BioXcel Therapeutics Reports Second Quarter 2023 Financial Results and Announces Strategic Reprioritization

August 14, 2023

Business to prioritize high-potential agitation market opportunities for BXCL501 in bipolar disorders, schizophrenia, and Alzheimer's disease

Commercial reprioritization intended to reduce related expenses by 80% with focus on market access through contracting with large hospital systems (IDNs)

Requested meeting with FDA to discuss TRANQUILITY II, audit plan, and data package to support potential submission of sNDA for BXCL501 in patients with agitation associated with mild to moderate dementia due to probable Alzheimer's disease

SERENITY III Phase 3 trial (part 2) protocol amendment in process to evaluate BXCL501 80 mcg dose for at-home use in bipolar disorder or schizophrenia agitation

Cash runway post-strategic reprioritization, expected through mid-2024; discussions underway with existing strategic finance partners

Company to host conference call at 8:00 a.m. ET today

NEW HAVEN, Conn., Aug. 14, 2023 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced its financial results for the second quarter ended June 30, 2023, and a strategic reprioritization to strengthen its focus and significantly reduce operating expenses.

Strategic Reprioritization and Clinical Development Initiatives
Following a comprehensive review of the business, the Company has determined to focus on high-potential agitation-market opportunities using its innovative, AI-based clinical drug development platforms. The Company intends to reduce more than 50% of its cash burn to approximately $80 million on a go-forward annualized basis. The Company will also be reducing its workforce from approximately 190 to 80 employees. These actions 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 deemed core to the Company's business, and a shift in focus to develop BXCL501 for use in the at-home setting in the treatment of agitation in schizophrenia, bipolar disorders, and in patients with mild to moderate dementia due to probable Alzheimer's disease.

"We are shifting our primary focus to development in the at-home setting while maintaining our value-creating core capabilities ranging from AI innovation to commercialization," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "Our AI-driven drug re-innovation approach has led to the capital-efficient development of product candidates in underserved therapeutic areas. We intend to prioritize our resources to develop BXCL501 for use in assisted living facility (ALF) and at-home settings and continue to advance our neuroscience pipeline. Unfortunately, this reprioritization requires us to reduce our workforce. We are grateful to all employees for their contributions and will support those who are impacted through their transitions."

Strengthened Focus on At-home Market Opportunities for BXCL501

TRANQUILITY Program: Alzheimer's Dementia Agitation

- **TRANQUILITY II**: Announced positive topline results in June 2023 from a Phase 3 trial that evaluated the safety and efficacy of BXCL501 for the acute treatment of agitation in mild to moderate dementia patients with probable Alzheimer's disease, who were 65 years and older living in ALFs and residential care settings and required minimal assistance with activities of daily living.
    - Trial met primary endpoint with the 60 mcg dose, with BXCL501 demonstrating a statistically significant 39% greater reduction in PEC score from baseline compared to placebo at 2 hours (p=0.0112).
    - Met a key secondary endpoint with statistically significant reduction (p=0.0185) in agitation symptoms versus placebo, as measured by PEC score change from baseline at 1 hour with 60 mcgdose.
    - 443 episodes for 149 patients were treated over 12 weeks across all doses; dosing with 60 mcg showed a similar reduction in agitation for first and all treated episodes at 1 and 2 hours, as measured by average change in PEC score.
    - An independent third party has been retained to initiate and perform a data integrity audit of the TRANQUILITY II clinical site where the principal investigator has engaged in misconduct.
    - Company has requested a meeting with FDA to discuss its TRANQUILITY program, including TRANQUILITY II, the data audit, TRANQUILITY III, and the data package that may be required to support submission of a supplemental New Drug Application (sNDA) seeking approval of BXCL501 for the acute treatment of agitation in mild to moderate dementia patients with probable Alzheimer's disease in the ALF and at-home setting.
    - Specifically, the Company plans to request feedback from FDA about the sufficiency of data from its TRANQUILITY I and II clinical trials, pharmacology, and toxicology package to support a potential sNDA submission seeking approval in the ALF and/or at-home settings. The Company has not conducted a clinical trial evaluating the

