have obtained U.S. Food and Drug Administration ("FDA") approval for IGALMI, because of the numerous risks and uncertainties associated with product development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will obtain marketing approval to commercialize any additional product candidates. If we are required by the FDA, or other regulatory authorities such as the European Medicines Agency ("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. For example, recent developments with respect to our TRANQUILITY II trial evaluating BXCL501 in patients with probable Alzheimer's disease may increase the likelihood that we experience such costs or delays, as discussed in the risk factor below entitled: "*Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease*."

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

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

Our strategic reprioritization and related reduction in force may not achieve our intended outcome.

In August 2023, we announced a broad-based strategic reprioritization (the "Reprioritization"). We have determined to take actions to reduce certain operational and workforce expenses that are no longer deemed core to our ongoing operations in order to extend our cash runway and drive innovation and growth in high potential clinical development and value creating opportunities. These actions will include a shift in commercial strategy for IGALMI™ in the institutional setting, a reduction of in-hospital commercialization expenses, a suspension of programs no longer determined to be core to ongoing operations, and a prioritization on at-home treatment setting opportunities for BXCL501. As part of this strategy, our Board of Directors approved a reduction of approximately 50% of the Company's current workforce.

The reduction in force may result in unintended consequences and costs, such as the loss of institutional knowledge and expertise, attrition beyond the intended number of employees, decreased morale among our remaining employees, and the risk that we may not achieve the anticipated benefits of the reduction in force. In addition, while positions have been eliminated, certain functions necessary to our operations remain, and we may be unsuccessful in distributing the duties and obligations of departed employees among our remaining employees. The reduction in workforce could also

make it difficult for us to pursue, or prevent us from pursuing, new opportunities and initiatives due to insufficient personnel, or require us to incur additional and unanticipated costs to hire new personnel to pursue such opportunities or initiatives. The workforce reduction could also harm our reputation, making our ability to recruit skilled personnel difficult. If we are unable to realize the anticipated benefits from the reduction in force, or if we experience significant adverse consequences from the reduction in force, our business, financial condition, and results of operations may be materially adversely affected.

In addition, we may not realize the benefits of or there may be unanticipated costs associated with our Reprioritization. As a result of the Reprioritization, including our strategic refocus, we may not generate material revenues from IGALMI in the near term because our commercial force will be significantly reduced. If we are unable to commercialize IGALMI in a different setting or be able to develop, receive marketing approval for and successfully commercialize BXCL501, BXCL701 and any of our other product candidates on our own or with any future collaborator, or experience delays because of any of these factors or otherwise, our business could be materially and substantially harmed.

In addition, because we have limited financial and managerial resources, under our Reprioritization, we intend to focus on specific product candidates, indications and development programs. We may also conduct several clinical trials for our product candidates in parallel over the next several years, which may make our decision as to which product candidates to focus on more difficult. As a result, we may forgo or delay pursuit of opportunities with other product candidates or other indications that could have had greater commercial potential or likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through future collaborations, licenses and other similar arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. If we are not successful in increasing our efficiency as a result of this Reprioritization, our efforts to develop and commercialize our product candidates may be delayed or halted and our business could be materially adversely impacted.

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 will require additional future funding to support current and anticipated future expenses. We currently anticipate continuing to develop and conduct clinical trials with respect to our current and any future product candidates; seek to identify and develop additional product candidates; acquire or in-license other product candidates or technologies; seek regulatory approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure to support the commercialization of products for which we may obtain marketing approval; require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization; maintain, expand and protect our intellectual property portfolio; hire and retain limited 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 may be required to expend significant funds to continue to commercialize IGALMI in the U.S. and advance the development of BXCL501, BXCL701, BXCL502 and our other product candidates. In addition, while we may seek one or more collaborators for future development of our current product candidates or any future product candidates that we may develop for one or more indications, we may not be able to enter into a collaboration for any of our product candidates for such indications on suitable terms, on a timely basis or at all. In any event, 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. We may also seek third-party investments in or other strategic options for our subsidiary, OnkosXcel. Further financing may not be available to us on acceptable terms, or at all. In addition, we are reliant on the financial institutions with which we hold our cash and cash equivalents. If such institutions were to close, we may not be able to recover all of our cash or cash

equivalents held at such institutions. Moreover, market volatility resulting from COVID-19, credit crises, adverse macroeconomic conditions, such as high interest or inflation rates, 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.

Management believes that, after giving effect to the Reprioritization, the Company's cash, cash equivalents and marketable securities of $127 million as of June 30, 2023 will allow the Company to fund its operations and meet its liquidity requirements into mid-2024. However, the expected cost benefit of the Reprioritization cannot be assured, and there can be no assurance that we will be able to extend our cash runway by restructuring or amending the terms of our OFA Facilities or otherwise. Furthermore, our estimate as to how long we expect our existing cash to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

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

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

As of June 30, 2023, we had aggregate principal indebtedness of $101.6 million outstanding under two financing agreements: a Credit Agreement and Guaranty (the "Credit Agreement") by and among the Company, as the borrower, certain subsidiaries of the Company from time to time party thereto as subsidiary guarantors, the lenders party thereto

(the “Lenders”), and Oaktree Fund Administration LLC (“OFA”) as administrative agent, and a Revenue Interest Financing Agreement (the “RIFA”; and together with the Credit Agreement, the “OFA Facilities ”) by and among the Company, the purchasers party thereto (the “Purchasers”) and OFA as administrative agent. Approximately $71.6 million of the indebtedness relates to the Credit Agreement, pursuant to which the Lenders have agreed to loan us up to an additional $65.0 million in senior secured term loans under satisfaction of certain conditions, and $30.0 million relates to the RIFA, pursuant to which the Purchasers agreed to fund an additional $90.0 million upon satisfaction of certain conditions. The RIFA requires us to make tiered revenue interest payments on U.S. net sales of IGALMI and any other future BXCL501 products equal to a royalty ranging from 0.375% to 7.750% of net sales of IGALMI and any other future BXCL501 products in the U.S., as well as certain additional payments to the Purchasers from time to time, to ensure that the aggregate amount of payments received by the Purchasers under the RIFA are at least equal to certain agreed upon minimum levels as of certain specified dates, subject to terms and conditions set forth in the RIFA.

Based on current revenue projections, we expect that we will be required to make cure payments for shortfalls under the Revenue Covenant (as defined in Note 8, Debt and Credit Facilities) in 2024. Such payments could be up to an aggregate of approximately $34,106, which includes up to $1,500 in aggregate prepayment fees, in the next twelve months from the date of this Quarterly Report on Form 10-Q. Subsequent to the three cure periods, the Company would default on the Credit Agreement and Guaranty if it is unable to satisfy the Revenue Covenant. Given the anticipated and/or potential payment obligations that may become due within the next 12 months under the Credit Agreement, we are in active discussions with lenders to potentially restructure our financing arrangements and/or waive the Revenue Covenant.

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

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

Restrictive covenants in the Credit Agreement and RIFA each place limits on our ability to conduct our business. The Credit Agreement contains customary representations and warranties and customary affirmative and negative covenants, including, among other things, restrictions on indebtedness, liens, investments, mergers, dispositions, prepayment of other indebtedness, and dividends and other distributions, subject to certain exceptions, including specific exceptions with respect to product commercialization and development activities. We must also comply with certain covenants under the Credit Agreement, including a financial covenant that requires we maintain a minimum cash liquidity amount of $15 million (or higher upon certain events) and minimum revenue thresholds beginning in with the fourth quarter of 2023. In addition, certain events, including receipt of a warning letter from the FDA, may constitute an event of default. The RIFA contains customary representations and warranties and certain restrictions on our ability to incur indebtedness and grant liens on intellectual property related to BXCL501. In addition, the RIFA provides that if certain events occur, including certain bankruptcy events, failure to make payments, a change of control, an out-license or sale of all of the rights in and to BXCL501 in the U.S., in each case except a permitted licensing transaction (as defined in the RIFA) and, subject to applicable cure periods, material breach of the covenants in the RIFA, OFA, at the direction of the Purchasers, may require us to repurchase certain of the Purchasers’ interests. Under the RIFA, we are required to use commercially reasonable efforts to develop and commercialize BXCL501 in the United States. To the extent as a result of the Reprioritization or otherwise, we fail to use such commercially

reasonable efforts, a breach of the RIFA could occur, which could also trigger an event of default under the Credit Agreement.

The Credit Agreement also contains certain regulatory-related events of default, which do not have cure periods, and could be triggered in connection with potential developments relating to the investigation and audit in connection with our TRANQUILITY II Phase 3 Trial. See risk factor below entitled "Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease."

If we cannot meet our debt service obligations, the holders of our indebtedness may accelerate such indebtedness and, to the extent such indebtedness is secured, foreclose on our assets. In such an event, we may not have sufficient assets to repay all of our indebtedness.

In addition, incurring indebtedness generally requires that a portion of cash flow from operating activities be dedicated to interest and principal payments. Debt service requirements could reduce our ability to use our cash flow to fund operations and capital expenditures, to capitalize on future business opportunities, including additional acquisitions, or to pay dividends or increase dividends. In addition, our indebtedness may reduce our flexibility to operate our business, adjust to changing business conditions, restrict us from making strategic acquisitions or cause us to make non-strategic divestitures or obtain additional financing. Any of these risks could materially adversely affect our business, results of operations or financial condition.

We have identified conditions and events that raise substantial doubt regarding our ability to continue as a going concern.

