BioXcel Therapeutics Reports First Quarter 2023 Financial Results and Recent Operational Highlights

May 8, 2023

IGALMI™ (dexmedetomidine) commercial momentum accelerating with doubling of formulary wins, unlocking more than $55 million in targeted market opportunity, and an additional $255 million scheduled to vote

Top-line data from pivotal SERENITY III Phase 3 trial (Part 1) for BXCL501 in bipolar or schizophrenia-associated agitation for at-home use expected in May 2023

Top-line data from repeat dosing of BXCL501 in Phase 1b trial for Major Depressive Disorder program in healthy volunteers expected in May 2023

Top-line data from pivotal TRANQUILITY II Phase 3 trial for BXCL501 for acute treatment of agitation associated with Alzheimer's disease expected in June 2023

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

NEW HAVEN, Conn., May 08, 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 first quarter ended March 31, 2023, and provided an update on key strategic initiatives.

"The first quarter marked a strong start to the year with numerous advancements in our clinical programs and continued commercial focus building the agitation market for our new therapeutic option in a historically underdiagnosed and underserved medical condition," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "We are gearing up to announce top-line data readouts in agitation from two Phase 3 pivotal trials as well as BXCL501 potential as an adjunctive treatment for chronic use in our MDD program. In addition, IGALMI's launch momentum is expanding our reach into addressable market opportunities. We believe the second quarter of 2023 represents a defining moment for the Company as we expand the full potential of BXCL501 in agitation for at-home use and long-term care settings, and in depression. These upcoming catalysts may have a transformational impact for patients in need and all our stakeholders."

Company Highlights

Neuroscience Franchise
IGALMI™ (dexmedetomidine) sublingual film
IGALMI is approved by the U.S. Food and Drug Administration (FDA) for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults.[1] *Up to an estimated 16 million institutional episodes occur annually within these two patient populations in the U.S.*[2-4*]

Key Commercial Parameters

- IGALMI launch is accelerating with continued deployment of integrated commercial team in 2023:
    - Secured more than 130 hospital formulary wins to date, which have doubled in the last two months; equates to $55 million of addressable market with Integrated Delivery Network (IDN) formulary approvals.
    - Six hundred additional Pharmacy & Therapeutics (P&T) Committee votes scheduled and approximately 25% of target IDN beds representing an additional $255 million of addressable market scheduled to vote.
    - More than half of all ordering hospitals have reordered; repeat orders demonstrate real-world utility and growing health care provider (HCP) interest.
    - Sales team has reached over 75% of 1,700 targeted hospitals with focus on deepening advocacy and driving demand.
    - Group Purchasing Organization (GPO) process largely complete with nearly 80% of targeted beds under contract.
    - Amplifying IGALMI awareness through multi-channel approach including digital and print media, peer influence initiatives, and Free Trial Program.

Medical Affairs

- Medical Science Liaison and Medical Managed Care teams engaging in scientific dialogue with HCPs ahead of IDN and hospital formulary reviews.
    - Observed a 73% increase in medical community requests for IGALMI clinical information over prior quarter.
    - Conducted a real-world survey of early-adopter HCPs; results expected to be presented at the American Society of

Health-System Pharmacists Annual Meeting in June 2023.
- Continuing extensive medical education initiatives through multiple clinical and scientific publications and presentations.

Development Pipeline

BXCL501, an investigational proprietary, sublingual film formulation of dexmedetomidine, has received Breakthrough Therapy and Fast Track designation for the acute treatment of agitation associated with dementia.

- **Alzheimer's Disease-related Agitation:** TRANQUILITY program is designed to evaluate BXCL501 for the acute treatment of Alzheimer's-related agitation; up to 100 million Alzheimer's-related agitation episodes are estimated to occur in the U.S. annually.[2*]
  - TRANQUILITY II: Trial is fully enrolled, and all patients have completed the study in assisted living facilities (ALFs) and residential care settings.
    - Data cleaning and verification in progress.
    - Top-line data from pivotal trial expected in June 2023.
  - TRANQUILITY III: Continuing enrollment of patients with moderate to severe dementia in long-term-care facilities.

- **Bipolar or Schizophrenia-related Agitation (At-Home Use)**: SERENITY III program is designed to evaluate BXCL501 for at-home use, where up to 23 million bipolar or schizophrenia-related agitation episodes are estimated to occur in the U.S. annually.[2-4*]

SERENITY III consists of two parts:

- Part 1: Assessing the efficacy and safety of 60 mcg dose in acute treatment of agitated patients with bipolar I or II disorder or schizophrenia.
  - Data cleaning and verification in progress.
  - Top-line efficacy data from pivotal trial expected in May 2023.
- Part 2: Evaluating the safety of 60 mcg dose, and a second 60 mcg dose is allowed if required, at-home.
  - Expect to initiate Part 2 in Q2 2023.

- **Adjunctive Treatment for Major Depressive Disorder (MDD) for At-Home Use:** Phase 1b Multiple Ascending Dose (MAD) trial was designed to test safety and tolerability of daily dosing of BXCL501 for seven days in healthy volunteers to inform proof-of-concept (POC) trial dose selection. In Phase 2 program, treatment in MDD patients will be evaluated with BXCL501 in combination with selective serotonin- or serotonin-norepinephrine reuptake inhibitors (SSRIs or SNRIs, respectively) to assess rapid antidepressant response. Over 300 million antidepressant prescriptions are filled annually in the U.S.,[5*] and current treatments are limited by slow onset of action and incomplete responses.

  - Data cleaning and verification in progress.
  - Top-line results are expected in May 2023.

