BioXcel Therapeutics Receives FDA Clearance of IND for Phase 2 Trial with BXCL501 for the Treatment of Agitation Associated with Delirium

October 26, 2020

Fifth potential indication for BXCL501, an orally dissolving thin film

NEW HAVEN, Conn., Oct. 26, 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 that its Investigational New Drug (“IND”) application for BXCL501, the Company’s novel, proprietary, orally dissolving, sublingual thin film formulation of dexmedetomidine (“Dex”), has received clearance by the Division of Psychiatry Products at the U.S. Food and Drug Administration (“FDA”) for the treatment of agitation associated with delirium. The Company plans to initiate a Phase 2 trial within the next several months.

“We are pleased to continue pursuing additional neuroscience opportunities with BXCL501, targeting agitation associated with delirium, a fifth potential indication for this candidate and a condition for which there is no FDA-approved treatment,” commented Vimal Mehta, Chief Executive Officer of BTI. “Agitation associated with delirium is commonly seen in numerous hospital settings, resulting in serious medical complications and extended hospital stays. Treatment choices are limited, and commonly used off-label therapies are not always effective or may result in prolonged, deep sedation. We believe that BXCL501’s unique mechanism of action, rapid onset, and ease of administration may quickly and directly treat the underlying agitation without over sedation, improving patient outcomes. In addition, this indication offers synergy with the commercial infrastructure being developed to support our first New Drug Application.”

This trial is a multicenter, randomized, double-blind, placebo-controlled, ascending, dose-finding, adaptive Phase 2 study designed to evaluate the safety, pharmacokinetics and efficacy of BXCL501 in intensive care unit patients experiencing agitation associated with delirium, including COVID-19 patients. Approximately 20 patients will be randomized into each sequential ascending dose cohort of BXCL501 (starting doses of 120 ug, 180 ug, 240 ug, or 300 ug), or matching placebos to determine the optimal starting dose that effectively and safely reduces agitation. Elderly delirium patients (65 years or older) in these cohorts will receive half the dose. The primary endpoint is the reduction in agitation measured by at least a 2-point drop in the Richmond Agitation Sedation Scale (“RASS”) at two hours post BXCL501 administration. The secondary endpoint is the earliest time at which a 2-point drop is seen in RASS after BXCL501 administration. An exploratory endpoint of this trial will be to determine the overall clinical improvement after drug administration using the Clinical Global Impression – Improvement Scale (“CGI-I”).

“Agitated patients with delirium are unable to calm themselves, rest or sleep, and often self-extubate, remove catheters and IV lines, complicating the overall patient care,” explains Dr. E. Wesley Ely, co-director of Vanderbilt University Medical Center’s Critical Illness, Brain Dysfunction, and Survivorship (“CIBS”) Center and study principal investigator. “The critical care world desperately needs a safer and more reliable way to relieve the agitation associated with delirium than the use of benzodiazepines, which surged during the COVID-19 pandemic. Patients deserve well-designed investigations of new approaches like the one we plan to conduct with BXCL501.”

About Agitation Associated with Delirium:

Delirium is a serious condition that occurs in a variety of hospital settings, including frequently in the intensive care unit. This condition may be caused by numerous underlying pathologic processes and disease states. Delirium is known to cause public health burden due to extended hospital stays, medical complications, increased financial costs and increased mortality. Agitation associated with delirium occurs in the majority of patients with this condition. Agitated patients with delirium are unable to calm themselves, rest or sleep and often self-extubate, remove catheters and IV lines thus complicating the overall patient care. With no FDA-approved treatments for this condition, current guidelines recommend sedative medications to maintain a light level of sedation in adult patients, which is frequently not achieved with commonly used therapies. A therapy that quickly and effectively reduces agitation, without causing excessive sedation, is needed to speed up recovery time and improve patient outcomes.

About the Richmond Agitation Sedation Scale (“RASS”)

The most commonly used and recommended instrument for agitation assessment in the ICU is the Richmond Agitation-Sedation Scale (“RASS”). The Richmond Agitation–Sedation Scale was developed in a collaborative effort with practitioners representing critical care physicians, nurses, and pharmacists and its validation and reliability is well documented. RASS is a 10-point scale, with four levels of anxiety or agitation (+1 to +4), one level to denote a calm and alert state (0), and 5 levels of sedation (-1 to -5). On one end of the RASS score, +4 represents a very combative, violent patient, who is dangerous to the staff. On the other end, -5 represents a patient who is unarousable, with no response to voice or physical stimulation.

About BXCL501

BXCL501 is an investigational, proprietary, orally dissolving, sublingual thin film formulation 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 disorders. BXCL501 has been granted Fast Track Designation by the U.S. Food and Drug Administration for the acute treatment of agitation in patients with schizophrenia, bipolar disorders and dementia. BXCL501 is being evaluated in a Phase 1b/2 trial (TRANQUILITY) for the treatment of agitation associated with dementia, and in a Phase 1b/2 study (RELEASE) for the treatment of opioid withdrawal symptoms. The Company also plans to initiate a Phase 2 trial in hospitalized patients suffering from agitation associated with delirium within the next several months.

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 in patients suffering from agitation associated with delirium. 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.

These forward-looking statements are based on management’s current expectations and beliefs. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause BTI’s actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: 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 June 30, 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 Investors sections 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 its 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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