BioXcel Therapeutics Reports Financial Results for the Fourth Quarter and Full Year 2024

March 27, 2025

Enrollment exceeds 60% of required number of patients in SERENITY At-Home pivotal Phase 3 safety trial evaluating 200 patients for acute treatment of agitation associated with bipolar disorders or schizophrenia

Topline data expected in second half of 2025 intended to support potential sNDA submission for label expansion of IGALMI® in the at-home setting

Strengthened cash position to advance BXCL501 program

NEW HAVEN, Conn., March 27, 2025 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience, today reported its financial results for the fourth quarter and full year 2024.

"We believe that our SERENITY program presents an exciting opportunity to address a substantial unmet medical need — the 23 million episodes of bipolar and schizophrenia-related agitation that occur annually in the United States at home[1-3] — and expand the market potential for our lead neuroscience asset BXCL501," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "We are pleased that patient enrollment in our SERENITY At-Home pivotal Phase 3 trial is progressing well and that we have recently strengthened our cash position to further advance this important study."

BXCL501 Late-Stage Clinical Programs

SERENITY Program

- **SERENITY At-Home Phase 3 Trial**: designed as a double-blind, placebo-controlled study to evaluate the safety of a 120 mcg dose of BXCL501 in 200 patients for acute treatment of agitation associated with bipolar disorders or schizophrenia in the at-home setting.
  - 24 clinical trial sites have been opened.
  - 127 patients have been enrolled, representing 63% of the required enrollment.
  - A Data Safety Monitoring Board (DSMB) is planned to assess safety.
  - Topline data expected in the second half of 2025.

TRANQUILITY Program

- **TRANQUILITY In-Care Phase 3 Trial:** designed as a double-blind, placebo-controlled study to evaluate the efficacy and safety of a 60 mcg dose of BXCL501 for acute treatment of agitation associated with Alzheimer's dementia in the care setting.
  - Developed plans following receipt of FDA feedback on trial protocol.
- **TRANQUILITY II Phase 3 Trial Site Inspection Closed by FDA**
  - The Company believes the reliability of data from its TRANQUILITY II Phase 3 trial of BXCL501 is further supported by the FDA closure of its site inspection under 21 C.F.R.20.64(d)(3) and release of the Establishment Inspection Report designating "Voluntary Action Indicated" for the site.

IGALMI® Market Presence

- The Company is continuing to supply IGALMI® (dexmedetomidine) sublingual film to current and future patients and providers through existing distribution channels, with minimal commercial support.

Equity Financing

- Closed $14 million equity financing and strengthened cash position to approximately $35 million, as of March 4, 2025, to advance SERENITY program.

Fourth Quarter and Full Year 2024 Financial Results

Net revenue from IGALMI® was $366,000 for the fourth quarter of 2024, compared to $376,000 for the same period in 2023.

Net revenue from IGALMI® was $2.3 million for the full year of 2024, compared to $1.4 million for 2023.

Cost of Goods Sold for the three months ended December 31, 2024 and 2023, were $832,000 and $714,000, respectively. Cost of goods sold is

related to the costs to produce, package, and deliver IGALMI to customers, as well as costs related to excess or obsolete inventory. The increase in Cost of goods sold for the three months ended December 31, 2024 is the result of higher charges for reserves for excess or obsolete inventory compared to the same period in 2023. Charges for reserves for excess or obsolete inventory were $778,000 and $696,000 in the three months ended December 31, 2024 and 2023, respectively.

Cost of Goods Sold was $2.1 million for the full year of 2024, compared to $1.3 million for 2023. The increase in Cost of goods sold is the result of higher charges for reserves for excess or obsolete inventory in 2024 compared to 2023. Charges for reserves for excess or obsolete inventory were $2.0 million and $1.2 million for 2024 and 2023, respectively.

Research and Development (R&D) expenses were $5.9 million for the fourth quarter of 2024, compared to $9.9 million for the same period in 2023.

R&D expenses were $30.4 million for the full year of 2024, compared to $84.3 million for the full year of 2023. The decreased expenses for both the fourth quarter and the full year were primarily attributable to a decrease in clinical trial activity associated with previously completed Phase 3 studies, a decrease in chemical, manufacturing, and control (CMC) costs, and a decrease in personnel related to the company's reprioritization.

Selling, General and Administrative (SG&A) expenses were $4.1 million for the fourth quarter of 2024, compared to $9.6 million for the same period in 2023.

SG&A expenses were $34.5 million for the full year 2024, compared to $83.4 million for 2023. The decreased costs for the fourth quarter and the full year 2024 were primarily attributable to a decrease in personnel and related costs, lower non-cash stock compensation costs, lower legal and professional fees, and lower commercial and marketing costs resulting from restructuring actions taken in 2024 and 2023.