OnkosXcel Therapeutics

OnkosXcel Therapeutics is a subsidiary of BioXcel Therapeutics focused on the sustained growth of the Company's immuno-oncology (I-O) franchise, including BXCL701, its most advanced I-O program. BXCL701 is an investigational, oral innate immune activator in development for the treatment of aggressive forms of prostate cancer and other solid and liquid tumors.

- Continuing to actively evaluate strategic options for OnkosXcel Therapeutics:

  - **Small Cell Neuroendocrine Prostate Cancer (SCNC) Program:** In 2023, it is estimated there will be 288,300[6] new prostate cancer patients in the U.S., with approximately 11,500 patients progressing to SCNC.[7]

    - Planned Phase 2b potential pivotal study for BXCL701 monotherapy and in combination with KEYTRUDA® (pembrolizumab) in SCNC expected to initiate in 2H 2023, subject to further discussions with FDA.
  - **Predictive Biomarker for BXCL701:** Additional findings on DPP9 overexpression, a potential actionable biomarker for BXCL701 response, expected to be presented at an upcoming medical meeting.
  - **Investigator-Sponsored Trials at Top Academic Centers**: BXCL701 to be studied in combination with KEYTRUDA® (pembrolizumab) at Georgetown Lombardi Cancer Center and Dana-Farber Cancer Institute in pancreatic cancer and AML, respectively.

First Quarter 2023 Financial Results

Net Revenue: Net revenue was approximately $206,000 for the quarter, in line with the fourth quarter. The Company expects to see a notable uptick in revenues in the second half of the year in connection with additional formulary approvals.

Research and Development (R&D) Expenses: R&D expenses were $27.8 million for the first quarter of 2023, compared to $18.6 million for the

same period in 2022. The increased expenses were primarily attributable to multiple clinical trials and CMC costs related to the upcoming three data readouts.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $23.6 million for the first quarter of 2023, compared to $12.9 million for the same period in 2022. The increased expenses were primarily attributable to personnel and sales, market access, and marketing costs associated with the commercialization of IGALMI in the U.S.

Net Loss: BioXcel Therapeutics had a net loss of $52.8 million for the first quarter of 2023, compared to a net loss of $31.5 million for the same period in 2022.

Cash and cash equivalents totaled $165.5 million as of March 31, 2023. BioXcel Therapeutics believes that full execution of its strategic financing with Oaktree and Qatar Investment Authority, and IGALMI revenues, would result in a cash runway into 2025.

Anticipated Milestones

- Top-line Clinical Trial Data Readouts
  - Pivotal SERENITY III Phase 3 trial (Part 1): May 2023
  - Phase 1b MAD trial for MDD program: May 2023
  - Pivotal TRANQUILITY II Phase 3 trial: June 2023
- Expected Clinical Trial Initiations
  - Pivotal SERENITY III Phase 3 trial (Part 2): Q2 2023
  - Phase 2b potential pivotal study of BXCL701 in SCNC: 2H 2023

Conference Call
BioXcel Therapeutics will host a conference call and webcast on May 8 at 8:00 a.m. ET to discuss its first 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 August 8, 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 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 its website.

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 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 has 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 probable 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; its ongoing marketing, commercialization and expansion efforts, plan and strategy for IGALMI; strategic options for OnkosXcel; the Company's participation in upcoming events and presentations; 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 significant indebtedness and other contractual obligations; its limited experience in drug discovery and drug development; 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 March 31, 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.

Website Disclosure
We announce material financial and operational information to our investors using press releases, SEC filings and public conference calls webcasts, as well as the Investors & Media section of our website at www.bioxceltherapeutics.com. We may use our website as a distribution channel of material information about the Company. 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.

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Source: BioXcel Therapeutics, Inc.

References and Notes

Prevalence estimates show prominent variability and gradients

1. IGALMI™ (dexmedetomidine) [package insert]. New Haven, CT. BioXcel Therapeutics, Inc.; 2022.
2. 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.
3. National Institute of Mental Health. Bipolar Disorder. Accessed April 5, 2022. https://rb.gy/lqz4rn.
4. UN Population Prospectus. Retrieved May 6, 2021. https://population.un.org/wpp.
5. IQVIA, 2021.
6. American Cancer Society. About Prostate Cancer. Accessed March 7, 2023. https://www.cancer.org/cancer/prostate-cancer/about/key-statistics.html.
7. B.R. Alabi, S. Liu and T. Stoyanova, Current and emerging therapies for neuroendocrine prostate cancer, Pharmacology and Therapeutics (2022), https://doi.org/10.1016/j.pharmthera.2022.108255.

BioXcel Therapeutics, Inc.

Statements of operations
(in thousands, except per share amounts)

	Three months ended March 31,	
	2023	2022
Revenues	$ 206	$ -
Operating expenses		
Cost of goods sold	$ 9	$ -
Research and development	27,800	18,559
Selling, general and administrative	23,595	12,921
Total operating expenses	$ 51,404	$ 31,480
Loss from operations	$ (51,198)	$ (31,480)
Other expense (income)		
Interest expense	3,367	7
Interest income	(2,015)	(15)
Other expense, net	246	-
Net loss and comprehensive loss	$ (52,796)	$ (31,472)
Net loss per share - basic and diluted	$ (1.84)	$ (1.12)
Weighted average shares outstanding - basic and diluted	28,616	27,980

Condensed Balance Sheets
(in thousands)

	March 31, 2023	December 31, 2022
Cash and cash equivalents	$ 165,521	$ 193,725

Working capital	$	148,509	$	169,970
Total assets	$	180,103	$	205,853
Long-term liabilities	$	98,339	$	96,180
Total liabilities	$	127,098	$	129,078
Total stockholders' equity	$	53,005	$	76,775