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

August 9, 2022

Commercially launched IGALMI™ (dexmedetomidine) sublingual film for the acute treatment of agitation in bipolar I or II disorder and schizophrenia in adult patients

SERENITY III pivotal trial evaluating at-home use of BXCL501 for the acute treatment of agitation in bipolar and schizophrenia patients planned to initiate in 2H 2022 following recent Type B meeting with FDA

Top-line data from TRANQUILITY II pivotal trial for agitation associated with Alzheimer's disease expected in 1H 2023; TRANQUILITY III enrollment initiating in 2H 2022

Top-line results from ongoing Phase 1 Major Depressive Disorder dose-selection trial in healthy volunteers expected in 1H 2023; Completed 30mcg and 60mcg seven-day daily dosing regimen cohorts

Well-funded with cash runway into 2025[1]; received $100 million in total from previously announced $260 million of strategic financing

To host conference call today, August 9, 2022, at 8:30 a.m. ET

NEW HAVEN, Conn., Aug. 09, 2022 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a commercial-stage 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, 2022 and provided an update on key strategic initiatives.

"BioXcel Therapeutics made tremendous progress in its journey to becoming a fully integrated AI-driven commercial-stage company with the potential to transform the agitation treatment landscape," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "This transformation is being driven by the continued execution of our land and expand strategy within our neuroscience franchise. We are focused on the commercial launch for our recently FDA approved drug IGALMI™ while significantly increasing the opportunity for BXCL501 through at-home, medical setting expansion and the pursuit of multiple additional indications for our BXCL501 franchise. We believe we are well-positioned and have laid a strong foundation to drive long-term sustainable growth."

Company Highlights

Neuroscience Franchise

IGALMI™ (dexmedetomidine) sublingual film

IGALMI was approved by the U.S. Food and Drug Administration (FDA) on April 5, 2022 for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults.[2] Within these two patient populations in the U.S., up to 25 million agitation episodes occur each year.[3-5]

- **Trade and Market Access:** Key focus on unlocking access and supporting unencumbered demand generation for IGALMI.
  - IGALMI made available in 120mcg and 180mcg doses through the Company's third-party logistics provider and for order through wholesalers as of early July.
  - Engagement underway with 59 high-value Integrated Delivery Networks (IDNs) and their affiliated hospitals.
  - Discussions and negotiations ongoing with major national Group Purchasing Organizations (GPOs), representing over 90% of beds in target hospitals.

- **Institutional Sales Force:** Deployed first phase of our national sales force to target high-priority accounts in late May, with expansion of commercial footprint in process. Engaging multiple stakeholders in target hospitals and progressing P&T formulary review discussions. Gathered field level market intelligence which further reinforces and augments current and future commercial strategy.
  - Early response to the IGALMI value proposition exceeding expectations across full spectrum of institutional and healthcare stakeholders.
  - Market dynamics are rapidly evolving, favoring a less invasive and voluntary approach to treating agitation.

- **Marketing:** Strategic marketing focused on branded campaigns to support sales force activities.
  - Peer-to-peer speaker programs educating healthcare providers (HCPs) of the importance of early intervention to mitigate agitation episodes.
  - Digital marketing efforts driving significant engagement resulting in over 100,000 visits to IGALMI HCP website.

- **Investigating Potential Expansion of BXCL501 for At-Home Use for Agitation Associated with Bipolar Disorders**

and Schizophrenia: Planning SERENITY III double-blinded, placebo-controlled, pivotal study designed to evaluate BXCL501 60mcg dose for at-home use. This strategic trial decision follows successful completion of Type B meeting with FDA and observed dose-dependent responses in a prior Phase 1/2b study assessing 60mcg, 80mcg, 120mcg, and 180mcg doses. Of the approximately 25 million agitation episodes that occur in the U.S. each year related to schizophrenia and bipolar disorders, approximately one-third occur outside of the institutional setting.[6]

SERENITY III will consist of two parts:

- First part of SERENITY III is similar to the SERENITY I and II pivotal trials and designed to assess efficacy and safety in acutely agitated bipolar and schizophrenia patients.
  - 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.
- Second part of SERENITY III designed to assess safety compared to placebo when self-administered at home.