Net Loss: BioXcel Therapeutics had a net loss of $10.9 million for the fourth quarter of 2024, compared to a net loss of $22.3 million for the same period in 2023. For the full year of 2024, BioXcel Therapeutics reported a net loss of $59.6 million, compared to a net loss of $179.1 million for the full year of 2023. The loss for the 2024 year includes approximately $6.2 million in non-cash stock-based compensation. Total cash used in operating activities for 2024 totaled approximately $72.0 million, down $83.0 million from 2023 cash used in operating activities of approximately $155.0 million.

Cash and cash equivalents totaled $29.9 million on December 31, 2024, compared to $65.2 million on December 31, 2023.

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 the SERENITY At-Home Phase 3 Trial

The SERENITY At-Home Phase 3 trial is a double-blind, placebo-controlled study designed to evaluate the safety of a 120 mcg dose of BXCL501 for the acute treatment of agitation associated with bipolar disorders or schizophrenia in the at-home setting. The trial is evaluating 200 patients with a history of agitation episodes residing at home either alone or with caregivers/informants. Patients will self-administer 120 mcg of BXCL501 or placebo when agitation episodes occur over the 12-week trial period, with safety data (adverse events) collected during the trial. Patients or caregivers/informants will complete a modified global impression of severity (mCGIs) and a clinical global impression of change (mCGI-C) two hours after dosing as an exploratory endpoint to evaluate use in the outpatient setting.

About the TRANQUILITY In-Care Phase 3 Trial

The TRANQUILITY In-Care trial is designed as a double-blind, placebo-controlled study to evaluate the efficacy and safety of a 60 mcg dose of BXCL501 over a 12-week period for agitation associated with Alzheimer's dementia in the care setting. The trial is expected to enroll approximately 150 patients 55 years and older who have mild, moderate, or severe dementia with mini-mental state examination scores of 0 to 25 and who reside in skilled nursing facilities, memory care units, or assisted living facilities. Patients will self-administer 60 mcg of BXCL501 or placebo when episodic agitation episodes occur over the trial period. The primary endpoint is expected to be a change from baseline in the Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score at two hours post-first dose. Additional PEC and Clinical Global Impressions – Improvement Scale measurements will also be obtained during the trial.

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. (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 DSMB meeting for the ongoing SERENITY trial; the supply of IGALMI through existing distribution channels; the potential for the results from the Company's completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates; the Company's current patent applications and potential Orange Book listings. 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 September 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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Source: BioXcel Therapeutics, Inc.
IGALMI® is a registered trademark of BioXcel Therapeutics, Inc.

References

1. Data on file relating to agitation episodes associated with schizophrenia or bipolar I or II disorder. BioXcel Therapeutics, Inc. New Haven, CT December 2020. Episode estimations may not reflect potential treatable episodes, and actual addressable market may be smaller.
2. Data from Wu EQ, Shi L, Birnbaum H, et al. Annual prevalence of diagnosed schizophrenia in the USA: a claims data analysis approach. Psychol Med. 2006;36(11):1535-1540. Estimates based on whether indications are approved for at-home use for the intended patient population and such patients are treatable. Episode estimations may not reflect potential treatable episodes, and actual addressable market may be smaller.
3. National Institute of Mental Health. Prevalence of bipolar disorder in adults. November 2017. Accessed December 16, 2022. https://www.nimh.nih.gov/health/statistics/bipolar-disorder. Episode estimations may not reflect potential treatable episodes, and actual addressable market may be smaller.

BioXcel Therapeutics, Inc.

Statements of Operations
(Unaudited, in thousands, except per share amounts)

	Three months ended December 31,		For the year ended December 31,	
	2024	2023	2024	2023
Revenues				
Product revenues	$ 366	$ 376	$ 2,266	$ 1,380
Operating expenses				
Cost of goods sold	832	714	2,143	1,260
Research and development	5,901	9,934	30,435	84,326
Selling, general and administrative	4,094	9,603	34,492	83,413
Restructuring costs	32	-	2,441	4,163
Total operating expenses	10,859	20,251	69,511	173,162
Loss from operations	(10,493)	(19,875)	(67,245)	(171,782)
Other (income) expense				
Interest expense, net	4,032	3,435	15,129	13,314
Interest income	(368)	(946)	(2,602)	(5,649)
Other (income) expense, net	(3,298)	(108)	(20,173)	(394)
Net loss and comprehensive loss	$ (10,859)	$ (22,256)	$ (59,599)	$ (179,053)
Net loss per share - basic and diluted	$ (3.57)	$ (12.09)	$ (23.51)	$ (98.33)
Weighted average shares outstanding - basic and diluted	3,039	1,841	2,535	1,821

Condensed Balance Sheets
(Unaudited, in thousands)

	December 31, 2024	December 31, 2023

Cash and cash equivalents	$	29,854	$	65,221
Total assets	$	38,338	$	73,702
Total liabilities	$	131,439	$	130,210
Total stockholders' equity (deficit)	$	(93,101)	$	(56,508)