at-home use of BXCL501 in mild to moderate dementia patients with probable Alzheimer's disease. However, the TRANQUILITY II trial included both ALF and residential settings. The Company seeks feedback from the FDA as to whether any additional clinical studies may be required.
    - The Company also plans to discuss TRANQUILITY III trial insights with FDA and has elected to pause 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 thus far, the Company believes that continued evaluation of BXCL501 in this population would require a different development program targeting more frequent or chronic use. The Company has chosen to focus its 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.
    - For a further discussion of the results from and certain risks associated with the TRANQUILITY II trial, please see the Company's Form 8-K filed with the SEC on June 29, 2023 and its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2023.

SERENITY Program: Agitation Associated with Bipolar Disorders or Schizophrenia (At-Home Use)

- **SERENITY III Part 1 (completed)**: assessed the safety and efficacy of a 60 mcg dose of BXCL501 using the same primary and secondary endpoints as used in SERENITY I and II, which supported IGALMI's approval, with patients in a monitored medical setting used as surrogates for the at-home setting.
    - Reported topline data in May 2023; did not meet the primary endpoint of change in PEC score from baseline at 2 hours with half (60 mcg) of the approved dose of IGALMI, but clinically meaningful efficacy results were observed, including a statistically significant change in PEC score from baseline at 4 hours, a secondary endpoint in the trial.
    - BXCL501 was well tolerated and demonstrated favorable safety results supporting potential for at- home use.

- **SERENITY III Part 2 (ongoing):** 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 potential safety and efficacy for at-home use. The Company believes that evaluation of an 80 mcg dose is further supported by previous clinical experience with an 80 mcg BXCL501 dose in a Phase 1b study in schizophrenia patients with agitation. The Company believes the totality of evidence provides support for evaluating this dose in Part 2. Further, the Company plans to meet with FDA to discuss the 80 mcg dose and the elements of the proposed protocol amendment. 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, though the secondary endpoints for Part 2 include a number of efficacy assessments.

Adjunctive treatment in Major Depressive Disorder (MDD)

- Phase 1b Multiple Ascending Dose (MAD) trial tested safety and tolerability of daily dosing of BXCL501, including in combination with duloxetine, for seven days in healthy volunteers.
    - Company reported positive topline results in May.
    - 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.

Neuroscience Pipeline Development

- The Company is planning to host a neuroscience R&D Day in H2 2023, which will:
    - Highlight its neuroscience pipeline, including BXCL502
    - Showcase its next-generation AI-based drug discovery and development platform that has identified potential new product candidate concepts (BXCL503 and BXCL504).

IGALMI Commercialization

IGALMI continued to build on its momentum through the second quarter, climbing to over 185 formulary approvals and unlocking up to $80 million in market potential. There are more than 650 additional formulary reviews scheduled, representing up to an additional $275 million in bipolar and schizophrenia agitation market opportunity. Recent substantial orders through the contracting process resulted in the doubling of Q2 revenues sequentially, a process which the Company will emphasize moving forward.

- The commercial reprioritization is designed to 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 ordering IGALMI and those with positive formulary status through trade, distribution, and medical support.

OnkosXcel Therapeutics Immuno-oncology Subsidiary

- Continuing to actively evaluate strategic options for OnkosXcel Therapeutics, including potential financing or strategic partnership, M&A, or sale.

Second Quarter 2023 Financial Results

Net Revenue: Net revenue of IGALMI was approximately $457 thousand for the quarter.

Research and Development (R&D) Expenses: R&D expenses were $27.0 million for the second quarter of 2023, compared to $17.9 million for the same period in 2022. The increased expenses were primarily attributable to increased clinical trial expenses for SERENITY III and TRANQUILITY II.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $25.9 million for the second quarter of 2023, compared to $18.4 million for the same period in 2022. The increased expenses were primarily attributable to an increase in personnel and related costs to support the commercialization of IGALMI™.

Net Loss: BioXcel Therapeutics had a net loss of $53.5 million for the second quarter of 2023, compared to a net loss of $37.7 million for the same period in 2022. The loss for the quarter included approximately $6.1 million in non-cash stock-based compensation.

