



BioXcel Therapeutics Announces First Patient Enrolled in Phase 1b/2 RELEASE Trial of BXCL501 for the Treatment of Opioid Withdrawal Symptoms

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Topline results expected in the first quarter of 2021

NEW HAVEN, Conn., June 11, 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 the first patient has been enrolled in the Phase 1b/2 RELEASE trial of BXCL501, the Company's proprietary sublingual thin-film formulation of dexmedetomidine ("Dex"), for the treatment of opioid withdrawal symptoms.

"The initiation of the RELEASE trial is a major milestone for our neuroscience program, as we believe it not only showcases BXCL501's promise across multiple indications, but also supports this candidate's potential as a sub-chronic treatment," commented Vimal Mehta, Ph.D., Chief Executive Officer of BTI. "As the leading cause of death in the U.S. for individuals under the age of 50 years old, opioid abuse is a widespread problem that has been heightened by the COVID-19 pandemic. In order to avoid the painful symptoms associated with opioid withdrawal, individuals often continue to abuse these drugs or relapse, highlighting the need for better treatments to halt the cycle of addiction. After witnessing promising results with intravenous Dex, we believe BXCL501 may offer advantages over the current standard of care, as well as provide patients with a non-opioid therapy to alleviate the physiological symptoms of withdrawal. We look forward to reporting topline results from the study in the first quarter of 2021."

The RELEASE trial is a multicenter, randomized, double-blind, placebo-controlled, ascending-dose Phase 1b/2 study designed to evaluate the safety, pharmacokinetics, tolerability and efficacy of BXCL501 in patients experiencing symptoms of opioid withdrawal. This study will enroll approximately 125 subjects with opioid use disorder who are physically dependent on opioids. During the 7-day treatment phase, BXCL501 will be evaluated in sequential, ascending dose cohorts of 30ug, 60ug, 90ug, 120ug, and 180ug, administered twice daily (BID), approximately 12 hours apart. The study will assess opioid withdrawal symptoms using both the Clinical Opiate Withdrawal Scale (COWS) and Short Opiate Withdrawal Scale of Gossop (SOWS-GOSSOP) over a 10-day period.

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 clinical studies across multiple neuropsychiatric indications. BXCL501 has also been granted Fast Track Designation by the U.S. Food and Drug Administration for the acute treatment of mild to moderate agitation in schizophrenia, bipolar disorder, and dementia.

A Phase 1b safety and efficacy study of BXCL501 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 the Phase 1b/2 TRANQUILITY trial for the treatment of agitation associated with dementia, as well as the Phase 1b/2 RELEASE trial for the treatment of opioid withdrawal symptoms.

About Opioid Drug Withdrawal:

According to the Centers for Disease Control and Prevention ("CDC"), the misuse of and addiction to opioids is a serious national crisis and is the leading cause of death in the U.S. for those under 50 years old. Between 1999-2017, almost 400,000 people died from an overdose involving an opioid, with greater than 47,000 deaths occurring in 2017 alone. The CDC estimates the total "economic burden" of prescription opioid misuse alone in the U.S. is \$78.5 billion a year, including the costs of healthcare, lost productivity, addiction treatment and criminal justice involvement. Opioid withdrawal is a condition characterized by symptoms such as anxiety, agitation, sleep problems, muscle aches, runny nose, sweating, nausea, vomiting, diarrhea and drug craving — that occur after stopping or reducing the use of opioids in anyone with physical dependence on opioids.

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.bioxccltherapeutics.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 potential for BXCL501 to be used as a sub-chronic treatment. 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 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 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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