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BioXcel Therapeutics Announces First Patient Enrolled in Phase 1b/2 Study of BXCL501 for Acute Treatment of Agitation Associated with Dementia

Foundational study with plans to address multiple types of agitation associated with dementia

Expands the potential therapeutic use of BXCL501 beyond neuropsychiatric disorders

Topline results expected in mid-2020

NEW HAVEN, Conn., Jan. 07, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics (“BTI” or “Company”) (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology, today announced that the first patient has been enrolled in a Phase 1b/2 study of BXCL501, the Company’s proprietary sublingual thin-film formulation of dexmedetomidine (“Dex”), for the acute treatment of agitation in patients with dementia.

“We are excited to advance the clinical development of BXCL501 in agitation associated with dementia, a third potential indication for our lead product candidate,” commented Robert Risinger, M.D., Vice President, Clinical Development of BTAI. “With no approved treatments, there is a significant need for better treatment options for the 6 million patients in the U.S. with dementia, many of whom will experience agitation during the course of their disease. We believe our candidate, BXCL501, with its unique mechanism and convenient oral dosing, has the potential to rapidly reduce agitation associated with dementia without excessive sedation or other undesired side effects. This study builds upon the foundation for a broad BXCL501 program in dementia, including the treatment of chronic agitation and the prevention of agitation using a wearable device in combination with BXCL501.”

The multicenter, randomized, double-blind, placebo-controlled, ascending dose Phase 1b/2 study is designed to evaluate the efficacy, pharmacokinetics, safety and tolerability of BXCL501 in adults 65 years and older who exhibit acute agitation associated with all forms of dementia, including Alzheimer's disease. The dementia program expands on the Phase 1b study performed with BXCL501 in 135 patients with agitation associated with schizophrenia, and also builds on the positive results observed in reducing agitation with intravenous Dex in Alzheimer’s disease patients. This is an adaptive design and is expected to assess multiple dose cohorts of BXCL501 or matching placebos. Following the completion of each dose cohort, a safety and tolerability review is expected to be performed to determine the next tested dose. The study is designed to assess agitation as measured by the Pittsburgh Agitation Scale, a validated clinical instrument, as well as improvement in the modified Cohen Mansfield Agitation Inventory and Positive and Negative Syndrome Scale,

Excitatory Component (“PEC”).

About BXCL501

BXCL501 is a potential first-in-class, proprietary sublingual thin film of dexmedetomidine, a selective alpha-2a receptor agonist for the treatment of acute agitation. BTI believes that BXCL501 directly targets a causal agitation mechanism and the Company has observed anti-agitation effects in multiple clinical studies across multiple neuropsychiatric indications. BXCL501 has also been granted Fast Track Designation by the U.S. Food and Drug Administration for the acute treatment of agitation.

A Phase 1b safety and efficacy study of BXCL501 yielded positive dose-response data. BXCL501 is being evaluated in the SERENITY program, consisting of two Phase 3 studies for the acute treatment of agitation in patients with schizophrenia (SERENITY I) and bipolar disorder (SERENITY II). BXCL501 is also being evaluated in a Phase 1b/2 trial for the treatment of agitation associated with dementia.

About Agitation Associated with Dementia
Dementia is a neurocognitive condition caused by damage to brain cells that leads to a decline in cognitive abilities and independent function. It affects approximately 6 million individuals in the United States, with Alzheimer’s disease accounting for 60-70% of these cases. During the course of the disease, patients with dementia often suffer from psychological and behavioral symptoms, such as agitation, which has been reported in up to 70% of patients. Agitation associated with dementia can negatively affect both the patient and caregiver’s quality of life. Caregiver burden can contribute significantly to burnout, which can result in premature institutionalization of the patient. Treating agitation associated with dementia has been a challenge for providers and current standards of care often have a slow onset of action and/or cause excessive sedation. There are currently no FDA-approved therapies for the treatment of dementia-related agitation, and off-label therapies have black box warnings associated with their use.

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology. BTI'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. BTI's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed for treatment of a rare form of prostate cancer, pancreatic cancer and advanced solid cancers in combination with other immuno-oncology agents. For more information, please visit http://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 clinical development initiatives and trials for BXCL501. When used herein, words including “anticipate,” “being,” “will,” “plan,” “may,” “continue,” 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 BTI's current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. BTI 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 BXCL501 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; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; its approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; 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 September 30, 2019, 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 BTI 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 BTI’s views as of any date subsequent to the date of this press release.

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