Cash and cash equivalents totaled $127.5 million as of June 30, 2023. As noted above, the Company is undertaking a strategic reprioritization, which includes a reduction in force of more than 50%, that is expected to reduce expenses significantly. In the absence of additional capital becoming available to the Company under the strategic financing agreements or otherwise, the Company estimates that its current cash and cash equivalents will last through mid-2024.

The Company's previously disclosed cash runway projection assumed the full utilization of its strategic financing agreements ($155 million of potential additional availability) with Oaktree Fund Administration LLC and Qatar Investment Authority. Based on recent events, the Company is not likely to be in a position to meet the milestones required to access additional capital under the financing agreements. The Company has initiated discussions with its strategic financing partners to amend the agreements. Successful modification of these agreements could further extend the Company's cash runway.

Conference Call
BioXcel Therapeutics will host a conference call and webcast on August 14, 2023 at 8:00 a.m. ET to discuss its second quarter 2023 financial results and provide an update on recent operational highlights. To access the call, please dial 877-407-5795 (domestic) and 201-689-8722 (international). A live webcast will be available on the Investors section of the corporate website, bioxceltherapeutics.com, and a replay will be available through November 14, 2023.

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

About IGALMI™(dexmedetomidine) sublingual film

INDICATION

IGALMI™ (dexmedetomidine) sublingual film is a prescription medicine, administered under the supervision of a health care provider, that is placed under the tongue or behind the lower lip and is used for the acute treatment of agitation associated with schizophrenia and bipolar disorder I or II in adults. The safety and effectiveness of IGALMI has not been studied beyond 24 hours from the first dose. It is not known if IGALMI is safe and effective in children.

IMPORTANT SAFETY INFORMATION

IGALMI can cause serious side effects, including:

- **Decreased blood pressure, low blood pressure upon standing, and slower than normal heart rate, which may be more likely in patients with low** blood volume, diabetes, chronic high blood pressure, and older patients. IGALMI is taken under the supervision of a healthcare provider who will monitor vital signs (like blood pressure and heart rate) and alertness after IGALMI is administered to help prevent falling or fainting. Patients should be adequately hydrated and sit or lie down after taking IGALMI and instructed to tell their healthcare provider if they feel dizzy, lightheaded, or faint.
- **Heart rhythm changes (QT interval prolongation)**. IGALMI should not be given to patients with an abnormal heart rhythm, a history of an irregular heartbeat, slow heart rate, low potassium, low magnesium, or taking other drugs that could affect heart rhythm. Taking IGALMI with a history of abnormal heart rhythm can increase the risk of torsades de pointes and sudden death. Patients should be instructed to tell their healthcare provider immediately if they feel faint or have heart palpitations.
- **Sleepiness/drowsiness**. Patients should not perform activities requiring mental alertness, such as driving or operating hazardous machinery, for at least 8 hours after taking IGALMI.
- **Withdrawal reactions, tolerance, and decreased response/efficacy**. IGALMI was not studied for longer than 24 hours after the first dose. Physical dependence, withdrawal symptoms (e.g., nausea, vomiting, agitation), and decreased

response to IGALMI may occur if IGALMI is used longer than 24 hours.

The most common side effects of IGALMI in clinical studies were sleepiness or drowsiness, a prickling or tingling sensation or numbness of the mouth, dizziness, dry mouth, low blood pressure, and low blood pressure upon standing.

These are not all the possible side effects of IGALMI. Patients should speak with their healthcare provider for medical advice about side effects.

Patients should tell their healthcare provider about their medical history, including if they suffer from any known heart problems, low potassium, low magnesium, low blood pressure, low heart rate, diabetes, high blood pressure, history of fainting, or liver impairment. They should also tell their healthcare provider if they are pregnant or breastfeeding or take any medicines, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Patients should especially tell their healthcare provider if they take any drugs that lower blood pressure, change heart rate, or take anesthetics, sedatives, hypnotics, and opioids.

Everyone is encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You can also contact BioXcel Therapeutics, Inc. at 1-833-201- 1088 or medinfo@bioxceltherapeutics.com.

