BioXcel Therapeutics Announces Initiation of a Phase 2 Study Designed to Assess Agitation-Associated Biomarkers and their Response to BXCL501

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Study aims to support BXCL501's potential market expansion in chronic agitation disease conditions

Topline results expected in the second quarter of 2020

NEW HAVEN, Conn., Feb. 18, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("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 the initiation of a Phase 2 study by researchers at Yale University designed to measure biomarkers associated with agitation in patients with schizophrenia and the response to treatment with BXCL501. The Company aims to utilize biomarkers to identify additional indications that exhibit the same physiological signals of hyperarousal, expanding the potential use of BXCL501 to new chronic disease indications.

"Building on the significant results from our Phase 1b trial in patients with agitation associated with schizophrenia, this study is designed to further confirm the calming capabilities of dexmedetomidine, the active ingredient in BXCL501, using an objective scale to measure signs of hyperarousal," commented Dr. Frank Yocca, Chief Scientific Officer of BTI. "In an agitated state, there are physiological changes that may occur, including differences in heart rate, electrodermal activity and EEG (electroencephalography), which have the potential to be used as an initial signal for treatment with BXCL501. In addition, we believe these biomarkers may have relevance for the treatment of additional distinct chronic indications characterized by nervous system arousal, including Post-Traumatic Stress Disorder and alcohol withdrawal symptoms."

"Managing agitation, a common symptom of neuropsychiatric conditions, is a burdensome challenge for both physicians and caregivers," added Dr. John Krystal, M.D., Robert L. McNeil, Jr. Professor of Translational Research and Professor of Psychiatry and of Neuroscience; Co-Director, Yale Center for Clinical Investigation and Chair, Department of Psychiatry at Yale School of Medicine. "The ability to detect bodily signals that indicate an agitated state prior to the onset of visible symptoms could be extremely beneficial to caregivers. An early signal will allow for sufficient time to proactively treat the agitation before it becomes dangerous to the individuals involved."

The study will assess biomarkers, such as heart rate variability, actigraphy, electrodermal activity and EEG in patients with schizophrenia. Measurements will be taken at baseline and after the dosing of BXCL501 to determine its ability to impact the physiological signals of agitation. Topline data is expected to be reported in the second quarter of 2020.

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, and the Company is preparing to initiate the Phase 1b/2 RELEASE trial of BXCL501 for the treatment of opioid withdrawal symptoms.

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 ability to assess biomarkers for agitation and the timing of data from such trials involving 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 and on the Company’s website at www.bioxceltherapeutics.com.

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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