As of June 30, 2023, we had $127.5 million in cash and cash equivalents. Based on our existing cash, availability funding facilities, we do not believe we have sufficient cash on hand to support current operations and service our debt obligations for at least one year from the date of issuance of the unaudited condensed consolidated financial statements appearing in this Quarterly Report on Form 10-Q. In order to mitigate the current and potential future liquidity issues, we are undertaking a Reprioritization and may, among other things, seek to raise capital through the issuance of common stock, restructure, refinance, and/or amend the terms of the Credit Agreement and RIFA (including with respect to regulatory related events of default that do not contain a cure period) or pursue other strategic alternatives. However, such transactions may not be successful and we may not be able to raise additional equity, restructure, refinance or amend the terms of the Credit Agreement and RIFA on acceptable terms, or at all. As such, there can be no assurance that we will be able to continue as a going concern.

Risks Related to the Discovery and Development of Product Candidates

We have limited experience in drug discovery and drug development.

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

Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease.

Following principal investigator misconduct at one of the clinical sites in our TRANQUILITY II Phase 3 clinical trial, we are investigating issues associated with the trial. This principal investigator had previously been subject to a

December 2022 FDA inspection of her clinical site in connection with the TRANQUILITY II clinical trial. At the conclusion of this inspection, the FDA issued an FDA Form-483 identifying three inspectional observations. These observations related to the principal investigator's failure to adhere to the informed consent form approved by the Institutional Review Board for a limited number of subjects whose records the FDA reviewed, maintain adequate case histories for certain patients whose records the FDA reviewed, and adhere to the investigational plan in certain instances. For example, the FDA cited the principal investigator's delay in informing the sponsor's medical monitor or pharmacovigilance safety vendor of a serious adverse event ('SAE"), for one of the subjects, which report was made to our vendor outside of the 24 hour time period prescribed by the clinical trial protocol. The principal investigator for this clinical site responded to the FDA observations within the time period requested. The FDA inspection remains open, however, as the FDA has not issued an Establishment Inspection Report.

In May 2023, it came to our attention that this same principal investigator in the TRANQUILITY II clinical trial may have fabricated email correspondence around the time of the FDA inspection, purporting to demonstrate that the investigator timely submitted to our pharmacovigilance safety vendor a report of an SAE from a different subject than the one cited in the FDA Form-483, and purporting to show that the vendor had confirmed receipt. Upon receipt of this information, we promptly initiated an investigation and received confirmation that the principal investigator fabricated the email correspondence related to the timing of the reporting of this SAE to our pharmacovigilance vendor to make it appear as though this SAE had been timely reported as required by the clinical trial protocol. We also confirmed that this SAE had been timely entered into the electronic data capture system, even though the SAE had not been separately reported to our pharmacovigilance safety vendor within the 24 hour timeframe required under the protocol.

In connection with this ongoing investigation, we were made aware that the fabricated email correspondence was provided to the FDA by the principal investigator's employer during the on-site inspection in December 2022. After unblinding of the data, we determined that the SAE that was the subject of this fabricated correspondence between the principal investigator and our pharmacovigilance vendor occurred in a subject in the placebo arm. This principal investigator has not participated in any other clinical trial sponsored or conducted by us. Moreover, the study was designed such that trained study staff other than principal investigators were to conduct assessments of the primary efficacy measure. Both we and the principal investigator's employer have reported this incident to the FDA.

As noted above, we are currently in the process of conducting an investigation into certain data integrity issues related to the TRANQUILITY II Phase 3 clinical trial and a number of independent third parties have been retained to conduct audits of the data integrity of the data from the clinical site where misconduct occurred. Our ongoing investigation and/or the planned independent audits may uncover new findings regarding the integrity of the trial data from this principal investigator's site, the accuracy of safety or efficacy findings, or the usability of the data in connection with a marketing application. We can provide no assurance regarding the timing of the completion of our own investigation, the timing of the completion of the independent audit of the trial site, or the outcome of the assessment and the impact on the study data from this clinical trial site or the impact of these issues on the study more broadly.

Moreover, the timing of our planned marketing application and prospects for regulatory approval of BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease may be adversely impacted by these developments. For example, even if we believe that data from the TRANQUILITY II trial have not been affected or compromised by the principal investigator's actions or other deficiencies at the trial site, the FDA may not accept or agree with our conclusions or analyses or may interpret or weigh their importance differently. Further, if we or the FDA determine that there are issues with data integrity and/or compliance with good clinical practice ("GCP") requirements at the trial site, we may be unable to use some or all of the subject data generated at this clinical site to support a marketing application. Any issues identified at this trial site may also be identified at other trial sites. If all of data were discarded, the TRANQUILITY II trial would no longer be adequately powered for statistical significance or would not be considered adequately well-controlled by the FDA, and in either case, we would need to conduct a new clinical trial before we are able to seek approval of BXCL501 for use in patients with Alzheimer's disease. If a substantial portion of the data were discarded, similar outcomes may also occur. Such a new clinical trial could take significant time, and there are no assurances we could raise the capital or have the liquidity to conduct such trial. If the Company conducts a new Phase 3 trial, such trial may have different safety or efficacy results from the topline data the Company previously announced for the TRANQUILITY II clinical trial. Further, any government investigation, disqualification, or debarment of, or proceeding or action against the principal investigator, or any

government investigation, proceeding or action against the Company, could further delay development and approval of BXCL501 for this indication, and otherwise have a material adverse effect on the Company, its financial condition (including triggering a potential event of default under our Credit Agreement), results of operations and prospects.

We have limited clinical data supporting potential safety or efficacy of BXCL501 for use in the at-home setting.

In August 2023, we announced our intention to pursue strategic reprioritization of our commercialization and development efforts. These actions include, among other things, a shift in focus to develop BXCL501 for use in the at-home treatment setting in the acute treatment of agitation in schizophrenia and bipolar disorders, and in both the at-home and ALF settings for the acute treatment of agitation in patients with mild-to-moderate dementia due to probable Alzheimer's dementia. Although we have conducted several clinical trials that evaluated BXCL501 in the institutional setting, we have limited data supporting BXCL501's potential use in the at-home setting. In particular, we have not conducted a clinical trial evaluating the at-home use of BXCL501 in Alzheimer's patients, and we have only completed Part 1 of our SERENITY III trial, evaluated the safety and efficacy of a 60 mcg dose of BXCL501 in patients with agitation associated with bipolar disorders or schizophrenia in an in-patient setting. Moreover, although we were encouraged by the topline results from Part 1 of SERENITY III we announced on May 25, 2023, the trial did not meet its primary efficacy endpoint of showing statistically significant separation from placebo on the mean change in total PEC score at 2 hours. Part 2 of the SERENITY III trial the study is primarily intended to evaluate the safety of BXCL501 over 12 weeks when used as-needed for episodes of agitation associated with schizophrenia and bipolar in the home setting. The primary objective of Part 2 is to describe the incidence of treatment-emergent adverse events, and not to evaluate efficacy. In addition, we plan to meet with the FDA to discuss our proposal to amend the SERENITY III Part 2 trial protocol to modify the dose evaluated in the study to include a single 80 mcg dose, rather than the 60 mcg dose we evaluated in Part 1 of the trial and the 60 mcg dose with optional second 60 mcg dose we are currently evaluating in Part 2 of the trial. Based on our prior data gathered from schizophrenia patients with the 80 mcg dose, as well as the results from our pharmacokinetic and pharmacodynamic modeling, we believe that 80 mcg may be an optimal dose to pursue for at home-use of BXCL501 in patients with agitation associated with schizophrenia and bipolar disorders, and plan to focus our SERENITY program on the 80 mcg dose. However, we have not previously evaluated the efficacy of an 80 mcg dose. Even if the protocol is amended to replace the current dosing regimen with a single 80 mcg dose in SERENITY III Part 2, and even if the study produces favorable data, such data may not be sufficient for approval without additional clinical studies evaluating efficacy of this dose.

Although we plan to seek feedback from the FDA regarding the potential of our ongoing or completed clinical trials to support submission of one or more sNDAs, it is possible that the FDA may not consider our available data adequate to support such submissions. In such case, we would be required to conduct additional clinical trials evaluating BXCL501 before we are able to submit an sNDA seeking approval of BXCL501 for at-home use in such population, if ever, which would increase our costs, and could have a material adverse effect on our prospects and results of operations.

In the near term, we are dependent on the success of IGALMI, and four of our product candidates, BXCL501, BXCL502, BXCL701 and BXCL702. If we are unable to complete the clinical development of or obtain marketing approval for our product candidates or successfully commercialize IGALMI and our other product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.

We currently have only one product that has received regulatory approval and may never be able to develop additional marketable product candidates. We are continuing to invest a significant portion of our efforts and financial resources in the commercialization of IGALMI and development of BXCL501, BXCL502, BXCL701 and BXCL702, as well as our other product candidates. In connection with the Reprioritization, we have significantly reduced the resources devoted to commercialization of IGALMI and it is possible that will have adverse consequences on the revenue that we are able to generate from IGALMI in the near term. 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 additional disease indications.

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

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

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

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

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

conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Top-line or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the top-line or preliminary data we previously published. For example, we are conducting an investigation into the data gathered during the TRANQUILITY II trial that could materially change the top-line results we reported on June 29, 2023, as described above in the risk factor entitled, "Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease." 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, 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 are 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 a product candidate's clinical development and may vary among jurisdictions. We obtained regulatory approval for our first product candidate for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder, which is in the early stages of commercialization. It is possible that none of our other product candidates, or any product candidates we may seek to develop in the future, will ever obtain regulatory approval.