Please see full Prescribing Information at igalmi.com.

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. is a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. The Company's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indications. The Company's commercial product, IGALMI™ (developed as BXCL501), is a proprietary, sublingual film formulation of dexmedetomidine approved for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. The safety and effectiveness of IGALMI have not been established beyond 24 hours from the first dose. For more information, please visit igalmi.com and also see the IGALMI full Prescribing Information. BXCL501 is under evaluation for at-home use for the acute treatment of agitation in bipolar and schizophrenia patients, for acute treatment of agitation associated with Alzheimer's disease, and as an adjunctive treatment for major depressive disorder. The safety and efficacy of BXCL501 for these uses have not been established. The Company is also developing BXCL502 as a potential therapy for chronic agitation in dementia. Under its subsidiary, OnkosXcel Therapeutics, the Company is developing BXCL701, an investigational, oral systemic innate immune activator for the treatment of aggressive forms of prostate cancer and other solid and liquid tumors. The safety and efficacy of BXCL502 and BXCL701 have not been established. For more information, please visit bioxceltherapeutics.com.

Forward-Looking Statements
This press release includes "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"). All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the Company's expected timing of, and data results from, trials and clinical studies involving its product candidates; the potential for the results from the Company's completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates, including the results from the TRANQUILITY II and SERENITY III clinical trials; its ongoing marketing, commercialization and expansion efforts, plan and strategy for IGALMI; statements regarding the Company's strategic reprioritization; anticipated cost savings; expected cash runway and cash burn rates; planned discussions with the FDA and paths to potential FDA approval of BXCL501; strategic options for OnkosXcel; the Company's participation in upcoming events and presentations; potential modifications to the terms of the Company's financing arrangements with Oaktree/QIA; future access to capital; and the Company's future financial and operational results, including future revenue growth. 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. In addition, any statements or information that refer to expectations, beliefs, plans, projections, objectives, performance or other characterizations of future events or circumstances, including any underlying assumptions, are forward- looking. All forward-looking statements are based upon the Company's current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. The Company may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation, its limited operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; its ability to successfully negotiate amended terms under the financing agreements to be able to access funding and to obtain relief under financial covenants; its significant indebtedness and potential payment obligations related to such indebtedness and other contractual obligations; risks associated with the strategic reprioritization; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY II Phase 3 trial and related audit; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502 BXCL701 and BXCL702 and other product candidates; its lack of experience in marketing and selling drug products; the risk that IGALMI or the Company's product candidates may not be accepted by physicians or the medical community in general; the failure of preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; undesirable side effects caused by the Company's product candidates; its novel approach to the discovery and development of product candidates based on EvolverAI; the significant influence of and dependence on BioXcel LLC; its exposure to patent infringement lawsuits; its reliance on third parties; its ability to comply with the extensive regulations applicable to it; impacts from data breaches or cyber-attacks, if any; impacts from the COVID-19 pandemic; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; its ability to commercialize its product candidates; and the other important factors discussed under the caption "Risk Factors" in its Annual Report on Form 10-K for the fiscal year ended December 31, 2022, as such factors may be updated from time to time in its other filings with the SEC, including without limitation, its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2023, which are accessible on the SEC's website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While the Company may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this press release.

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Source: BioXcel Therapeutics, Inc.

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BioXcel Therapeutics, Inc.
Statements of operations
(in thousands, except per share amounts)

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Revenues	$ 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
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)
Net loss per share - basic and diluted	$ (1.83)	$ (1.35)	$ (3.68)	$ (2.47)
Weighted average shares outstanding - basic and diluted	29,187	27,989	28,903	27,985

Condensed Balance Sheets
(in thousands)

	June 30, 2023	December 31, 2022
Cash and cash equivalents	$ 127,545	$ 193,725
Working capital	$ 103,491	$ 169,970
Total assets	$ 140,097	$ 205,853
Long-term liabilities	$ 99,489	$ 96,180
Total liabilities	$ 134,233	$ 129,078
Total stockholders' equity	$ 5,864	$ 76,775