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

May 9, 2022

Received FDA approval of IGALMI™ (dexmedetomidine) sublingual film for acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults

Top-line data readout in Q4 2022/early Q1 2023 for TRANQUILITY II Phase 3 trial evaluating BXCL501 in acute treatment of agitation associated with Alzheimer's disease

Extended cash runway into 2025 with $260 million strategic financing for IGALMI commercial launch and clinical and regulatory milestones

U.S. national salesforce deployment to commence on May 23, 2022

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

NEW HAVEN, Conn., May 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 first quarter ended March 31, 2022 and provided an update on key strategic initiatives.

"2022 has already been a transformative year during which we have made monumental progress on our journey to become the leading AI-enabled neuroscience company. Our recent achievements across our clinical, business, and commercial priorities, highlighted by our first FDA approval of IGALMI, have positioned us to realize our 2022 goals and beyond," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "Building on this momentum, we are excited to launch IGALMI, advance our proven technology, and continue delivering innovative medicines to patients and caregivers."

Company Highlights

Neuroscience Franchise

IGALMI™ Approval and Commercial Launch

IGALMI was approved by the FDA on April 5, 2022 for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults.[1] Within these two patient populations in the U.S., up to 25 million agitation episodes occur each year.[2-4]

The Company is focused on the following key commercial launch activities to provide access to patients and caregivers:

- **Institutional Sales Force**: Seasoned sales specialists were onboarded, undergoing training, and will be deployed for launch. The team has an average of over 21 years of industry experience, 14 years in hospital settings, and 8 product launches.
  - Launch meeting to be held in mid-May in preparation for national salesforce deployment on May 23rd across priority regions and customers.
- **Market Access Team**: Currently engaging with group purchasing organizations (GPOs) and high value Integrated Delivery Networks (IDNs).
- **Trade Launch:** Product load into distribution channels in Q3 2022.

Clinical Pipeline

BXCL501, a proprietary, sublingual film formulation of dexmedetomidine, is being investigated in multiple neuropsychiatric conditions. BXCL501 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 designed to maximize BXCL501 opportunity to treat Alzheimer's Disease-related agitation. There are an estimated 100 million agitation episodes in Alzheimer's patients occurring in the U.S. annually.[5]
  - TRANQUILITY II: First patient dosed; top-line data readout in Q4 2022/early Q1 2023.
  - TRANQUILITY III: Trial underway with enrollment initiating in 2H 2022.
  - Selection of 40 mcg and 60 mcg dosing regimens in pivotal trial supported by breakthrough designation from the FDA, along with positive efficacy, safety, and tolerability data observed in 100 patients in the Phase 1b/2 TRANQUILITY trial and a recent study of BXCL501 40 mcg.

- **Adjunctive Treatment for Major Depressive Disorder (MDD):** Initiated Phase 1 multiple ascending dose trial in healthy volunteers.

Geographic Expansion

- **Marketing Authorization Application:** Submission to the European Medicines Agency (EMA) for BXCL501 for acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults in 2Q 2022.
- **Japan Entry**: Company exploring market entry strategies to expand access of IGALMI to patients in Japan.

OnkosXcel Therapeutics

Established OnkosXcel Therapeutics as 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 and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors.

- **Metastatic Castration-Resistant Prostate Cancer (mCRPC) Program:** Following positive Phase 2 data for BXCL701 in combination with KEYTRUDA® (pembrolizumab) announced this past February, continued ongoing Phase 2 trial in mCRPC patients with either small cell neuroendocrine carcinoma (SCNC) or adenocarcinoma phenotype.
    - Expect to complete enrollment of 28-patient SCNC cohort in 2H 2022.
    - First patient enrolled in adenocarcinoma randomized trial expansion evaluating BXCL701 monotherapy vs. BXCL701-KEYTRUDA combination therapy.

- **Solid Tumors Program (Checkpoint Naïve and Refractory):** Additional efficacy data from MD Anderson-led open-label Phase 2 basket trial of BXCL701 and KEYTRUDA is expected in 2H 2022.

Strategic Financing

- **Announced $260 Million Strategic Financing with Oaktree and Qatar Investment Authority:** Full execution of this financing would extend the Company's cash runway into 2025 to support the commercial launch of IGALMI and achieve key clinical and regulatory milestones.

First Quarter 2022 Financial Results

Research and Development Expenses: Research and development expenses were $18.6 million for the first quarter of 2022, compared to $14.7 million for the same period in 2021. The increased expenses were primarily attributable to clinical trial costs related to the Company's TRANQUILITY program.

General and Administrative Expenses: General and administrative expenses were $12.9 million for the first quarter of 2022, as compared to $11.6 million for the same period in 2021. The increase was primarily due to personnel and costs related to the commercial launch readiness efforts for IGALMI in the U.S.

Net Loss: BioXcel Therapeutics reported a net loss of $31.5 million for the first quarter of 2022, compared to a net loss of $26.4 million for the same period in 2021.

As of March 31, 2022, cash and cash equivalents totaled approximately $200 million. This excludes contributions from the $260 million strategic financing announced in April. To date the Company has drawn $70M of the loan agreement and has met the milestone to receive $30M of the royalty financing which is expected to be drawn in the second quarter of 2022.

Conference Call

BioXcel Therapeutics will host a conference call and webcast May 9, 2022, at 8:30 a.m., ET, to discuss its first 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 August 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 Alzheimer's disease. 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 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 by the FDA 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. BXCL501 is also being evaluated for the acute treatment of agitation associated with 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 and, under its subsidiary OnkosXcel Therapeutics, 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 the commercialization and medical affairs plans for IGALMI, timing and data from clinical trials for BXCL501 and BXCL701, the timing of the Company's MAA application for BXCL501, and 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 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; the Company has no experience in marketing and selling drug products; 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 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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Source: BioXcel Therapeutics, Inc.

References

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. Tractenberg, R Neuropsychiatry Clin Neuroscience 14:1 Winter 2002

BioXcel Therapeutics, Inc.

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

	Three months ended March 31,	
	2022	2021
Revenues	$ -	$ $ -
Operating expenses		
Research and development	$ 18,559	$ 14,741
General and administrative	12,921	11,638
Total operating expenses	$ 31,480	$ 26,379
Loss from operations	$ (31,480)	$ (26,379)
Other income (expense)		
Interest income, net	8	3
Net loss and comprehensive loss	$ (31,472)	$ (26,376)
Net loss per share - basic and diluted	$ (1.12)	$ (1.08)
Weighted average shares outstanding - basic and diluted	27,980	24,524

Condensed Balance Sheets
(Unaudited, in thousands)

	March 31, 2022	December 31, 2021
Cash and cash equivalents	$ 200,435	$ 232,968
Working capital	$ 191,565	$ 220,145
Total assets	$ 211,045	$ 239,439
Long-term liabilities	$ 1,028	$ 1,105
Total liabilities	$ 17,025	$ 17,772
Total stockholders' equity	$ 194,020	$ 221,667