BioXcel Therapeutics Reports Second Quarter 2024 Financial Results

August 6, 2024

Planning initiation of SERENITY At-Home pivotal Phase 3 trial of BXCL501 for acute treatment of agitation associated with bipolar disorders or schizophrenia

Advancing plans for TRANQUILITY In-Care pivotal Phase 3 trial with BXCL501 for agitation associated with Alzheimer's dementia

Reported positive topline results from IGALMI™ post-marketing requirement (PMR) study

Conference call set for 8:00 a.m. ET today

NEW HAVEN, Conn., Aug. 06, 2024 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology, today announced its financial results for the second quarter of 2024.

"We are on track with our business priorities as we focus on bringing BXCL501 to the greatest number of patients in need," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "We are pleased with the progress with our SERENITY and TRANQUILITY programs and our focused market-access strategy for IGALMI™. Our confidence in our lead neuroscience asset is underpinned by its broad therapeutic potential across multiple neuropsychiatric conditions and its growing intellectual property portfolio."

Late-Stage Clinical Programs

- SERENITY At-Home* Pivotal Phase 3 Trial: designed to evaluate the safety of a 120 mcg dose of BXCL501 in the at-home setting for agitation associated with bipolar disorders or schizophrenia.
  - Recently received feedback on protocol from U.S. Food and Drug Administration (FDA).
- TRANQUILITY In-Care Pivotal Phase 3 Trial: designed to evaluate the efficacy and safety of a 60 mcg dose of BXCL501 for agitation associated with Alzheimer's dementia (AAD).
  - Protocol being finalized for planned submission to FDA.

IGALMI™ (dexmedetomidine) Sublingual film

Post-marketing Requirement (PMR) Study

- Reported positive topline results from PMR study evaluating PRN (as-needed) treatment of IGALMI™ for agitation associated with bipolar disorders or schizophrenia.
  - Study achieved its objective and demonstrated no evidence of tachyphylaxis, tolerance, or withdrawal with 180 mcg dose (highest approved dose).
  - Although this PMR study was not statistically powered to evaluate repeat dose efficacy, a reduction in agitation was observed for each episode occurring during the seven-day study period, and no serious adverse events were reported following treatment.

Commercialization

- IGALMI™ net revenue grew 90% in Q2 2024 over Q1 2024 driven by focused market-access strategy and increased contracting with psychiatric care clinics and behavioral health facilities using a small commercial team.

Patent Portfolio

The Company continues to strengthen its intellectual property portfolio for IGALMI™.

- Recently received a U.S. Patent and Trademark Office (USPTO) Notice of Allowance for U.S. Patent Application No. 18/526,686 for IGALMI™. Once issued by the USPTO, the patent is expected to have an expiration date of January 12, 2043, and will be submitted for listing in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (commonly known as the "Orange Book").
- This is expected to be the 11th listed U.S. patent for IGALMI™ in the Orange Book.

OnkosXcel Therapeutics

- Late-breaking abstract on preliminary findings from a Phase 2 investigator-sponsored trial of BXCL701 and KEYTRUDA® (pembrolizumab) in metastatic pancreatic ductal adenocarcinoma (PDAC) presented at 2024 ASCO Annual Meeting by Dr. Benjamin Weinberg, Principal Investigator, Georgetown University Lombardi Comprehensive Cancer Center.

Second Quarter 2024 Financial Results

Net Revenue: Net revenue from IGALMI was $1.1 million for the second quarter of 2024, compared to $457 thousand for the same period in 2023, representing a 141% increase. Sequential quarterly revenue increased 90% in Q2 2024 from Q1 2024. The increased revenue for both periods was primarily driven by an increase in contracting with psychiatric care clinics and behavioral health facilities.

Research and Development (R&D) Expenses: R&D expenses were $8.0 million for the second quarter of 2024, compared to $27.0 million for the same period in 2023. The decreased expenses were primarily attributable to the wind-down of the SERENITY III and TRANQUILITY II and III trials, as well as decreased professional fees, personnel, and related costs.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $9.5 million for the second quarter of 2024, compared to $25.9 million for the same period in 2023. The reduced expenses were primarily attributable to a decrease in personnel and costs associated with the commercialization of IGALMI compared to the second quarter of 2023, driven by the Company's strategic reprioritization announced in August 2023.

Net Loss: BioXcel Therapeutics had a net loss of $8.3 million for the second quarter of 2024**, compared to a net loss of $53.5 million for the same period in 2023. The Company used $23.2 million in operating cash during the second quarter of 2024.

Cash and cash equivalents totaled $56.3 million as of June 30, 2024.

**SERENITY At-Home represents the redesigned SERENITY III trial.*

***In the second quarter of 2024, the loss from operations of $17.3 million was offset by unrealized gains related to derivative liabilities.*

Conference Call and Webcast
BioXcel Therapeutics will host a conference call and webcast today, August 6, 2024, at 8:00 a.m. ET to discuss its second quarter 2024 financial results. To access the call, please dial 877-407-5795 or +1 201-689-8722. A live webcast will be available on the Investors section of the corporate website, bioxceltherapeutics.com and a replay will be available for 90 days.