Our current product candidates, or any that may be developed in the future, could fail to receive regulatory approval for many reasons, including the following:

- the FDA, or comparable foreign regulatory authorities, may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA, or comparable foreign regulatory authorities, that a product candidate is safe and effective for its proposed indication;

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

We have limited experience in completing clinical trials of product candidates. Consequently, we may not have the necessary capabilities, including adequate staffing, to successfully manage the execution and completion of 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 our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate or may restrict its distribution. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

We have only submitted one NDA to the FDA and have not submitted any similar marketing applications to comparable foreign authorities, for any product candidate, and we cannot be certain that our product candidates currently in development, or any than may be developed in the future, will be successful in clinical trials or receive regulatory approval. Further, our product candidates currently in development, or any than may be developed in the future, may not receive regulatory approval even if we believe they are successful in clinical trials. For example, the top-line results from the SERENITY III trial showed that Part 1 of the trial did not meet its primary efficacy endpoint. Although we are planning to seek FDA's feedback on whether the data from this trial, together with supporting data from our ongoing and completed clinical trials of BXCL501 in patients with bipolar disorders and schizophrenia, could support submission of a supplemental NDA seeking to expand IGALMI's label to include at-home use, there is no guarantee that the FDA will agree that the results from our completed, ongoing or proposed clinical trials will support any additional regulatory approvals for BXCL501. If we do not receive regulatory approvals for additional product candidates, we may not be able to continue our operations. For any 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 IGALMI or our other product candidates are not as significant as we estimate, we may not generate significant revenues from sales of IGALMI or such other product candidates, if approved.

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

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. For instance, the regulatory landscape related to clinical trials in the EU recently evolved. The EU Clinical Trials Regulation ("CTR"), which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. While the Clinical Trials Directive required a separate clinical trial application ("CTA"), to be submitted in each member state, to both the competent national health authority and an independent ethics committee, the CTR introduces a centralized process and only requires the submission of a single application to all member states concerned. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state. The assessment procedure of the CTA has been harmonized as well, including a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state's decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed. The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. For clinical trials whose CTA was made under the Clinical Trials Directive before January 31, 2022, the Clinical Trials Directive will continue to apply on a transitional basis for three years. Additionally, sponsors could choose to submit a CTA under either the Clinical Trials Directive or the CTR until January 31, 2023 and, if authorized, those are governed by the Clinical Trials Directive until January 31, 2025. By that date, all ongoing trials will become subject to the provisions of the CTR.

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

Clinical trials are expensive, time-consuming, difficult to design, difficult to conduct, 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, in accordance with applicable law and regulations. Failure can occur at any time during the clinical trial process. Although we are planning for certain clinical trials relating to BXCL501, BXCL701, BXCL502 and our other product candidates, there can be no assurance that the FDA, or other comparable foreign regulatory authorities, will accept our proposed trial designs as sufficient to establish the safety and/or efficacy of our product candidates.

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 ("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 health care resources to combat epidemics, such as occurred during the COVID-19 pandemic;
- obtaining institutional review board approval at each site, or independent ethics committee approval at any sites outside the U.S.;
- dependence on the needs and timing of third-party collaborators;
- changes to clinical trial protocols;

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

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board ("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, which increases the risk that such CROs or trial sites may fail to perform in accordance with regulatory requirements, clinical trial protocols or with the agreements governing their services to us. For example, investigator misconduct affecting our TRANQUILITY II trial, which evaluated BXCL501 in patients with probable

Alzheimer's disease, may have a material adverse impact on our development program for BXCL501 in these patients, as described more fully in the risk factor above entitled: "*Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease*."

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 due to differences in health care services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries. For example, if the current conflict between Russia and Ukraine spreads to other regions, it may adversely impact our ability to conduct trials.

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

We depend on enrollment of patients in our clinical trials 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, 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 has been, and may in the future be, impacted by governmental restrictions and diversion of health care resources resulting from COVID-19. Many pharmaceutical companies are conducting clinical trials in patients with the disease indications that our product candidates are designed to target. As a result, we must compete with them for clinical sites, physicians and the limited number of patients who fulfill the stringent requirements for participation in clinical trials. Also, due to the confidential nature of clinical trials, we do not know how many of the eligible patients may be enrolled in competing studies and who are consequently not available to us for our clinical trials. Our clinical trials may be delayed or terminated due to the inability to enroll enough patients. The delay or inability to meet planned patient enrollment may result in increased costs and delay or termination of our trials, which could have a harmful effect on our ability to develop products.

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

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, BXCL502, BXCL701, BXCL702 and our other product candidates in patients, in many cases, is ongoing and it is possible that there may be side effects associated with their use. Results of our trials could reveal a high and unacceptable severity and prevalence of these or other side effects. For example, in our Phase 2 clinical trial of BXCL701 for the treatment of emergent neuroendocrine prostate cancer, one patient experienced acidosis with a fatal outcome. Although the clinical investigator could not determine that the fatality was related to treatment with BXCL701, it is possible that BXCL701 could be tied to unacceptable side effects in the future.

If we observe drug-related AEs or other unacceptable safety concerns in clinical trials, we, the FDA, the IRBs at the institutions in which our studies are conducted, or the DSMB could suspend or terminate our clinical trials or the FDA, or comparable foreign regulatory authorities, could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. For example, the FDA placed Point Therapeutics, Inc.'s IND for BXCL701 on clinical hold following an increase in observed mortality in patients receiving BXCL701 in a Phase 3 trial in patients with non-small cell lung cancer. Though we believe that this result was caused by, among other things, an imbalance in the disease severity of patients enrolled in the active arm of the clinical trial, there is no guarantee that excess mortality will not be observed in future clinical studies. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the clinical trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles observed in our clinical trials and upon commercialization of any of our product candidates that may receive regulatory approval. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if we or others later identify undesirable side effects caused by IGALMI or any other product candidate that receives marketing approval, a number of potentially significant negative consequences could result, including:

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

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

The discovery and development of product candidates based on EvolverAI, BioXcel LLC's proprietary pharmaceutical discovery and development engine, and our artificial intelligence ("AI") platform is novel and unproven, and we do not know whether we will be able to develop any products of commercial value.

We are leveraging the Company's AI platform and BioXcel LLC's EvolverAI, a proprietary pharmaceutical discovery and development engine, 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 the Company's AI platform and BioXcel LLC's EvolverAI to create medicines for defined patient populations may potentially enable drug research and clinical development that is more efficient than conventional drug research and development, our approach is novel. Although we obtained FDA approval for IGALMI, because our approach is novel, the cost and time needed to develop our product candidates is difficult to predict, and our efforts may not result in the discovery and development of commercially viable medicines. We may also be incorrect about the effects of our product and 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.

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

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

- research programs to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product candidates. If we are unable to identify suitable additional compounds for preclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price;

- compounds found through the Company's AI platform and BioXcel LLC's EvolverAI may not demonstrate efficacy, safety or tolerability;

- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance;

- competitors may develop alternative therapies that render our potential product candidates non-competitive or less attractive; or

- a potential product candidate may not be capable of being produced at an acceptable cost.

Regulators may limit our ability to develop or implement our proprietary AI algorithms and/or may eliminate or restrict the confidentiality of our proprietary technology, which could have an adverse effect on our business, results of operations, and financial condition.

Our future success depends on our ability to continue to develop and implement our proprietary AI algorithms and models, and to maintain the confidentiality of this technology. Changes to existing regulations, their interpretation or implementation, or new regulations could impede our use of this technology or require that we disclose our proprietary technology to our competitors, which could impair our competitive position and result in an adverse effect on our business, results of operations and financial condition.

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

If a product candidate is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA Fast Track designation. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the applicable FDA review team during product development and, once an NDA is submitted, the product candidate may be eligible for priority review if the relevant criteria are met. A Fast Track product candidate may also be eligible

for rolling review, where the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA. We obtained Fast Track designation for BXCL501 for the acute treatment of mild-to-moderate agitation associated with schizophrenia, bipolar disorder, and dementia, and we may seek Fast Track designation for other indications or for one or more of our other product candidates, but we might not receive such designations from the FDA. However, even if we receive Fast Track designation, Fast Track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with Fast Track designation compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA's priority review procedures.

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

We obtained Breakthrough Therapy Designation for BXCL501 for the acute treatment of agitation associated with dementia, and we may seek additional Breakthrough Therapy designations for our product candidates if the clinical data support such a designation for one or more product candidates. A Breakthrough Therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Product candidates designated as Breakthrough Therapies by the FDA also receive the benefits associated with Fast Track designation, including the potential for rolling review of an NDA.

Designation as a Breakthrough Therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a Breakthrough Therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under non-expedited FDA review procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the period for FDA review or approval will not be shortened.

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

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

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

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

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

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

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

The regulation of companion diagnostics in the EU will be subject to further requirements since the IVDR introduces a new classification system for companion diagnostics. 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 ("MA") application for the medicinal product has been submitted through the centralized procedure. For other substances, the notified body can seek the opinion from a national competent authority or the EMA.

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

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

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

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

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

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

Further, the policies of the FDA and other regulatory authorities may change, and additional government regulations may be enacted that could impose extensive and ongoing regulatory requirements and obligations on any product candidate for which we obtain marketing approval. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the U.S. or abroad.