SERENITY III will utilize many of the same investigators and clinical sites as SERENITY I and II and is expected to initiate in 2H 2022.

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.

Indication Expansion

- **Alzheimer's Disease-related Agitation:** TRANQUILITY program is designed to capture Alzheimer's-related agitation market opportunity. There are an estimated 100 million agitation episodes in Alzheimer's patients occurring in the U.S. annually.[7]
  - TRANQUILITY II: top-line data readout expected in 1H 2023.
  - TRANQUILITY III: enrollment expected to begin in 2H 2022.
- **Adjunctive Treatment for Major Depressive Disorder (MDD):** Ongoing Phase 1 trial evaluating BXCL501 daily dosing designed to inform dose selection in future proof-of-concept study evaluating daily BXCL501 dosing in MDD patients. Over 300 million antidepressant prescriptions are filled annually in the U.S. and current treatments are limited by slow onsets of action and incomplete responses.[8]
  - Double-blind, placebo-controlled, multiple ascending dose (MAD) selection trial in healthy volunteers includes two cohorts of 18 patients, each having completed seven days of daily dosing of 30mcg or 60mcg compared to placebo.
    - Dose escalation in current cohort also enrolling to evaluate 80mcg.
    - Additional cohorts planned to evaluate safety and assess tolerability of a range of doses administered once daily and twice daily.
    - Top-line results expected in 1H 2023.

OnkosXcel Therapeutics

Established a wholly owned subsidiary to focus on the sustained expansion and optimization of the Company's immuno-oncology (I-O) franchise, including its most advanced I-O program, BXCL701. BXCL701 is an investigational orally administered, systemic innate immune activator in development for the treatment of aggressive forms of prostate cancer.

- **Strategic Advancements:** Fully functioning subsidiary dedicated to executing focused strategy and developing innovative oncology pipeline. Evaluating strategic options, which may include third party investments, aimed to fully capture unique value creation opportunity in areas of high unmet medical need.
- **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 carcinoma (SCNC) or adenocarcinoma phenotype.
  - Expect to complete enrollment of 28-patient SCNC cohort in 2H 2022.
  - Continued enrollment in adenocarcinoma randomized trial expansion evaluating BXCL701 monotherapy vs. BXCL701-KEYTRUDA combination therapy.

Corporate Updates

- **Strengthened Board of Directors:** Appointed Michael P. Miller to the Company's Board of Directors, to provide strategic leadership and commercial growth expertise.
- **Enhanced Intellectual Property:** Received two Notices of Allowance for patents (issued from patent application numbers

17/560,392 and 17/560,423) related to IGALMI to cover film formulations containing dexmedetomidine and methods of treating agitation using the films.

Second Quarter 2022 Financial Results

Research and Development Expenses: Research and development expenses were $17.9 million for the second quarter of 2022, compared to $13.5 million for the same period in 2021. The increased expenses were primarily attributable to clinical trial costs related to the Company's TRANQUILITY program.

Selling, General and Administrative Expenses: Selling, general and administrative expenses were $18.4 million for the second quarter of 2022, as compared to $14.1 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 $37.7 million for the second quarter of 2022, compared to a net loss of $27.6 million for the same period in 2021.

As of June 30, 2022, cash and cash equivalents totaled approximately $233.5 million. This excludes $30 million of contributions from the $260 million strategic financing announced in April. To date the Company has met the milestones and has received $100 million from the agreement.

Conference Call

BioXcel Therapeutics will host a conference call and webcast August 9, 2022, at 8:30 a.m., ET, to discuss its second 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 BioXcel website, www.bioxceltherapeutics.com, and a replay of the call will be available through November 9, 2022.

