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BioXcel Therapeutics Completes Dosing Two Cohorts of Agitated Schizophrenia Patients with BXCL501 in a Phase 2 Efficacy Trial

Total of 96 agitated patients and healthy volunteers have received multiple doses of BXCL501, a proprietary sublingual thin film

On track to announce top line results from Phase 2 efficacy trial in 3Q 2019

Planning to initiate first Phase 3 pivotal trial in 4Q 2019

NEW HAVEN, Conn., June 10, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI), announced today that it has completed dosing of the first two cohorts of agitated schizophrenia patients in its Phase 2 efficacy trial of BXCL501, the Company's proprietary sublingual thin-film formulation of dexmedetomidine ("Dex"). BTI plans to use the results from this study to pursue two planned Phase 3 pivotal trials in agitated schizophrenia and bipolar disorder patients currently expected to begin in 4Q 2019. BTI is a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology.

The first two cohorts in this double-blind, placebo-controlled, Phase 2 efficacy trial totaling 54 patients were completed expeditiously within three weeks of study initiation. Currently, the Company plans to enroll approximately 200 agitated schizophrenia patients. The primary endpoint of the study is change from baseline measured by the total score of Positive and Negative Symptom Scale - Excitatory Component scale[1] ("PEC"). The secondary endpoints include assessment using the Agitation Calmness Evaluation Scale[2] ("ACES").

Vimal Mehta, Ph.D., Chief Executive Officer of BTI, stated, "We are pleased with the progress of our Phase 2 efficacy study with BXCL501 in agitated schizophrenia patients, a study we believe will support the launch of our pivotal Phase 3 studies in late 2019. We have now treated 96 subjects with BXCL501 to date, in addition to the positive data from our human Proof-of-Concept studies in multiple neuropsychiatric conditions with intravenous ("IV") Dex. In parallel, we are accelerating clinical development plans for agitated dementia, opioid withdrawal symptoms and hyperactive delirium outside the currently on-going schizophrenia study. We continue to believe that agitation represents a multi-billion dollar burden to the healthcare system and is severely lacking effective and safe non-invasive treatment options."

Robert Risinger, M.D., Vice President, Clinical Development of BTI, added, "We are delighted to be advancing the efficacy trial of our potent selective alpha-2 agonist, BXCL501.

With successful completion of dosing in the first two patient cohorts, we are excited and looking forward to the results from this efficacy trial that are expected in 3Q 2019. Data from the Phase 1 pharmacokinetic (bioavailability) and safety study of BXCL501 exhibited clinically favorable features by achieving targeted exposure levels and the study met both its primary and secondary endpoints. We believe that results from our two BXCL501 clinical studies will lay a solid foundation to launch several Phase 3 pivotal trials across multiple neuro-psychiatric conditions with high unmet medical needs."

[1] PEC is a five-item scale that measures symptoms of agitation with each item rated from 1 (absent) to 7 (extreme).

[2] ACES is a one-item scale that measures overall agitation and sedation ranging from 1 (marked agitation) to 9 (unarousable).

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 shown anti-agitation effects in multiple clinical studies across multiple neuropsychiatric indications. A Phase 1 pharmacokinetic (bioavailability) and safety study of BXCL501 yielded positive top-line data. It is initially being evaluated for the acute treatment of agitation resulting from schizophrenia in a Phase 2 efficacy trial. The Company plans to unveil future development plans for BXCL501 in agitated dementia, opioid withdrawal symptoms and hyperactive delirium through 2019.

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 the 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, the commencement of clinical trials, the availability and results of data from clinical trials 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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