BioXcel Therapeutics Announces Completion of Patient Enrollment in Phase 3 SERENITY Trials with BXCL501

May 14, 2020

Topline results, expected in July of 2020, are intended to support a New Drug Application filing

NEW HAVEN, Conn., May 14, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (“BTI” or “Company”) (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology, today announced full enrollment of its pivotal Phase 3 SERENITY trials evaluating BXCL501 for the acute treatment of agitation in patients with schizophrenia and bipolar disorder. The Company expects to report topline data from both trials in July of 2020.

“Completing patient enrollment in our two Phase 3 studies marks a significant milestone for BTI, and lays the foundation for future growth as we look to pursue clinical trials for several additional indications for BXCL501,” commented Vimal Mehta, Ph.D., Chief Executive Officer of BTAI. “The BTI team has remained focused, with our clinical programs on track despite the ongoing COVID-19 pandemic. We’re looking forward to reporting results from the two SERENITY registration trials, which we expect will be in July, to be followed by a potential New Drug Application submission in early 2021.”

The SERENITY studies are randomized, double-blinded, placebo-controlled, adaptive trials of up to 750 patients, 18 to 75 years of age. SERENITY I is evaluating patients with agitation associated with schizophrenia, with each of three arms receiving BXCL501 at 120 micrograms, 180 micrograms or placebo, respectively. SERENITY II is evaluating patients with agitation associated with bipolar disorder, also in three arms, receiving BXCL501 at 120 micrograms, 180 micrograms or placebo, respectively. The primary endpoint of the trials is reducing acute agitation measured by the Positive and Negative Syndrome Scale, examining the Excited Component (“PEC”) change from baseline compared to placebo. A 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 difficult to manage 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, costing approximately $40 billion annually in treatment related expenses. 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 may be necessary to avoid the costly and traumatic use of coercive techniques, like physical restraint and seclusion, which require admission and prolonged hospitalization.

About BXCL501

BXCL501 is an investigational 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 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 in patients with schizophrenia 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 (TRANQUILITY) for the treatment of agitation associated with dementia, and the Company is preparing to initiate a Phase 1b/2 study (RELEASE) of BXCL501 for the treatment of opioid withdrawal symptoms.

About BioXcel Therapeutics, Inc.:

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes 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, an investigational 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 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; undesirable side effects caused by BTI's product candidates; 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; impacts from the COVID-19 pandemic; 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 March 31, 2020, 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 the Investor page of our 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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