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 TRANQUILITY II and III
Initiated in December of 2021, 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 expand the evaluation of patients who experience agitation across diverse medical settings and across the range of dementia severity. TRANQUILITY II and III are designed to maximize the opportunity 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 40 mcg or 60 mcg of BXCL501 or placebo whenever agitation episodes occur over a three-month period. TRANQUILITY II 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) and Pittsburgh Agitation Scale (PAS) total scores. The primary efficacy endpoint for both studies is change in PEC score from baseline measured at two hours after the initial dose and subsequent doses.

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. is a commercial-stage 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 www.IGALMIhcp.com and also see the IGALMI full Prescribing Information. BXCL501 is under investigation for the acute treatment of agitation associated with probable Alzheimer's disease, and as an adjunctive treatment for major depressive disorder. 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 immunity activator, for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. For more information, please visit www.bioxceltherapeutics.com.

Forward-Looking Statements
This press release includes "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this press release include but are not limited to: statements regarding the Company's expected timing of, and data results from, trials and clinical studies involving its product candidates; planned discussions with regulators; its commercial plan and strategy for IGALMI and strategic options for OnkosXcel; and the Company's future financial and operational results. When used herein, words including "anticipate," "will," "plan," "may," "continue," "intend," "designed," "goal" and similar expressions are intended to identify forward-looking statements. 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 quarterly period ended March 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, 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.

Contact Information

Corporate

BioXcel Therapeutics
Erik Kopp
1.203.494.7062
ekopp@bioxceltherapeutics.com

Investor Relations

BioXcel Therapeutics
Brennan Doyle
1.475.355.8462
bdoyle@bioxceltherapeutics.com

Media

FTI Consulting
Helen O’Gorman
1.718.408.0800
helen.ogorman@fticonsulting.com

References and Notes

1. Assumes full funding under our strategic financing agreements entered into on April 19, 2022, including funding of remaining tranches subject to regulatory and financial milestones and certain other conditions.
2. IGALMI™ (dexmedetomidine) [package insert]. New Haven, CT: BioXcel Therapeutics, Inc.; 2022.
3. 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.
4. National Institute of Mental Health. Bipolar Disorder. Accessed April 5, 2022. https://rb.gy/lqz4rn
5. UN Population Prospectus. Retrieved May 6, 2021. https://population.un.org/wpp.
6. Longitudinal patient claims from Symphony Health Solutions
7. Tractenberg, R Neuropsychiatry Clin Neuroscience 14:1 Winter 2002
8. NIH/WHO, SAMHSA, NIMH, Pratt et al, 2017

Bioxcel Therapeutics, Inc. (BTAI)
Consolidated Statements of Operations
(Unaudited in thousands except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses				
Research and development	$ 17,906	$ 13,509	$ 36,593	$ 28,250
Selling, general and administrative	18,382	14,104	31,175	25,742
Loss from operations	$ (36,288)	$ (27,613)	$ (67,768)	$ (53,992)
Other income (expense)				
Interest income	204	10	219	20
Interest expense	(1,586)	(16)	(1,593)	(23)
Net loss	$ (37,670)	$ (27,619)	$ (69,142)	$ (53,995)
Net loss per-basic and diluted	$ (1.35)	$ (1.11)	$ (2.47)	$ (2.18)
Weighted average shares outstanding -				

basic and diluted	27,989	24,962	27,985	24,744

Condensed Consolidated Balance Sheets
(Unaudited, in thousands)

	June 30, 2022	December 31, 2021
Cash and cash equivalents	$ 233,452	$ 232,968
Working Capital	$ 224,929	$ 220,145
Total assets	$ 248,235	$ 239,439
Long-term liabilities	$ 63,978	$ 1,105
Total liabilities	$ 83,973	$ 17,772
Total stockholders' equity	$ 164,262	$ 221,667