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

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

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

Regulatory authorities in the U.S. generally do not regulate the behavior of physicians in their choice of treatments. Regulatory authorities do, however, restrict communications by pharmaceutical companies on off-label use. If we are found to have promoted our products for any off-label uses, the U.S. federal government (and other foreign governments) could levy civil, criminal and/or administrative penalties, and seek fines against us. The FDA, or other

regulatory authorities, could also require that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of IGALMI or our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

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

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

Separately, in response to the COVID-19 pandemic, the FDA postponed most inspections of domestic and foreign manufacturing facilities at various points. Even though the FDA has since resumed standard inspection operations of domestic facilities where feasible, the FDA has continued to monitor and implement changes to its inspectional activities to ensure the safety of its employees and those of the firms it regulates as it adapts to the evolving COVID-19 environment, and any resurgence of the virus or emergence of new variants may lead to further inspectional delays. Regulatory authorities outside the U.S. have adopted similar restrictions or other policy measures in response to COVID-19. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

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

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

consuming, and could result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction.

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

In addition to regulations in the U.S., should we or our collaborators pursue marketing approvals for IGALMI, and for BXCL501, BXCL502, BXCL701, BXCL702 and our other product candidates internationally, we and our collaborators will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we, or our collaborators, obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country.

We expect to pursue marketing approvals for IGALMI, and may pursue marketing approvals for BXCL501, BXCL502, BXCL701, BXCL702 and our other product candidates in Europe and other jurisdictions outside the U.S. with collaborative partners. The time and process required to obtain regulatory approvals and reimbursement in Europe and other jurisdictions may be different from those in the U.S. Also, regulatory approval in one jurisdiction does not ensure approvals in any other jurisdiction; however, negative regulatory decisions in any jurisdiction may have a negative impact on the regulatory process in other jurisdictions.

Following a national referendum and enactment of legislation by the government of the United Kingdom (“UK”), the UK formally withdrew from the EU on January 31, 2020 and ratified a trade and cooperation agreement governing its future relationship (commonly referred to as “Brexit”). The agreement, which was applied provisionally from January 1, 2021 and entered into force on May 1, 2021, addresses trade, economic arrangements, law enforcement, judicial cooperation and a governance framework including procedures for dispute resolution, among other things. Because the agreement merely sets forth a framework in many respects and will require complex additional bilateral negotiations between the UK and the EU as both parties continue to work on the rules for implementation, significant political and economic uncertainty remains about how the precise terms of the relationship between the parties will differ from the terms before withdrawal.

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

Therefore, there remains political and economic uncertainty regarding to what extent the regulation of medicinal products will differ between the UK and the EU in the future. Any divergences will increase the cost and complexity of running our business, including with respect to the conduct of clinical trials. Brexit also materially impacted the

regulatory regime with respect to the approval of our product candidates. Great Britain is no longer covered by the EU's procedures for the grant of MAs (Northern Ireland is covered by the centralized authorization procedure and can be covered under the decentralized or mutual recognition procedures). As of January 1, 2021, all existing centralized MAs were automatically converted into UK MAs effective in Great Britain and issued with a UK MA number on January 1, 2021 (unless MA holders opted out of this scheme). A separate MA is now required to market drugs in Great Britain. It is currently unclear whether the regulator in the UK, the MHRA, is sufficiently prepared to handle the increased volume of MA applications that it is likely to receive. Any delay in obtaining, or an inability to obtain, any regulatory approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in Great Britain and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in Great Britain for our product candidates, which could significantly and materially harm our business. The UK's withdrawal from the EU and the associated uncertainty has had and may continue to have a significant adverse effect on global economic conditions and the stability of global financial markets and could significantly reduce global market liquidity and restrict the ability of key market participants to operate in certain financial markets. Asset valuations, currency exchange rates and credit ratings may be especially subject to increased market volatility. Any of these factors could have a significant adverse effect on our business, financial condition, results of operations and prospects.

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

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

In the U.S., we are subject to various federal and state 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, and our ability to successfully commercialize our products in the U.S. We may have to comply with similar laws and regulations outside the U.S. These laws include:

- the federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug for which payment may be made under a federal 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 ("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 health care 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 health care program, unless an exception applies;

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

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

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws, which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to health care 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 health care 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 health care providers.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state or foreign health care programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, any of which could adversely affect our ability to market our products and adversely impact our financial results.

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

Our inability to retain sufficient clinical trial liability insurance at an acceptable cost to protect against potential liability claims could prevent or inhibit our ability to conduct clinical trials for product candidates we develop. We may be unable to obtain appropriate levels of such insurance. Even if we do secure clinical trial liability insurance for our programs, we may not be able to achieve sufficient levels of such insurance. Any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that exceeds the limits of our insurance coverage. We have supplemented our clinical trial coverage with product liability coverage in connection with the commercial launch of IGALMI and expect that we would similarly supplement our coverage for any of our other product candidates that may receive regulatory approval, but 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 or if we fail to accurately forecast demand or manage our inventories, our business will suffer because we might not be able to fund future operations.

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

- the price of our products relative to other products for the same or similar treatments;
- the perception by patients, physicians and other members of the health care community of the effectiveness, utility 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.

Our results of operations could be materially harmed if we are unable to accurately forecast customer demand for IGALMI and manage our inventory. To ensure adequate inventory supply, we must forecast inventory needs and place orders with our suppliers based on our estimates of future demand for IGALMI. Our ability to accurately forecast demand for IGALMI could be negatively affected by many factors, including our failure to accurately manage our expansion strategy, product introductions by competitors, an increase or decrease in customer demand for IGALMI or for products of our competitors, our failure to accurately forecast customer acceptance of new products, unanticipated changes in general market conditions or regulatory matters, and weakening of economic conditions or consumer confidence in future economic conditions. Inventory levels in excess of customer demand may result in inventory write-downs or write-offs, which would cause our gross margin to be adversely affected and could impair the strength of our brand. Conversely, if we underestimate customer demand for IGALMI, our third-party contract manufacturer may not be able to deliver products to meet our requirements, and this could result in damage to our reputation and customer relationships. In addition, if we experience a significant increase in demand, additional supplies of raw materials or additional manufacturing capacity may not be available when required on terms that are acceptable to us, or at all, or suppliers or our third-party manufacturers may not be able to allocate sufficient capacity in order to meet our increased requirements, which could have an adverse effect on our ability to meet customer demand for IGALMI and our results of operations.

We seek to maintain sufficient levels of inventory to protect ourselves from supply interruptions. As a result, we are subject to the risk that a portion of our inventory will become obsolete or expire, which could have a material adverse effect on our earnings and cash flows due to the resulting costs associated with the inventory impairment charges and costs required to replace such inventory.

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

Regulatory authorities in some jurisdictions, including the U.S. and EU, may designate drugs intended for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the U.S., or a patient population greater than 200,000 individuals in the U.S. where

there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, orphan drug designation is granted by the European Commission based on a scientific opinion of the EMA's Committee for Orphan Medicinal Products. A medicinal product may be designated as orphan if its sponsor can establish that (i) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects no more than 5 in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (iii) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the medicinal product will be of significant benefit to those affected by the condition. The application for orphan designation must be submitted before the application for MA.

In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the U.S. provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same disease or condition for seven years. In limited circumstances, the applicable exclusivity period is 10 years in the EU. The EU exclusivity period can be reduced to six years if, at the end of the fifth year, it is established that a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

In January 2021, the FDA granted Orphan Drug Designation to BXCL701 for the treatment of soft tissue sarcoma. In September 2019, the FDA granted Orphan Drug Designation to BXCL701 for the treatment of acute myeloid leukemia. Prior to 2019, the FDA granted Orphan Drug Designation to BXCL701 for the treatment of pancreatic cancer and melanoma. We may seek Orphan Drug Designations for BXCL701 in other diseases or conditions or for other product candidates. There can be no assurances that we will be able to obtain such designations.

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

If we are unable to develop satisfactory sales and marketing capabilities, we may not succeed in commercializing IGALMI or any product candidate for which we may obtain regulatory approval.

We have limited experience in marketing and selling drug products. We have not entered into arrangements for the sale and marketing of IGALMI, BXCL501, BXCL502, BXCL701, BXCL702 or any other product candidate. Typically, pharmaceutical companies would employ groups of sales representatives and associated sales and marketing staff numbering in the hundreds to thousands of individuals to call on the large number of physicians and hospitals. Following our Reprioritization, we may need to rebuild a commercial sales and marketing team if we seek to modify

our commercial strategy for IGALMI or initiate commercial sales for any product candidate in the future, which will likely require significant cost. 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. We may also need to hire additional personnel skilled in marketing and sales for our direct marketing and selling efforts. 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 maintenance and expansion of our direct sales force or establishment of a contract sales force, or a combination thereof, as applicable, to market our products is expensive and time-consuming and could delay any product launch. In addition, reputational harm from the Reprioritization may adversely impact our efforts to hire personnel skilled in marketing and sales. Further, we can give no assurances that we will be able to maintain a direct and/or contract sales force for any period of time or that our sales efforts will be sufficient to grow our revenues or that our sales efforts will ever lead to profits. A direct sales force has in the past and may in the future subject us to higher fixed costs than those of companies that market competing products through independent third parties, due to the costs that we bear associated with employee benefits, training, and managing sales personnel. As a result, we could be at a competitive disadvantage. Additionally, these fixed costs may slow our ability to reduce costs if needed, which could have a material adverse effect on our business, financial condition, and results of operations.

We operate in a highly competitive and rapidly changing industry.

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

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

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

Established biopharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety 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 products and product candidates, if any, that we commercialize. The inability to compete with existing or subsequently introduced drugs would harm our business, financial condition and results of operations.

