BioXcel Therapeutics Announces FDA Clearance of IND Application for BXCL501 for the Treatment of Opioid Withdrawal Symptoms

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Company is preparing to initiate Phase 1b/2 RELEASE trial

NEW HAVEN, Conn., Feb. 05, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (“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 that its Investigational New Drug (“IND”) application for BXCL501, the Company’s proprietary sublingual thin-film formulation of dexmedetomidine (“Dex”), has received clearance from the U.S. Food and Drug Administration (“FDA”) for the treatment of opioid withdrawal symptoms.

“The FDA clearance of our IND application for opioid withdrawal, a fourth indication, is an important step in our plans to build a neuroscience franchise around the multiple therapeutic opportunities with BXCL501,” commented Vimal Mehta, Chief Executive Officer of BTI. “Opioid overdose is reported as the number one cause of death for those under 50 years old in the U.S., and the distressing and challenging symptoms that come with opioid withdrawal are a primary reason for relapse. There is an urgent need for better treatment options to help manage the debilitating withdrawal symptoms and aid this underserved population from continued opioid abuse. BXCL501, our investigational non-opioid therapy, may offer key advantages to treating symptoms due to its intrinsic potency and favorable delivery method. We believe this study will build on the encouraging results we observed in our intravenous (“IV”) Dex trial, which appeared effective in reducing opioid withdrawal symptoms.”

Opioid withdrawal is an emotional and physiological medical condition that may be driven by the excessive drive of noradrenergic neurons that originate from the locus coeruleus in the brainstem. BXCL501 selectively activates alpha-2a adrenergic receptors, which decreases excessive neuronal firing, alleviating the physiological symptoms of opioid withdrawal.

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 subjects with opioid use disorder who are physically dependent on opioids. Patients will be randomized into multiple dose cohorts of BXCL501, or matching placebo, administered twice daily for five days. The study will assess opioid withdrawal symptoms using both the Clinical Opiate Withdrawal Scale and Short Opiate Withdrawal Scale of 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 multiple 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 agitation.

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 a Phase 1b/2 trial for the treatment of agitation associated with dementia, and the Company is preparing to initiate the Phase 1b/2 RELEASE trial of BXCL501 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 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 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, pancreatic cancer and advanced solid cancers 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 effects of BXCL501 treatment on opioid withdrawal symptoms and the timing of 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; 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 and on the Company’s 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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