BioXcel Therapeutics Reports Third Quarter 2022 Financial Results and Recent Operational Highlights

November 10, 2022

IGALMI™ (dexmedetomidine) well-positioned for growth in 2023 based on increasing market access, efficient targeting, favorable market drivers, and sales force expansion in all major U.S. geographies

Top-line pivotal data for TRANQUILITY II trial investigating BXCL501 for Alzheimer's-related agitation expected in 1H 2023

Top-line pivotal data for SERENITY III trial investigating at-home use of BXCL501 for acute treatment of bipolar and schizophrenia-related agitation expected in 1H 2023

Completed multiple, seven-day daily dosing regimen cohorts in ongoing Phase 1 dose-selection trial in healthy volunteers to assess Major Depressive Disorder (MDD) program for at-home use; top-line results expected in 1H 2023

To host conference call today, November 10, 2022, at 8:30 a.m. ET

NEW HAVEN, Conn., Nov. 10, 2022 (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 third quarter ended September 30, 2022 and provided an update on key strategic initiatives.

"In the four months since IGALMI's trade launch, BioXcel Therapeutics is advancing its leadership position in the agitation-treatment market," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "In parallel, we are anticipating pivotal trial data readouts investigating BXCL501 for the treatment of Alzheimer's-related agitation, and bipolar and schizophrenia-related agitation in an at-home setting. We are well-positioned to potentially capture 139 million annual agitation episodes in the U.S.[1-5] Our company is rooted in AI-driven innovation, and we are proud to be at the forefront of developing transformative medicines in neuroscience."

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.[6] Up to 16 million institutional episodes occur annually within these two patient populations in the U.S.[1-3]

Commercial

- **Increased Market Access:** Generated strong initial interest in IGALMI from key stakeholder groups to drive formulary access and pull-through demand.
  - *Group Purchasing Organization (GPO) Progress:* Signed contract with largest GPO covering nearly 50% of target beds; in active discussions with the other leading GPOs.
  - *Pharmacy and Therapeutics (P&T) Committee Progress:* Nearly 470 P&T decisions are scheduled, with more than a dozen formulary wins and early product utilization recorded in the quarter.
  - *Integrated Delivery Network (IDN) Progress:* Recently deployed Corporate Account Directors (CADs) are dedicated to driving formulary and contracting process with 59 high-value IDNs and affiliated hospitals.
    - Formulary voting currently scheduled for approximately 41,000 (21%) target IDN beds.

- **Deployed IGALMI Institutional Sales Force:** Integrated commercial team to cover entire U.S. agitation market.
  - In May, initially launched institutional sales force with 26 reps covering 700 target hospitals, with 75% reach.
  - By December 1, expected to have deployed a total sales force of 70 reps to cover approximately 1,700 target hospitals.
  - Precise targeting of agitation opportunities by leveraging 81 billion claims records spanning over five years.
  - Positive market response and progress to date on access since launch, combined with expanded sales footprint, positions IGALMI well for sales growth in 2023.

- **Augmented Marketing Efforts:** Peer speaker programs and digital marketing driving IGALMI interest and awareness, with focus on message amplification in 2023.

- **Hosted IGALMI Commercial Day:** Outlined evolving agitation market dynamics, formulary process and contracting, positive initial momentum, and launch performance metrics of commercial success.

Medical Affairs

- **Increased Field Team Engagement:** Medical Science Liaison and Medical Managed Care teams actively engaged with medical community and P&T committee members.
    - Participated in numerous clinical and pharmacoeconomic discussions with GPO and P&T formulary decision-makers, utilizing dynamic budget impact model and physical restraint cost-estimator tools.
- **Published Two Manuscripts From Pivotal SERENITY Trials**: first manuscript from SERENITY I in *Journal of Clinical Psychiatry* and a post-hoc analysis of SERENITY I and II data in *Advances in Therapy*.
- **Participated / Presented at Leading Medical Conferences:** Psych Congress, Emergency Nurses Association (ENA) Conference, American College of Emergency Physicians (ACEP) Scientific Assembly, American Psychiatric Nurses Association (APNA) Conference, and Academy of Managed Care Pharmacy (AMCP) NEXUS.

Clinical Pipeline

BXCL501, a 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 in Alzheimer's-related agitation, where 100 million agitation episodes are estimated to occur in the U.S. annually.[4]
    - TRANQUILITY II: On track to announce top-line data in 1H 2023.
    - TRANQUILITY III: Expect to initiate enrollment in December 2022.
    - Independent Data and Safety Monitoring (DSM) committee periodically reviews subject safety and tolerability, recommending study continuation.