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

There can be no assurance that IGALMI, or BXCL501, BXCL502, BXCL701, BXCL702 and our other product candidates or any other product candidate successfully developed by us, independently or with partners, if approved, will be accepted by physicians, hospitals and other health care facilities. IGALMI competes, and BXCL501, BXCL502, BXCL701, BXCL702 and any future product candidates we develop will compete, with a number of products manufactured and marketed by major pharmaceutical and biotechnology companies. The degree of market acceptance of IGALMI and any drugs we develop depends on a number of factors, including:

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

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

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

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

The U.S. government and other governments have shown significant interest in pursuing health care reform. Any government-adopted reform measures could adversely impact the pricing of health care products and services in the U.S. or internationally and the amount of reimbursement available from governmental agencies or other third-party payors. The continuing efforts of the U.S. and foreign governments, insurance companies, managed care organizations and other payors of 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 health care availability, methods of delivery or payment for products and services, or sales, marketing or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons, including policies advanced by the current executive administration in the U.S., new health care legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

For example, in the U.S., the ACA has substantially changed the way health care is financed by both government health plans and private insurers, and significantly impacts the pharmaceutical industry. For example, the ACA imposed a non-deductible excise tax on pharmaceutical manufacturers or importers that sell branded prescription drugs to government programs. 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 required to provide a discount on branded prescription drugs 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 changes to the Public Health Service's 340B drug pricing program (the "340B program") including expansion of the list of eligible covered entities that may purchase drugs under the program.

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

In addition, other legislative changes have been proposed and adopted in the U.S. since the ACA was enacted. These changes include the Budget Control Act of 2011, which resulted in aggregate reductions of Medicare payments to providers, which went into effect on April 1, 2013, and, due to subsequent legislative amendments to the statute, will remain in effect through 2032, unless additional Congressional action is taken. Furthermore, the American Taxpayer Relief Act of 2012, further reduced Medicare payments to several types of providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. More recently, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, beginning January 1, 2024.

Most significantly, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 ("IRA") into law. This statute marks the most significant action by Congress with respect to the pharmaceutical industry since adoption of the ACA in 2010. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services to implement many of these provisions through guidance, as opposed to regulation, for the initial years. For that and other reasons, it is currently unclear how the IRA will be effectuated, and while the impact of the IRA on the pharmaceutical industry and our business cannot yet be fully determined, it is likely to be significant.

The cost of prescription pharmaceuticals in the U.S. will likely continue to be the subject of considerable discussion. Members of Congress and the Biden Administration have indicated they will continue to pursue further legislative or administrative measures to control prescription drug costs. There have been several Congressional inquiries, as well as legislative and regulatory initiatives and executive orders designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. We cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

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

It is likely that federal and state legislatures within the U.S. and foreign governments will continue to consider changes to existing health care legislation. We cannot predict the reform initiatives that may be adopted in the future or whether initiatives that have been adopted will be repealed or modified. The continuing efforts of governments, insurance companies, managed care organizations and other payors of health care services to contain or reduce costs of health care may adversely affect the demand for IGALMI and any other 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.

In the EU, similar developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the EU, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the U.S. and EU, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

On December 13, 2021, Regulation No 2021/2282 on Health Technology Assessment (“HTA”) amending Directive 2011/24/EU, was adopted. While the regulation entered into force in January 2022, it will only begin to apply from January 2025 onwards, with preparatory and implementation-related steps to take place in the interim. Once the regulation becomes applicable, it will have a phased implementation depending on the concerned products. This regulation intends to boost cooperation among EU member states in assessing health technologies, including new medicinal products as well as certain high-risk medical devices, and providing the basis for cooperation at the EU level for joint clinical assessments in these areas. The regulation will permit EU member states to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment

of the innovative health technologies with the most potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement.

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

We participate in the Medicaid Drug Rebate Program ("MDRP") and other federal and state government pricing programs in the U.S., and we may participate in additional government pricing programs in the future. These programs generally require manufacturers to pay rebates or otherwise provide discounts to government payors in connection with drugs that are dispensed to beneficiaries of these programs. As a condition of having federal funds being made available for covered outpatient drugs under Medicaid and Medicare Part B, a manufacturer must enroll in the MDRP. Under this program, we must pay a rebate to state Medicaid programs for each unit of our covered outpatient drug dispensed to a Medicaid beneficiary and paid for by a state Medicaid program. Medicaid drug rebates are based on pricing data that we must report on a monthly and quarterly basis to CMS. For the MDRP, this data includes the average manufacturer price ("AMP") for each drug and, in the case of an innovator product, like IGALMI, the best price. If we become aware that our MDRP price reporting submission for a prior period was incorrect or has changed as a result of recalculation of the pricing data, we must resubmit the corrected data for up to three years after the data originally was due. Further, under the IRA, AMP figures we report will also be used to calculate a rebate on Medicare Part D utilization, triggered by price increases that outpace inflation. If we fail to provide information timely or are found to have knowingly submitted false information to the government, we may be subject to civil monetary penalties and other sanctions, including termination from the MDRP, which would result in payment not being available for our covered outpatient drugs under Medicaid or, if applicable, Medicare Part B. Failure to make necessary disclosures and/or to identify overpayments additionally could result in allegations against us under the Federal False Claims Act and other laws and regulations.

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

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

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

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

Risks Related to Our Relationship with BioXcel LLC

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

As of June 30, 2023, BioXcel LLC owned approximately 29% of the economic interest and voting power of our outstanding common stock and BioXcel LLC is controlled by BioXcel Holdings, Inc. Dr. Vimal Mehta is a co-founder and significant stockholder of BioXcel Holdings, Inc. Krishnan Nandabalan is a co-founder and serves as a senior executive and member of the board of BioXcel LLC and BioXcel Holdings, Inc. See "*The management of and beneficial ownership in BioXcel LLC by our executive officers and our directors may create, or may create the appearance of, conflicts of interest.*" below. Even though BioXcel LLC controls less than a majority of the voting power of our outstanding common stock, it may influence the outcome of such corporate actions so long as it owns a significant portion of our common stock.

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

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

No assurance can be given that any equity holder of BioXcel LLC or the Company will not claim in a lawsuit that such terms in fact are not in the best interests of BioXcel LLC or the Company and its applicable equity holders, that the directors and officers of BioXcel LLC or the Company breached their fiduciary duties in connection with such agreements and that any disclosures by the Company 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 would seek indemnification from BioXcel LLC under the terms of these agreements against any damages or other costs, which could be substantial, no such indemnification has yet been agreed to or may be agreed to and be in effect. Further, any such litigation would be time-consuming and would divert focus and resources from the development of our product candidates and our business, including but not limited to possibly delaying our clinical trials due to our management having to spend time and attention on such litigation.

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

We rely, in part, on BioXcel LLC and access to its EvolverAI, a research and development engine created and owned by BioXcel LLC, to identify, research and develop potential product candidates in neuroscience and immuno-oncology. We negotiated the Services Agreement with BioXcel LLC pursuant to which BioXcel LLC shall perform product identification and related services for us utilizing its EvolverAI. Under the Services Agreement, we have an option, exercisable until December 31, 2024, to enter into a collaborative services agreement with BioXcel LLC pursuant to which BioXcel LLC shall perform product identification and related services for us utilizing its EvolverAI. To maintain the ability to exercise the foregoing option, pursuant to an amendment to the Services Agreement effective as of April 19, 2022, the Company agreed to pay BioXcel LLC $18,000 per month from March 13, 2023, to December 31, 2024. The parties are obligated to negotiate the collaborative services agreement in good faith and to incorporate reasonable market-based terms, including consideration for BioXcel LLC reflecting a low, single-digit royalty on net sales and reasonable development and commercialization milestone payments, provided that (i) development milestone payments shall not exceed $10 million in the aggregate and not be payable prior to proof of concept in humans and (ii) commercialization milestone payments shall be based on reaching annual net sales levels, be limited to 3% of the applicable net sales level, and not exceed $30 million in the aggregate. BioXcel LLC shall continue to make such product identification and related services available to us until at least December 31, 2024.

In addition, at the time of our initial public offering (“IPO”), BioXcel LLC granted us (i) 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 and (ii) an exclusivity agreement in the neuroscience and immuno-oncology fields whereby BioXcel LLC agreed not develop drugs, or engage in preclinical discovery for the purpose of developing drugs, in the neuroscience and immuno-oncology fields for or on behalf of a third party, utilizing EvolverAI or otherwise. This first right to negotiate and exclusivity period expired on March 12, 2023 and there is no assurance that we will extend the terms of the agreement. We are continuing to assess our ongoing business needs. If our rights under the Services Agreement were to become limited or if we are otherwise precluded from conducting research and development using EvolverAI, or if BioXcel LLC does not 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 we cannot control whether 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. BioXcel LLC is not currently restricted from using EvolverAI to perform drug discovery services for our direct competitors, and if we do not extend the exclusivity period in the neuroscience and immuno-oncology fields, this could harm our competitive position and adversely affect our future operating results and financial condition.

The management of and beneficial ownership in BioXcel LLC may create, or may create the appearance of, conflicts of interest.

The management of and beneficial ownership in BioXcel LLC by our executive officer and our directors may create, or may create the appearance of, conflicts of interest. For example, our Chief Executive Officer and director on our Board, Vimal Mehta, Ph.D., is a co-founder and significant stockholder of BioXcel Holdings, Inc., which controls BioXcel LLC. A director on our Board, Krishnan Nandabalan, Ph.D., is a manager and officer of BioXcel LLC, as well as a director, officer, and significant stockholder of BioXcel Holdings, Inc. Additionally, as of June 30, 2023, Dr. Nandabalan, through his beneficial ownership of BioXcel LLC, owned approximately 30% of the Company. Management and ownership of BioXcel LLC by affiliates, 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 and 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 and expose us to litigation risk.

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

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

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

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

BioXcel LLC may experience challenges with the acquisition, development, enhancement, or deployment of technology necessary for EvolverAI. We may face similar challenges with other AI platforms that we utilize, including our own in-house proprietary platform.

