



BioXcel Therapeutics Achieved Targeted Exposures of BXCL501 Designed For Non-Invasive Acute Treatment of Agitation in Neuropsychiatric Diseases

May 20, 2019

Phase 2 efficacy trial in agitated schizophrenia patients anticipated to commence imminently

BXCL501 is a potential first-in-class selective alpha-2a receptor agonist, formulated as a proprietary sublingual thin film of dexmedetomidine

Targeted exposure levels that were observed to be therapeutic in prior IV Dex study rapidly achieved in preliminary pharmacokinetic profile from Phase 1 study

On track to initiate first Phase 3 pivotal trial in 2H 2019 with NDA submission expected in 2020

Global development plans in agitated dementia, opioid withdrawal symptoms and hyperactive delirium to expand market will be unveiled through 2019

Conference call to discuss results and BXCL501 program advancement on May 20, 2019 at 8.30 ET

NEW HAVEN, Conn., May 20, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI), today announced positive top line data from its Phase 1 pharmacokinetic (PK) (bioavailability) and safety study of BXCL501, a proprietary sublingual thin-film formulation of dexmedetomidine (Dex), for the acute treatment of agitation across multiple neuropsychiatric indications. Administration of BXCL501 in the Phase 1 pharmacokinetic and safety study successfully achieved targeted exposure levels that were observed to be therapeutic in the Company's prior IV Dex study and BTI intends to advance BXCL501 into a Phase 2 trial to evaluate efficacy in agitated schizophrenia patients. We believe the results from the Phase 2 study are expected to facilitate powering of a planned Phase 3 pivotal trial. BTI is a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify and advance next wave of medicines in neuroscience and immuno-oncology.

The IND-opening Phase 1 study was a double-blinded placebo-controlled, single-dose, dose-escalation study of BXCL501 that enrolled 42 adult volunteers across various dosing groups. The primary endpoints of the study were PK and safety, while secondary endpoints included assessment of pharmacodynamics (PD) and the relationship between BXCL501 concentrations and PD endpoints.

Findings from the study indicate that BXCL501 rapidly achieved targeted exposure levels consistent with the levels observed in the intravenous ("IV") Dex study in schizophrenia patients that the Company announced in November 2018. Results from the Phase 1 study also showed dose-proportional PK consistent with the IV Dex study with PD effects lasting 4 to 6 hours, which we believe are clinically favorable features.

Additionally, clinical data from the study indicates BXCL501 was well tolerated. There were no serious adverse events. The most common adverse event was drowsiness, observed at rates similar to placebo. All adverse events were mild to moderate and transient. There was no clear sedative effect in comparison with placebo. Cardiovascular changes were not clinically meaningful. A maximum tolerated dose was not reached.

Vimal Mehta, Ph.D., Chief Executive Officer of BTI, added, "We are excited to report that sublingual administration of BXCL501 in our Phase 1 study achieved drug exposures that we believe will be therapeutic in the acute treatment of agitation. Additionally, the PK profile of sublingual BXCL501 demonstrated dose proportionality and suggests the potential for a rapid onset of action. Agitation is believed to represent a multibillion dollar burden to the healthcare system severely lacking effective non-invasive treatment options. We believe that robust datasets from this new Phase 1 PK and safety study provide a strong rationale for advancing the clinical development of the BXCL501 program. With successful completion of this study and positive findings, we are now a step closer to our objective of potentially providing acutely agitated patients with a non-invasive and easy-to-administer treatment option."

Robert Risinger, M.D., Vice President, Clinical Development of BTI, commented, "We are pleased to advance our potent selective alpha-2 agonist, BXCL501, by expanding our clinical development program. Multiple proof-of-concept studies have shown that Dex produced a calming effect in illnesses where agitation is a common symptom. If approved, we believe BXCL501 may offer patients and physicians a novel approach to treat acute agitation associated with a broad spectrum of conditions. We believe that BXCL501 may provide rapid relief in a delivery form that can easily be administered in all clinical settings where agitated patients are encountered."

Conference Call:

BTI will host a conference call today at 8:30 a.m. ET. To access the call please dial 1-800-289-0438 (domestic) and 1-323-994-2082 (international) and provide the passcode 2971079. A live webcast of the call and a copy of the Company's presentation materials that will be discussed on the call will be available on the Investors sections of the BTI website at www.bioxccltherapeutics.com. The archived webcast will be available through June 20, 2019.

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 using IV (intravenous) Dex has shown anti-agitation effects in multiple clinical studies.

About BioXcel Therapeutics, Inc.:

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence approaches to identify and advance next wave of medicines 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 and for treatment of pancreatic cancer in combination with other immuno-oncology agents. 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, statements that relate to the advancement and development of BXCL501 and BXCL701, the commencement of clinical trials, the availability and results of data from clinical trials, the planned timing of BTI’s submission of its first New Drug Application with the FDA and other information that is not historical information. 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 period ended March 31, 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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