- **Bipolar or Schizophrenia-related Agitation (At-Home Use)**: SERENITY III program is designed to evaluate BXCL501 for at-home use, where 23 million at-home Rx and self-managed agitation episodes occur in the U.S. annually.[5]

SERENITY III consists of two parts:

- Part one: top-line efficacy data is expected in 1H 2023. Similar to SERENITY I and II, this trial will evaluate a 60mcg dose with the primary objective to assess efficacy in acute treatment of agitated bipolar I and II and schizophrenia patients.
- Part two: evaluation of the safety of self-administration of a 60mg dose at-home in comparison with placebo is expected to initiate in 1H 2023.

SERENITY III is expected to utilize similar investigators and clinical sites as the SERENITY I and II trials, which formed the basis for the FDA's approval of IGALMI.

- **Adjunctive Treatment for Major Depressive Disorder (MDD) for At-Home Use:** Ongoing Phase 1 trial is designed to test safety and tolerability of daily dosing of BXCL501 to inform dose selection for evaluating its potential use in combination with selective serotonin reuptake inhibitors (SSRIs) / serotonin-norepinephrine reuptake inhibitors (SNRIs) in MDD patients. Over 300 million antidepressant prescriptions are filled annually in the U.S., and current treatments are limited by slow onset of action and incomplete responses.[7]
    - Top-line results from the Phase 1 trial are expected in 1H 2023.
    - Progress to date includes multiple seven-day daily dosing regimen cohorts:
        - Daily dosing of 30mcg, 60mcg, or 80mcg is completed.
        - Twice daily dosing of 30mcg in the AM and 60mcg in the PM is completed.
        - Twice daily dosing of 40mcg in the AM and 80mcg in the PM is ongoing.
    - After a maximum tolerable dose is determined, the final cohort will initiate to test a daily maximum well-tolerated dose in combination with daily SNRI.

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, orally administered, systemic innate immune activator in development for the combination treatment of aggressive forms of prostate cancer.

- **Strategic Advancements:** Continued to evaluate strategic options, including potential third-party investments, enhanced operational capabilities, and progressed clinical and regulatory development plans for BXCL701.
- **Metastatic Castration-Resistant Prostate Cancer (mCRPC) Program:** Continued ongoing Phase 2 trial for BXCL701 in combination with KEYTRUDA (pembrolizumab) in mCRPC patients with small cell neuroendocrine (SCNC) or adenocarcinoma phenotype.
    - Presented updated, preliminary results demonstrating composite response rate of 33% for first 15 evaluable SCNC patients, and median duration of response of 9 months for the 5 responders at the Prostate Cancer Foundation Annual Scientific Retreat in October.
    - Completed enrollment of 28-patient SCNC cohort and expect to present final efficacy data from SCNC cohort in early 2023.

- Continued enrollment in adenocarcinoma randomized trial expansion evaluating BXCL701 monotherapy vs. BXCL701-KEYTRUDA combination therapy.

- **Predictive Biomarkers for BXCL701:** Presenting poster on potential predictive biomarkers for BXCL701 responsiveness in patients with leukemias at 37th Annual Meeting of the Society for Immunotherapy of Cancer (SITC) on November 10, 2022 *(poster embargo lifts on November 10 at 9 a.m. ET)*. Those biomarkers have potential implications for solid tumors.

Corporate Updates
Enhanced Intellectual Property:

- For the BXCL501 pipeline, two U.S. patents (10,792,246 & 11,478,422) have been issued with two additional Notices of Allowance received from the U.S. Patent and Trademark Office, along with seven patent allowances/issuances from foreign patent offices. The newly issued/allowed U.S. patents are expected to be included in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (commonly known as the Orange Book).

- For BXCL701, eight patent allowances/issuances were received from foreign patent offices.

Third Quarter 2022 Financial Results

Net Revenue: Net revenue of $137,000 for the quarter resulted from early product trial with limited market access. Due to the Company's direct shipping model to hospitals, no wholesaler stocking was expected.

Research and Development (R&D) Expenses: R&D expenses were $22.1 million for the third quarter of 2022, compared to $11.9 million for the same period in 2021. The increased expenses were primarily attributable to an increase in clinical trial costs as the Company expanded its BXCL501 clinical program for Alzheimer's-related agitation, at-home use, and MDD.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $17.1 million for the third quarter of 2022, as compared to $14.9 million for the same period in 2021. The increase was primarily due to personnel and costs related to the launch of IGALMI in the U.S.

Net Loss: BioXcel Therapeutics reported a net loss of $41.8 million for the third quarter of 2022, compared to a net loss of $26.8 million for the same period in 2021. The Company used $31.5 million in operating cash during the third quarter.

As of September 30, 2022, cash and cash equivalents totaled approximately $232.3 million.

Conference Call
BioXcel Therapeutics will host a conference call and webcast November 10, 2022, at 8:30 a.m., ET, to discuss its third quarter 2022 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 of the call will be available on the Investors section of the corporate website, bioxceltherapeutics.com, and a replay will be available through February 10, 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 is indicated for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. Limitations of Use: The safety and effectiveness of IGALMI have not been established beyond 24 hours from the first dose.

