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

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Pivotal trial will evaluate efficacy and safety of BXCL501 in Alzheimer's patients in nursing homes with moderate to severe dementia

Estimated 100 million annual Alzheimer's-related agitation episodes [1] could potentially increase current U.S. market opportunity for BXCL501 by more than six-fold [2-4]

TRANQUILITY II enrollment on track, with top-line data anticipated in 1H 2023

NEW HAVEN, Conn., Dec. 19, 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 that the first patient has been dosed in the pivotal Phase 3 TRANQUILITY III trial of BXCL501 (dexmedetomidine) sublingual film, the Company's proprietary, orally dissolving film, under investigation for the acute treatment of agitation in patients with Alzheimer's disease (AD). AD is the most prevalent type of dementia in the U.S.[5] The TRANQUILITY program includes two investigational studies, TRANQUILITY II and TRANQUILITY III, which are designed to evaluate the safety and efficacy of BXCL501 for the acute treatment of Alzheimer's-associated agitation in adults 65 years and older in assisted living or residential care facilities and nursing homes.

"The prevalence of Alzheimer's disease is unfortunately increasing and there remains no FDA-approved product indicated for patients experiencing agitation associated with this condition," said Robert Risinger, M.D., Chief Medical Officer, Neuroscience of BioXcel Therapeutics. "With two pivotal trials underway in our TRANQUILITY program, we are aiming to expand BXCL501's potential to treat the full spectrum of episodic and intermittent chronic agitation market, and address the costly health-care burden related to Alzheimer's agitation."

There are approximately 100 million reported agitation episodes that occur in the U.S. each year related to Alzheimer's disease. [1] The number of adults over the age of 65 with AD is expected to double from 5.8 million in 2020 to 11.8 million in 2040 [6], representing a significant and growing market opportunity for BXCL501. This potential opportunity is in addition to the current acute treatment of agitation associated with schizophrenia or bipolar I or II disorder market opportunity. Approximately 16 million episodes of schizophrenia and bipolar disorder-associated agitation occur in institutional settings and, when combined with at-home episodes, 23 million annually in the U.S.[2-4]

TRANQUILITY II and III will evaluate the safety and efficacy of BXCL501 in patients who experience agitation across diverse settings and across the range of dementia severity. Each trial will enroll approximately 150 patients with dementia ages 65 years and older who will self-administer 40mcg or 60mcg of BXCL501 or placebo under the supervision of a trained research staff member whenever agitation episodes occur over a three-month period. TRANQUILITY II will assess patients in assisted living or residential care facilities requiring minimal assistance with activities of daily living. TRANQUILITY III will assess patients residing predominantly in nursing homes with moderate to severe dementia who require moderate or greater assistance with activities of daily living. The primary efficacy endpoint for both studies is change in Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score from baseline measured at two hours after the initial dose and subsequent doses.

About BXCL501
BXCL501 is an investigational proprietary, orally dissolving film formulation of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist. BioXcel Therapeutics believes that BXCL501 potentially targets an important mediator of agitation, and the Company has observed anti-agitation results in multiple clinical studies across several neuropsychiatric disorders. BXCL501 is under investigation for the acute treatment of agitation associated with bipolar I or II disorder or schizophrenia in the at-home setting, for the acute treatment of Alzheimer's-related agitation, and as an adjunctive treatment for major depressive disorder. The safety and efficacy of BXCL501 for these investigational uses have not been established. BXCL501 has been granted Breakthrough Therapy designation for the acute treatment of agitation associated with dementia and Fast Track designation for the acute treatment of agitation associated with schizophrenia, bipolar disorders, and dementia.

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 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 Alzheimer's-related agitation, 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, the Company’s expected timing of, trial design and data results from, clinical trials of BXCL501, and potential market opportunities for BXCL501. When used herein, words including “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 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 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; and the other important factors discussed under the caption “Risk Factors” in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 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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