BioXcel Therapeutics Prepares to Initiate Pivotal Phase 3 Studies with BXCL501 Following Positive End-of-Phase 2 Meeting with FDA

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The Company plans to initiate the pivotal studies by year end, with topline data reported mid-2020

The first sublingual, non-invasive acute treatment candidate designed for agitation in neuropsychiatric diseases

NEW HAVEN, Conn., Dec. 03, 2019 (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 it has reached a general agreement with the U.S. Food and Drug Administration (FDA) on the key elements of the designs for its SERENITY (Sub-Lingual DExmedetomidine in Agitation Associated With SchizophRENIa and Bipolar Disorder STudY) program, two Phase 3 studies of BXCL501 for the acute treatment of agitation in patients with schizophrenia and bipolar disorder. Following the successful completion of its end-of-phase 2 meeting with the FDA, the Company plans to initiate the SERENITY studies by year end, with available topline results expected in mid-2020.

“Our pivotal Phase 3 studies are designed to support the regulatory approval of BXCL501 as a non-injectable, acute treatment for agitation in patients with schizophrenia and bipolar disorder,” commented Vimal Mehta, Chief Executive Officer of BTI. “There is significant need for an easy-to-use, safe and fast acting therapy for the 8 million people in the U.S. that suffer from agitation associated with neuropsychiatric diseases. We’re highly encouraged by our interactions with the FDA, and are one step closer to providing physicians with a potentially more effective and easier-to-use therapy to help safely manage their patients.”

The SERENITY studies closely follow the Company’s successful Phase 1b trial in 135 schizophrenia patients, where there were statistically significant, clinically meaningful rapid and durable reductions in PEC score at 120 and 180 microgram doses and acceptable safety profile. For the Phase 3 program, which will take place in many of the centers that participated in the Phase 1b trial, there will be two randomized, double-blinded, placebo-controlled, adaptive trials of up to 750 patients 18 to 75 years of age. SERENITY I will enroll patients with agitation associated with schizophrenia, with each arm receiving either BXCL501 at 120 micrograms, 180 micrograms, or placebo. SERENITY II will evaluate patients with agitation associated with bipolar disorder, also with each of three arms receiving either BXCL501 at 120 micrograms, 180 micrograms, or placebo.

The primary endpoint of both trials will be the reduction of symptoms of acute agitation in patients with schizophrenia and bipolar disorder using the Positive and Negative Syndrome Scale, measuring the Excited Component (PEC) change from baseline compared to placebo. The key secondary endpoint includes determining the earliest time where an effect on agitation is apparent as measured by the change from baseline in PEC total score.

About Agitation in Neuropsychology

Agitation is a common and costly (approximately $40 billion per year health care burden) symptom associated with a number of psychiatric conditions, including schizophrenia and bipolar disorder. It is estimated that approximately 19 million people are at risk of agitation and 8.3 million in the U.S. suffer from agitation each year. Early identification and prompt intervention to relieve agitation are essential to avoid symptomatic escalation and emergence of aggression. Recent consensus guidelines emphasize the need for non-coercive management strategies to protect the therapeutic alliance between patients and their healthcare providers—an alliance that is critical for the effective management of chronic psychiatric conditions. A non-invasive therapy that causes rapid symptom relief and de-escalates agitation is necessary to avoid the costly and traumatic use of coercive techniques, like physical restraint and seclusion, which require admission and prolonged hospitalization.

About the PEC (PANSS-EC or the Positive and Negative Syndrome Scale-Excitatory Component) Score for Agitation

The PEC score is a validated regulatory endpoint for measuring acute agitation in schizophrenia and bipolar patients. This scale is used in clinical research to rate the severity of a patient’s acute agitation. The PEC score is comprised of 5 elements associated with agitation: poor impulse control, tension, hostility, uncooperativeness, and excitement; each scored 1 (minimum) to 7 (maximum). The PEC is the sum of these 5 subscales and thus ranges from 5 to 35.

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 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. BTI plans to initiate by year end the SERENITY (Sub-Lingual DExmedetomidine in Agitation Associated With SchizophRENIa and Bipolar Disorder STudY) program, which will evaluate BXCL501 for the acute treatment of agitation resulting from schizophrenia and bipolar disorder. The Company also plans to initiate a Phase 1b/2 study of BXCL501 in patients with agitation associated with Dementia in the fourth quarter of 2019, with topline data expected in the first half of 2020.

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 evaluated 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 in development for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an investigational orally administered systemic innate immunity activator in development 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 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 and the Company’s future growth and position to execute on key milestones. 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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