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 TRANQUILITY II and III

TRANQUILITY II and III are pivotal Phase 3 trials evaluating BXCL501 for the acute treatment of agitation in patients with probable Alzheimer's disease (AD). The trials include patients who experience agitation across diverse medical settings and across the range of dementia severity. On days when an acute episode of agitation arises, and pharmacological treatment is necessary, the efficacy and safety of dosing is assessed. TRANQUILITY II and III are designed to evaluate the safety and efficacy of BXCL501 for the potential treatment of the full spectrum of agitation associated with AD. Each trial will enroll approximately 150 dementia patients 65 years and older who will self-administer 40mcg or 60mcg of BXCL501 or placebo whenever agitation episodes occur over a three-month period. TRANQUILITY II initiated in December 2021, and will assess patients in assisted living or residential facilities requiring minimal assistance with activities of daily living. TRANQUILITY III will assess patients residing in nursing homes with moderate to severe dementia and require moderate or greater assistance with activities of daily living. The studies will assess agitation as measured by the changes from baseline in the Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score, and include Pittsburgh Agitation Scale (PAS) scores and Clinical Global Impression (CGI). The primary efficacy endpoint for both studies is change in PEC total score from baseline measured at two hours after the initial dose.

About SERENITY III

SERENITY III is a double-blinded, placebo-controlled, pivotal study designed to evaluate BXCL501 60mcg dose for at-home use. This strategic trial decision follows a Type B meeting with the U.S. FDA and observed dose-dependent responses in a prior Phase 1/2b study assessing 20mcg, 60mcg, 80mcg, 120mcg, and 180mcg doses. The first part of SERENITY III is similar to the SERENITY I and II pivotal trials and is designed to assess the efficacy and safety in acutely agitated patients with bipolar disorder or schizophrenia. The primary efficacy endpoint is change from baseline in Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score at two hours after dosing compared to placebo. The second part of SERENITY III is designed to assess safety of 60 mcg doses compared to placebo when self-administered at home. SERENITY III is expected to utilize many of the same investigators and clinical sites as SERENITY I and II.

About Major Depressive Disorder (MDD) Healthy Volunteer Study

The MDD development program has begun with a Phase 1 double-blind, placebo-controlled, multiple ascending dose (MAD) selection trial in healthy volunteers evaluating BXCL501 daily dosing. It is designed to inform dose selection in a future proof-of-concept study evaluating daily BXCL501 dosing in MDD patients. The current study includes cohorts of 18 volunteers who complete seven days of daily dosing. Cohorts of 30mcg, 60mcg, and 80mcg once daily have completed, as have cohorts of twice daily doses; 30mcg in the AM and 60mcg in the PM. Dose escalation is continuing with a cohort testing 40mcg in the AM and 80mcg in PM completing. Additional dose cohorts are planned. Once a maximum tolerable dose (MTD) is determined, escalation will be discontinued and a final cohort will initiate to test a daily maximal well-tolerated dose/ schedule in combination with daily SNRI for one week.

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 indices. 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 IGALMIhcp.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, orally administered, systemic innate immune activator for the treatment of aggressive forms of prostate cancer. 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 and commercialization efforts, plan and strategy for IGALMI; strategic options for OnkosXcel; 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 and BXCL701 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; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; impacts from the COVID-19 pandemic; its ability to commercialize its product candidates; and the other important factors discussed under the caption “Risk Factors” in its Quarterly Report on Form 10-Q for the quarter ended June 30, 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 September 30, 2022, 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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References and Notes

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BioXcel Therapeutics, Inc.

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

Three months ended September 30,		Nine months ended September 30,	
2022	2021	2022	2021

Revenues				
Product revenues	$ 137	$ -	$ 137	$ -
Operating expenses				
Cost of goods sold	$ 11	$ -	$ 11	$ -
Research and development	22,062	11,933	58,780	40,183
Selling, general and administrative	17,054	14,879	48,097	40,621
Total operating expenses	$ 39,127	$ 26,812	$ 106,888	$ 80,804
Loss from operations	$ (38,990)	$ (26,812)	$ (106,751)	$ (80,804)
Other (income) expense				
Interest expense (income), net	2,877	(1)	4,251	2
Other (income) expense, net	(62)	—	(53)	—
Net loss and comprehensive loss	$ (41,805)	$ (26,811)	$ (110,949)	$ (80,806)
Net loss per share - basic and diluted	$ (1.49)	$ (0.96)	$ (3.96)	$ (3.13)
Weighted average shares outstanding - basic and diluted	28,022	27,972	27,997	25,832

Condensed Balance Sheets
(Unaudited, in thousands)

	September 30, 2022	December 31, 2021
Cash and cash equivalents	$ 232,314	$ 232,968
Working capital	$ 218,242	$ 220,145
Total assets	$ 244,818	$ 239,439
Long-term liabilities	$ 94,413	$ 1,105
Total liabilities	$ 117,832	$ 17,772
Total stockholders' equity	$ 126,986	$ 221,667