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 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.

About BXCL501
Outside of its approved indication by the U.S. Food and Drug Administration as IGALMI™ (dexmedetomidine) sublingual film, BXCL501 is an investigational proprietary, orally dissolving film formulation of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist. BXCL501 is under investigation by BioXcel Therapeutics for the acute treatment of agitation associated with Alzheimer's dementia and for the acute treatment of agitation associated with bipolar I or II disorder or schizophrenia in the at-home setting. The safety and efficacy of BXCL501 for these investigational uses have not been established. BXCL501 has been granted Breakthrough Therapy designation by the FDA for the acute treatment of agitation associated with dementia and Fast Track designation for the acute treatment of agitation associated with schizophrenia, bipolar disorders, and dementia.

About BXCL701
BXCL701 is an investigational, oral innate immune activator designed to initiate inflammation in the tumor microenvironment. Approved and experimental immunotherapies often fail to address cancers that appear "cold." Therefore, BXCL701 is being evaluated to determine if it can render "cold" tumors "hot," making them more detectable by the adaptive immune system and thereby facilitating the development of a strong anticancer immune response. OnkosXcel Therapeutics' preclinical data support BXCL701's potential synergy with both current checkpoint inhibitors and emerging immunotherapies directed to activate T-cells. BXCL701 is a potential therapy for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. BXCL701 has received Orphan Drug Designation from the FDA in four indications: acute myelogenous leukemia, pancreatic cancer, stage IIb to IV melanoma, and soft tissue sarcoma. The FDA designated as a Fast Track development program the investigation of BXCL701 in combination with a checkpoint inhibitor for treatment of patients with metastatic small cell neuroendocrine prostate cancer (SCNC) with progression on chemotherapy and no evidence of microsatellite instability. An 800+-subject clinical database, with data collected by the Company and others, supports the ongoing development of BXCL701.

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. (Nasdaq: BTAI) is a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience. Its wholly owned subsidiary, OnkosXcel Therapeutics, is focused on the development of medicines in 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. 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 and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements related to: the Company's planned advancement of its TRANQUILITY and SERENITY trials and the trial designs thereof; potential market opportunity for BXCL501; the potential for the results from the Company's completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates; its ongoing commercial strategy for IGALMI; the Company's current patent applications and potential Orange Book submissions. When used herein, words including "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; the impact of the reprioritization; its significant indebtedness, ability to comply with covenant obligations and potential payment obligations related to such indebtedness and other contractual obligations; the Company has identified conditions and events that raise substantial doubt about its ability to continue as a going concern; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY program; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502, BXCL701 and BXCL702 and other product candidates; the number of episodes of agitation and the size of the Company's total addressable market may be overestimated, and approval that the Company may obtain may be based on a narrower definition of the patient population; 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 Company still faces extensive and ongoing regulatory requirements and obligations for IGALMI; 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; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; risks associated with federal, state or foreign health care "fraud and abuse" laws; and its ability to commercialize its product candidates, as well as the important factors discussed under the caption "Risk Factors" in its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2024, as such factors may be updated from time to time in its other filings with the SEC, which are accessible on the SEC's website at www.sec.gov and the Investors section of the Company's website at www.bioxceltherapeutics.com. 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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Statements of Operations
(Unaudited, in thousands, except per share amounts)

	Three months ended June 30,		Six months ended June 30,	
	2024	2023	2024	2023
Revenues				
Product revenues	$ 1,104	$ 457	$ 1,686	$ 663
Operating expenses				
Cost of goods sold	62	26	141	34
Research and development	8,032	26,973	19,433	54,773
Selling, general and administrative	9,450	25,872	22,715	49,467
Restructuring costs	856	-	856	-
Total operating expenses	18,400	52,871	43,145	104,274
Loss from operations	(17,296)	(52,414)	(41,459)	(103,611)
Other (income) expense				
Interest expense, net	3,700	3,259	7,307	6,627
Interest income	(671)	(1,621)	(1,618)	(3,636)
Other (income) expense, net	(12,026)	(537)	(12,058)	(291)
Net loss and comprehensive loss	$ (8,299)	$ (53,515)	$ (35,090)	$ (106,311)
Net loss per share - basic and diluted	$ (0.21)	$ (1.83)	$ (0.99)	$ (3.68)
Weighted average shares outstanding - basic and diluted	40,253	29,187	35,560	28,903

Condensed Balance Sheets
(Unaudited, in thousands)

	June 30, 2024	December 31, 2023
Cash and cash equivalents	$ 56,271	$ 65,221
Total assets	$ 65,435	$ 73,702
Total liabilities	$ 139,736	$ 130,210
Total stockholders' equity (deficit)	$ (74,301)	$ (56,508)