BioXcel LLC operates in businesses that require sophisticated computer systems and software for data collection, data processing, cloud-based platforms, analytics, statistical projections and forecasting, mobile computing, social media analytics and other applications and technologies. BioXcel LLC seeks to address its technology risks by increasing its reliance on the use of innovations by cross-industry technology leaders and adapt these for their pharmaceutical, biotechnology, biopharmaceutical, diagnostic, medical device and contract research and manufacturing clients. Some of the technologies supporting the industries they serve are changing rapidly and we must continue to adapt to these changes in a timely and effective manner at an acceptable cost. They also must continue to deliver data to their clients in forms that are easy to use while simultaneously providing clear answers to complex questions. We also utilize our own in-house AI platform.

There can be no guarantee that we or BioXcel LLC will be able to develop, acquire or integrate new technologies, that these new technologies will meet our and BioXcel LLC's needs or achieve our expected goals, or that we will be able to do so as quickly or cost-effectively as our competitors. Significant technological change could render BioXcel LLC's EvolverAI or other AI platforms that we utilize obsolete. BioXcel LLC's and our 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. If EvolverAI or other AI and machine learning models that we use are incorrectly designed, do not operate properly, the data we use to train them is incomplete, inadequate or biased in some way, or if we do not have sufficient

rights to use the data on which our AI and machine learning models rely, the performance of our products, services and businesses, as well as our reputation, could suffer or we could incur liability through the violation of laws, third-party privacy rights or contracts to which we are a party. BioXcel LLC or we may experience difficulties that could delay or prevent the successful design, development, testing, and introduction of advanced versions of EvolverAI, limiting our ability to identify new product candidates. New services, or enhancements to existing EvolverAI services, may not adequately meet our requirements. Any of these failures could have a material adverse effect on our operating results and financial condition.

Risks Related to Our Reliance on Third Parties

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

We entered into a commercial supply agreement with ARx, LLC ("ARx") pursuant to which ARx has agreed to exclusively manufacture and supply us with all of our worldwide demand of film formulation of Dex to be used for the commercial supply of IGALMI and for ongoing clinical trials of our product candidate BXCL501, subject to certain alternative supply provisions. If ARx is unable or ceases to produce our supply of Dex in sufficient quantities as and when needed, our business would be harmed because there can be no assurance that we will be able to identify or enter into agreements with alternative suppliers on a timely basis on acceptable terms, if at all. An interruption in our ability to sell our products to customers could occur if we encounter delays or difficulties in securing Dex, or if the quantity or quality supplied does not meet our specifications, or if we cannot then obtain an acceptable substitute. If any of these events occur, our business and operating results could be harmed. Our specified minimum annual payment could adversely affect our cash flows, such as in times when we have sufficient inventory and would otherwise be able to use our cash for other purposes.

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

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

We, ARx, the Patheon pharma services division of Thermo Fisher Scientific Inc., and/or our other third-party manufacturers may be adversely affected by developments outside of our control, and these developments may delay or

prevent further manufacturing of our products. Adverse developments may include labor disputes, resource constraints, shipment delays, inventory shortages, lot failures, unexpected sources of contamination, lawsuits related to our manufacturing techniques, equipment used during manufacturing, or composition of matter, unstable political environments, acts of terrorism, war, natural disasters, and other natural and man-made disasters. If BioXcel LLC, we, ARx, the Patheon pharma services division of Thermo Fisher Scientific Inc., or our other third-party manufacturers were to encounter any of the above difficulties, or otherwise fail to comply with contractual obligations, our ability to provide any product for clinical trial or commercial purposes would be jeopardized. This may increase the costs associated with completing our clinical trials and commercial production. Further, production disruptions may cause us to terminate ongoing clinical trials and/or commence new clinical trials at additional expense. We may also have to take inventory write-offs and incur other charges and expenses for products that fail to meet specifications or pass safety inspections. Moreover, as a result of the COVID-19 pandemic, third-party manufacturers have been and may in the future be affected, which could disrupt their activities and, as a result, we could face difficulty sourcing key components necessary to produce supply of our commercial product and product candidates, which may negatively affect our preclinical and clinical development activities. If production difficulties cannot be solved with acceptable costs, expenses, and timeframes, we may be forced to abandon our clinical development and commercialization plans, which could have a material adverse effect on our business, prospects, financial condition, and the value of our securities.

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

In order to conduct clinical trials of our product candidates and commercialize any approved product candidates, we, or our manufacturers, including ARx, and the Patheon pharma services division of Thermo Fisher Scientific Inc., 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 approved products or 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 approved products or 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 approved products, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our approved products or 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 products and product candidates may include the formation of collaborative arrangements with third parties. Collaborators have significant discretion in determining the efforts and resources they apply and may not perform their obligations as expected. Potential third-party collaborators include biopharmaceutical, pharmaceutical and biotechnology companies, academic institutions and other entities. Third-party collaborators may assist us in:

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

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

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

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

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

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

In addition if any of our relationships with our 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 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. For example, investigator misconduct during our TRANQUILITY II trial evaluating BXCL501 in patients with probable Alzheimer's disease could require us to conduct additional clinical trials before we are able to seek or obtain approval for BXCL501 for use in this patient population, as described more fully in the risk factor above entitled: "*Developments relating to our TRANQUILITY II Phase 3 trial may impact the timing of our development plans for, and prospects for seeking or obtaining regulatory approval of, BXCL501 for the acute treatment of agitation associated with dementia in patients with probable Alzheimer's disease*." As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If the third parties conducting our GCP preclinical studies or our clinical trials do not perform their contractual duties or obligations, experience work stoppages, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the clinical data they obtain is compromised due to their failure to adhere to our clinical trial protocols or to GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties. Switching or adding CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences

work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Risks Related to Our Business and Industry

Pandemics, epidemics or outbreaks of an infectious disease, such as COVID-19, may materially and adversely impact our business, including our preclinical studies and clinical trials.

As a result of 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 health care resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site data monitoring, due to limitations on travel imposed or recommended by governments, employers and others or interruption of clinical trial subject visits and study procedures, which may impact the integrity of subject data and clinical study endpoints;
- interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;
- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems;
- interruptions in preclinical studies due to restricted or limited operations resulting from restrictions on our on-site activities;
- limitations on employee resources that would otherwise be focused on the conduct of our preclinical studies and clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- impacts from prolonged remote work arrangements, such as strains on our business continuity plans, cybersecurity risks, and inability of certain employees to perform their work remotely; and
- interruption or delays to our sourced discovery and clinical activities.

If we or any of the third parties with whom we engage were to experience shutdowns or other business disruptions from COVID-19 or other pandemics, epidemics or outbreaks of infectious disease, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted.

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 with BioXcel LLC. We have expanded our management team to include an operational ramp-up of additional technical staff required to achieve our business objectives. We may need to continue to expand our managerial, commercial, operational, technical, and scientific, financial, and other resources to manage our

operations and clinical trials, continue our research and development activities, and any approved product candidates. Our management and scientific personnel, systems and facilities currently in place may not be adequate to support our future growth.

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

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

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

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

Our success depends largely upon the continued services of our key executive officers, Vimal Mehta, our Chief Executive Officer, President and a member of our Board, as well as the other principal members of our management, scientific, clinical teams and commercial readiness teams. We do not maintain “key person” insurance for any of these executive officers or any of our other key employees. We also rely on our leadership team in the areas of research and development, marketing, services and selling, general and administrative functions. From time to time, there may be changes in our executive management and leadership teams resulting from the hiring or departure of executives or other key employees, which could disrupt our business. The replacement of one or more of our executive officers or other key employees would likely involve significant time and costs and may significantly delay or prevent the achievement of our business objectives.

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

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

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

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

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

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

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

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

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

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with any regulations applicable to us, to provide accurate information to regulatory authorities, to comply with manufacturing standards we have established, to comply with federal and state health care 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 health care 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 during 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, and other natural and man-made disaster or business interruptions. Our phones, electronic devices and computer systems and those of our directors, advisors, contractors, consultants, CROs, and collaborators are vulnerable to damages, theft and accidental loss, negligence, unauthorized access, terrorism, war, electronic and telecommunications failures, and other natural and man- made disasters. Several of our employees conduct business outside of our headquarters and leased or owned facilities. These locations may be subject to additional security and other risk factors due to the limited control of our employees. If such an event as described above were to occur in the future, it may cause interruptions in our operations, delay research and development programs, clinical trials, regulatory activities, manufacturing and quality assurance activities, sales and marketing activities, hiring, training of employees and persons within associated third parties, and other business activities. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

Likewise, we will rely on third parties, including ARx, to manufacture IGALMI and our product candidates and to conduct clinical trials, and similar events as those described in the prior paragraph relating to their business systems, equipment and facilities could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidate could be delayed or altogether terminated.

Data breaches or cyber-attacks could disrupt our business operations and information technology systems or those of third parties on which we rely, 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 and those of third parties on which we rely 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, or those of third parties on which we rely, could occur through a variety of methods such as from cyber-attacks and cyber-intrusions over the Internet, malware, computer viruses, email spoofing, attachments to e-mails, persons inside or outside our organization or persons with access to systems inside our organization. The risk of such intrusions, threats to data and information technology systems and breaches has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We use our information technology systems to protect confidential or sensitive product candidate, clinical trial, employee and Company information.

Any attack on such systems that results in disruptions to our operations, or the unauthorized release or loss of such information, could have a material adverse effect on our business reputation, increase our costs and expose us to material legal claims and liability. If the unauthorized release or loss of product candidate, clinical trial, employee or other confidential or sensitive data were to occur, our operations and financial results and our share price could be adversely affected.

