BioXcel Therapeutics Announces First Patient Dosed in TRANQUILITY II Phase 3 Trial for Acute Treatment of Agitation in Patients with Alzheimer's Disease

May 3, 2022

Top-line data for TRANQUILITY II anticipated in Q4 2022/early Q1 2023

Phase 3 TRANQUILITY III study underway with patient enrollment initiating in H2 2022

NEW HAVEN, Conn., May 03, 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 the first patient has been dosed in the Phase 3 TRANQUILITY II study of BXCL501, the Company's proprietary, orally dissolving thin film formulation of dexmedetomidine, for the acute treatment of agitation in patients with Alzheimer's disease (AD). The pivotal Phase 3 TRANQUILITY program includes two studies, TRANQUILITY II and TRANQUILITY III, which are designed to evaluate the safety and efficacy of BXCL501 in adults 65 years and older in assisted living or residential facilities and nursing homes.

"There are an estimated 100 million agitation episodes annually in the U.S. associated with Alzheimer's disease [1], which have a devastating impact on patients and their caregivers," said Robert Risinger, M.D., Chief Medical Officer of BioXcel Therapeutics. "We believe the recent FDA approval of BXCL501 for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults has laid a strong foundation for pursuing this Alzheimer's-related agitation program to potentially address this debilitating symptom for patients. Importantly, we are also expanding TRANQUILITY II to more than 10 clinical trial sites in the U.S. and with no current FDA approved treatments for agitation associated with this disease, we are making strong and swift efforts to potentially bring BXCL501 and its proven ability to address agitation to this large market."

The Company's decision to continue the evaluation of both the 40 and 60 mcg dosing regimens in the TRANQUILITY II and III pivotal trials is further supported by results from a recent 46 patient, multicenter, placebo-controlled study evaluating the efficacy, safety and tolerability of BXCL501 40 mcg dose in patients with agitation associated with dementia. Previously, BXCL501 was granted Breakthrough Therapy designation from the U.S. Food and Drug Administration (FDA) for the acute treatment of agitation associated with dementia. BXCL501 demonstrated statistically significant reductions in agitation measures with both the 30 and 60 mcg doses as measured by multiple scales with no severe or serious adverse events.

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 Alzheimer's Disease

Alzheimer's disease is the most prevalent type of dementia in the United States. By 2040, approximately 12 million Americans aged 65 years and over are expected to be impacted by the condition, double the approximately 6 million Americans impacted in 2020.[2,3] Of these patients, up to 70% experience agitation, with an estimated 100 million agitation episodes occurring in the United States every year.[1,4] There are no approved therapeutic options for the acute treatment of agitation related to dementia, including Alzheimer's disease.

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. 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, 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 timing and data from the TRANQUILITY II and TRANQUILITY III trials and the potential value of BXCL501 as a treatment option for agitation in patients with AD. When used herein, words including "anticipate," "will," "plan,"

“potential,” “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; the Company’s need for substantial additional funding and ability to raise capital when needed; the Company’s dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502 and BXCL701 and other product candidates; 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; the regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, expensive and inherently unpredictable; the Company has limited experience in drug discovery and drug development; regulatory agencies, may not accept or agree with the Company’s assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and the Company in general; its ability to enroll patients in its clinical trials; undesirable side effects caused by the Company’s products and product candidates; its novel approach to the discovery and development of product candidates based on EvolverAI; regulatory agencies may not accept or agree with the Company’s assumptions, estimates, calculations, conclusions, or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and the Company in general; the Company has no experience in marketing and selling drug products and has not entered into arrangements for the sale and marketing of IGALMI™; IGALMI™ or the Company’s product candidates may not be accepted by physicians or the medical community in general; the Company may not be able to obtain marketing approvals for BXCL501, BXCL502 or BXCL701; the Company may need substantial additional funding to develop and conduct clinical trials with respect to its product candidates and support its operations; the Company must comply with extensive regulations applicable to it; and healthcare reform could adversely impact future commercial success. These and other important factors are discussed under the caption “Risk Factors” in its Annual Report on Form 10-K for the year ended December 31, 2021, 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.

Contact Information

Corporate

BioXcel Therapeutics
Erik Kopp
1.203.494.7062
ekopp@bioxceltherapeutics.com

Investor Relations

FTI Consulting
Matt Ventimiglia
1.212.850.5624
matthew.ventimiglia@fticonsulting.com

Media

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

Source: BioXcel Therapeutics, Inc.

[1] Estimate based on company market research

[2] Prevalence of bipolar disorder in adults. November 2017. Accessed June 24, 2021. https://www.hcp.med.harvard.edu/ncs/ftpdir/NCS-R_12-month_Prevalence_Estimates.pdf

[3] Alzheimer’s Association

[4] Tractenberg, R Neuropsychiatry Clin Neuroscience 14:1 Winter 2002