While we maintain some of our own critical information technology systems, we also depend on third parties to provide important information technology services relating to several key business functions. Our measures to prevent, detect and mitigate these threats, including password protection, firewalls, backup servers, threat monitoring and periodic penetration testing, may not be successful in preventing a data breach or limiting the effects of a breach. Because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may also experience security breaches that may remain undetected for an extended period. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence. Furthermore, the security measures employed by third-party service providers may prove to be ineffective at preventing breaches of their systems. Although we maintain insurance for our business, the coverage under our policies may not be adequate to compensate us for all losses that may occur.

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

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal data, such as information that we may collect in connection with clinical trials in the U.S. and abroad. Additionally, our use of AI and machine learning may be subject to laws and evolving regulations regarding the use of AI or machine learning, controlling for data bias, and anti-discrimination. 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. Most healthcare providers, including research institutions from which we obtain patient health information, are subject to privacy and security regulations promulgated under HIPAA. If we are determined to act as a covered entity or business associate under HIPAA and be directly regulated under HIPAA, any person acting on our behalf may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information.

Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. Such laws and regulations will be subject to interpretation by various courts and other governmental authorities, thus creating potentially complex compliance issues for us and our future customers and strategic partners. In addition, the California Consumer Privacy Act ("CCPA") went into effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal information. The CCPA provides for civil penalties for violations, as well as a private

right of action for data breaches that has increased the likelihood of, and risks associated with data breach litigation. The CCPA may increase our compliance costs and potential liability, and many similar laws have been proposed at the federal level and in other states in the U.S. Further, the California Privacy Rights Act ("CPRA") passed in California, generally went into effect in January 2023, and significantly amends CCPA, imposing 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 also creates a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. Additional compliance investment and potential business process changes may be required. If we are subject to or affected by HIPAA, the CCPA, the CPRA or other domestic privacy and data protection laws, any liability from failure to comply with the requirements of these laws could adversely affect our financial condition.

In Europe, the General Data Protection Regulation ("GDPR") went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the U.S. In July 2020, the Court of Justice of the EU ("CJEU") limited how organizations could lawfully transfer personal data from the EU/EEA to the U.S. by invalidating the Privacy Shield for purposes of international transfers and imposing further restrictions on the use of standard contractual clauses ("SCCs"). In March 2022, the U.S. and EU announced a new regulatory regime intended to replace the invalidated regulations; however, this new EU-US Data Privacy Framework has not been implemented beyond an executive order signed by President Biden on October 7, 2022 on Enhancing Safeguards for United States Signals Intelligence Activities. European court and regulatory decisions subsequent to the CJEU decision of July 2020 have taken a restrictive approach to international data transfers. As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the standard contractual clauses cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results.

Further, from January 1, 2021, companies have had to comply with the GDPR and also the UK GDPR, which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR (i.e., fines up to the greater of £17,500 or 4% of global turnover).

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.

Increased scrutiny of and evolving expectations for environmental, social and governance ("ESG") initiatives may impose additional costs or otherwise adversely impact our business.

There has been an increased focus from investors, capital providers, shareholder advocacy groups, other market participants, customers, and other stakeholder groups regarding companies' ESG initiatives. While we may at times engage in voluntary initiatives (such as voluntary disclosures, certifications, or goals, among others) or commitments to improve the ESG profile of our Company and/or offerings, such initiatives or achievements of such commitments may be costly and may not have the desired effect. Additionally, some investors may use third-party or proprietary ESG ratings to guide their investment strategies and, in some cases, may choose not to invest in us if they believe our ESG practices are inadequate. The criteria by which companies' ESG practices are assessed are evolving, which could result in greater expectations of us and cause us to undertake costly initiatives to satisfy such new criteria. Alternatively, if we

elect not to or are unable to satisfy new criteria or do not meet the criteria, some investors may conclude that our policies with respect to ESG are inadequate and choose not to invest in us.

If our ESG practices do not meet evolving investor or other stakeholder expectations and our standards, reputation, ability to attract or retain employees and desirability as an investment or business partner could be negatively impacted. Similarly, our failure or perceived failure to adequately pursue or fulfill any ESG goals and objectives or to satisfy various reporting standards, if any, could expose us to additional regulatory, social or other scrutiny, the imposition of unexpected costs, or damage to our reputation, which in turn could have a material adverse effect on our business and could cause the market value of our common stock to decline.

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

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

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

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

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

Our ability to use our net operating losses and tax credits to offset future taxable income and income tax liabilities may be limited.

As of December 31, 2022, the Company had federal net operating loss carryforwards ("NOLs") of approximately $222.4 million and state NOLs of approximately $214.5 million. If not utilized, the federal and state NOLs, which are subject to expiration, will begin to expire in 2037. Federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely but may only be used to offset 80% of our taxable income in future taxable years beginning after December 31, 2020. As of December 31, 2022, we also had approximately $10.0 million of federal orphan drug credits and research and development credits, or tax credits, which will begin to expire in 2037 if not utilized. The utilization of such NOLs and tax credits and realization of tax benefits in future years depends upon our having taxable income and income tax liabilities.

In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-ownership change NOLs and tax credits to offset future taxable income or income tax liabilities. For these purposes, an ownership change generally occurs where the aggregate change in stock ownership, of one or more stockholders or groups of stockholders owning at least 5% of a corporation's stock, exceeds 50 percentage points over a rolling three-year period. We may have experienced ownership changes in the past, and future changes in our stock ownership, many of which are outside of our control, could result in ownership changes in the future. Our state NOLs or tax credits may also be impaired under state law. Accordingly, even if we attain profitability, we may not be able to utilize a material portion of our NOLs or tax credits. We have recorded a full valuation allowance related to our NOLs and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

Risks Related to Our Intellectual Property

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

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our current and future approved products and product candidates, the processes used to manufacture them and the methods for using them, as well as successfully defending these patents against third-party challenges. We are the owner of record of patents and patent applications pending in the U.S. and in certain foreign jurisdictions. Patents issued from non-provisional applications, which are typically filed from provisional patent applications or from PCT applications that enter the national phase. Neither provisional patent applications nor PCT applications issue directly as patents. We own PCT patent applications relating to our platform technologies covering methods of use and applications of the platform technologies.

We cannot be certain that any future patents will issue with claims that cover our product candidates. Our ability to stop third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

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

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third-party will not have priority over patent applications filed or in-licensed by us, or that we or our

licensors will not be involved in interference, opposition, reexamination, review, reissue, post grant review or invalidity proceedings before U.S. or non-U.S. patent offices.

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

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

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

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

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

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

particular field or territory, in which case our ability to develop or commercialize products covered by the license agreement will be impaired.

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

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

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

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

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

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

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

Our product candidates have been or will be submitted to the FDA for approval under Section 505(b)(2) of the FDCA. Section 505(b)(2) permits the submission of an NDA where at least some of the information required for approval comes from studies that were not conducted by, or for, the applicant and on which the applicant has not obtained a right of reference. The 505(b)(2) application would enable us to reference published literature and/or the FDA's previous findings of safety and effectiveness for a branded reference drug with the same active ingredient. For NDAs submitted under Section 505(b)(2) of the FDCA, the patent certification and related provisions of the Hatch-Waxman Act apply. In accordance with the Hatch-Waxman Act, such NDAs may be required to include paragraph IV certifications, that certify that any patents listed in the FDA's Orange Book, with respect to any product referenced in the 505(b)(2) application, are invalid, unenforceable or will not be infringed by the manufacture, use or sale of the product that is the subject of the 505(b)(2) NDA.

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

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

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

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

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

which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform.

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

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

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

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

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

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

We also rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Furthermore, any license agreements we enter into in the future may require us to notify, and in some cases license back to the licensor, certain additional proprietary information or intellectual property that we developed using the rights licensed to us under these agreements. Any such licenses back to the licensor could allow our licensors to use that proprietary information or intellectual property in a manner that could harm our business. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its transparency initiative, is currently considering whether to make additional information publicly available on

a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

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

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

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

We may be subject to competition despite the existence of intellectual property we license or own. We can give no assurances that our intellectual property claims will be sufficient to prevent third parties from designing around patents we own or license and developing and commercializing competitive products. The existence of competitive products that avoid our intellectual property could materially adversely affect our operating results and financial condition.

Furthermore, limitations, or perceived limitations, in our intellectual property may limit the interest of third parties to partner, collaborate or otherwise transact with us, if third parties perceive a higher than acceptable risk to commercialization of our products or future products.

Our drug re-innovation approach involves the filing of patent applications covering new methods of use and/or new formulations of previously known, studied and/or marketed drugs. Although the protection afforded by our patent and patent applications may be significant with respect to BXCL501, BXCL502, BXCL701 and BXCL702, when looking at our patents' ability to block competition, the protection offered by our patents may be, to some extent, more limited than the protection provided by patents claiming the composition of matter of entirely new chemical structures previously unknown. If a competitor were able to successfully design around any method of use and formulation patents we may have in the future, our business and competitive advantage could be adversely affected.

We may elect to sue a third-party, or otherwise make a claim, alleging infringement or other violation of patents, trademarks, trade dress, copyrights, trade secrets, domain names or other intellectual property rights that we either own or license from BioXcel LLC. If we do not prevail in enforcing our intellectual property rights in this type of litigation, we may be subject to:

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

A third-party may also challenge the validity, enforceability or scope of the intellectual property rights that we license or own; and the result of these challenges may narrow the scope or claims of or invalidate patents that are integral to our product candidates in the future. There can be no assurance that we will be able to successfully defend

patents we own in an action against third parties due to the unpredictability of litigation and the high costs associated with intellectual property litigation, amongst other factors.

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

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

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

Risks Related to Owning our Common Stock

The price of our common stock may fluctuate substantially.

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

- sale of our common stock by our stockholders, executives, and directors;
- volatility and limitations in trading volumes of our shares of common stock;
- speculative trading in and short sales of our stock, as well as trading phenomena such as the “short squeeze” and “short and distort” schemes;
- 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.

Developments with respect to the recently completed TRANQUILITY II trial have subjected us to a number of additional risks and uncertainties, including regulatory, stockholder or other actions, loss of investor confidence and negative impacts on the trading price of our common stock.

In December 2022, the FDA conducted an inspection of one of the clinical trial sites in the Phase 3 TRANQUILITY II clinical trial, where the principal investigator enrolled approximately 40% of the subjects who

participated in the trial. At the conclusion of this inspection, the FDA issued an FDA Form 483 identifying three inspectional observations. These observations related to the principal investigator's failure to adhere to the informed consent form approved by the IRB for a limited number of subjects whose records the FDA reviewed, maintain adequate case histories for certain patients whose records the FDA reviewed, and adhere to the investigational plan in certain instances. In May 2023, it came to our attention that this same principal investigator in the TRANQUILITY II clinical trial may have fabricated email correspondence purporting to demonstrate that the investigator timely submitted to our pharmacovigilance safety vendor a report of an SAE from a different subject than the one cited in the FDA Form 483, and purporting to show that the vendor had confirmed receipt. Upon receipt of this information, we promptly initiated an investigation and subsequently received confirmation that the principal investigator fabricated the email correspondence related to the timing of the reporting of this SAE to our pharmacovigilance vendor to make it appear as though this SAE had been timely reported to the pharmacovigilance vendor as required by the clinical trial protocol. We are currently in the process of conducting an investigation into certain clinical trial and data integrity issues related to the TRANQUILITY II Phase 3 clinical trial and a number of independent third parties have been retained to conduct audits of the data from the trial site where misconduct occurred. These audits and the investigation may take a significant amount of time, may be costly, and may lead to the conclusion that there is a risk that FDA will not accept the TRANQUILITY II data in support of an sNDA. These developments have caused us to incur substantial expenses for legal, accounting, tax and other professional services and has diverted our management's attention from our business and could continue to do so. In addition, as a result of these developments, investors may lose confidence in our previous clinical trial results, current preclinical and clinical trial plans and our future projections, the price of our common stock may decline and we have been, and may be subject to future, shareholder or other litigation or regulatory enforcement actions in connection with the conduct of the TRANQUILITY II trial.

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 June 30, 2023, our directors, executive officers and BioXcel LLC, and their respective affiliates, beneficially owned approximately 36% of our outstanding shares of common stock. As a result, these stockholders, acting together, would have significant control over the outcome of matters submitted to our stockholders for approval, including the election of directors and any merger, consolidation or sale of all or substantially all of our assets. In addition, these stockholders, acting together, would have significant control over the management and affairs of our company. Accordingly, this concentration of ownership might harm the market price of our common stock by:

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

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

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

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

Under Sections 3(a)(1)(A) and (C) of the 1940 Act, a company generally will be deemed to be an "investment company" for purposes of the 1940 Act if (1) it is, or holds itself out as being, engaged primarily, or proposes to engage primarily, in the business of investing, reinvesting or trading in securities or (2) it engages, or proposes to engage, in the business of investing, reinvesting, owning, holding or trading in securities and it owns or proposes to acquire investment securities having a value exceeding 40% of the value of its total assets (exclusive of U.S. government

securities and cash items) on an unconsolidated basis. We do not believe that we are an "investment company," as such term is defined in 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 smaller reporting companies, which could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act") and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"), reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, Section 107 of the JOBS Act also provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are not electing to delay such adoption of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an "emerging growth company." We will remain and "emerging growth company" until December 31, 2023.

We are also a smaller reporting company, and we will remain a smaller reporting company until, as of fiscal year end, we determine that either (1) our annual revenues are at least $100 million and our voting and non-voting common stock held by non-affiliates is at least $250 million measured on the last business day of our most recent second fiscal quarter, or (2) our voting and non-voting common stock held by non-affiliates is at least $700 million measured on the last business day of our most recent second fiscal quarter. Similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosure, are exempt from the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide selected financial data, supplemental financial information or risk factors.

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

A securities class action has been filed against the Company, which could result in significant costs and/or liabilities; and, as a public Company, we continue to be at risk of securities class action litigation.

On July 7, 2023, plaintiff Katelyn Martin filed a class action complaint against the Company and certain executives in the United States District Court for the District of Connecticut, captioned *Martin v. BioXcel Therapeutics, et al.*, 3:23-cv-00915 (D. Conn). The complaints generally allege violations of Sections 10(b) and 20A of the Securities and Exchange Act of 1934 (the "Exchange Act") and SEC Rule 10b-5 promulgated thereunder, based on certain public statements related to the development of BXCL501, TRANQUILITY II and TRANQUILITY III between December

15, 2021 and June 28, 2023. Pursuant to the Private Securities Litigation Reform Act, other investors may seek to serve as lead plaintiff and pursue these claims on behalf of a putative class of investors. The allegations and claims at issue in matter may be amended or supplemented in the future, including when a lead plaintiff is appointed by the Court. The potential costs and liabilities associated with this litigation are uncertain. The above-captioned action may result in substantial costs or liabilities, as well as a diversion of management's attention and resources, which could harm our business and result in a decline in the market price of our common stock.

We may be at risk of costs and liabilities if further securities class actions or claims are filed against the Company. Biotechnology and pharmaceutical companies with publicly traded stock or who obtain funding through the stock market often experience significant stock price volatility, based on events beyond their control, including outcomes of clinical trials, actions of regulators and product approvals. Such further litigation, may result in substantial costs and a diversion of management's attention and resources, which could harm our business and result in a decline in the market price of our common stock.

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

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

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

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

As a publicly traded company we have incurred and will continue to incur significant legal, accounting and other expenses. The obligations of being a public company in the U.S. require significant expenditures and place significant demands on our management and other personnel, including costs resulting from public company reporting obligations under the Exchange Act and the rules and regulations regarding corporate governance practices, including those under the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, and the listing requirements of the stock exchange on which our securities are listed. These rules require the establishment and maintenance of effective disclosure and financial controls and procedures, internal control over financial reporting and changes in corporate governance practices, among many other complex rules that are often difficult to implement,

monitor and maintain compliance with. Moreover, despite reforms made possible by the JOBS Act, the reporting requirements, rules, and regulations will make some activities more time-consuming and costly, particularly after we are no longer an "emerging growth company" beginning December 31, 2023. In addition, we expect these and similar 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 such insurance. Our continued compliance with applicable requirements and to keep pace with new regulations requires management and other personnel to devote a substantial amount of their time, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems.

General Risk Factors

If securities or industry analysts do not publish research or reports, or publish unfavorable research or reports about our business, our stock price and trading volume may decline.

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

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

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

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

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

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

In 2017, the U.S. government enacted comprehensive federal income tax legislation that includes significant changes to the taxation of business entities. These changes include, among others, a permanent reduction to the corporate income tax rate. Notwithstanding the reduction in the corporate income tax rate, the overall impact of this tax reform is uncertain, and our business and financial condition could be adversely affected. Future changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of any foreign earnings, and the deductibility of expenses under future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Item 2. Unregistered Sales of Equity Securities, and Use of Proceeds and Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

(a) On August 8, 2023, the Board of Directors of the Company approved a broad-based strategic reprioritization (the "Reprioritization"). The Company has determined to take actions to reduce certain operational and workforce expenses that are no longer deemed core to the Company's ongoing operations in order to extend its cash runway and drive innovation and growth in high potential clinical development and value creating opportunities. These actions will include a shift in commercial strategy for IGALMI™ in the institutional setting, a reduction of in-hospital commercialization expenses, a suspension of programs no longer determined to be core to ongoing operations, and a prioritization on at-home treatment setting opportunities for BXCL501. As part of this strategy, the Company's Board of Directors approved a reduction of approximately 50% of the Company's current workforce. We began notifying impacted employees of the Reprioritization on August 14, 2023. Operating expenses are expected to be reduced by approximately $80 million per year, and the Reprioritization initiatives are expected to extend the Company's cash runway into mid-2024. The Reprioritization is expected to be complete by the end of the third quarter of 2023.

As a result of the Reprioritization, the Company estimates that it will incur approximately $7 million to $8 million in aggregate costs, consisting of severance and benefit payments, notice pay, and related expenses, all of which are expected to be paid in cash. The estimated costs that the Company expects to incur and the expected timing to complete the Reprioritization are subject to a number of assumptions, and actual results may differ. The Company may also incur other cash or non-cash charges or cash expenditures not currently contemplated due to events that may occur as a result of, or in association with, the Reprioritization.

(b) None

(c) 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	
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					*
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					*
32.1	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					**
32.2	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					**
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document					*
101.SCH	Inline XBRL Taxonomy Extension Schema Document					*
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document					*
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document					*
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document					*
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document					*
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).					*

* Filed herewith.

** Furnished herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

BioXcel Therapeutics, Inc.

Dated: August 14, 2023

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

Dated: August 14, 2023

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 June 30, 2023 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: August 14, 2023

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 June 30, 2023 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: August 14, 2023

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 June 30, 2023, 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: August 14, 2023

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 June 30, 2023, 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: August 14, 2023

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