BioXcel Therapeutics Announces NIH NIDA Grant to Columbia University to Support Further Studies of BXCL501 (Sublingual Dexmedetomidine) for Treating Opioid Withdrawal

August 1, 2022

Multi-year-funded award to support a 160-patient, randomized-controlled study in patients undergoing opioid withdrawal treatment

Over 142 million opioid prescriptions dispensed in the U.S. in 2020[1]

More than 1.7 million people in the U.S. suffered from substance use disorders related to prescription opioid pain relievers in 2016[1]

NEW HAVEN, Conn., Aug. 01, 2022 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a commercial-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced that the National Institute on Drug Abuse, part of the National Institutes of Health (NIH), has awarded Columbia University a multi-year NIDA grant to fund clinical testing of BXCL501 (sublingual dexmedetomidine) as a potential treatment for opioid withdrawal. The initial grant provides approximately $3.3 million for the project from August 1, 2022 through July 31, 2024 to support a 4-arm Phase 2b study comparing BXCL501 180 mcg and 240 mcg BID to placebo and Lucemyra™, to be followed by approximately $4.5 million to support a registrational study upon the completion of certain milestones.

Opioid abuse has long been recognized as a national health crisis. More than 142 million opioid prescriptions were dispensed in the U.S. in 2020, according to the Centers for Disease Control and Prevention (CDC), which also estimates that 1.7 million people in the U.S. suffered from substance use disorders related to prescription opioid pain relievers in 2016. In addition, the drug overdose epidemic claimed an estimated 104,000 lives in the 12-month period ending in September 2021[1] and a White House Council of Economic Advisers recently assessed the cost of the opioid crisis at $1 trillion[2].

Columbia University enrolled patients in the RELEASE trial — a multicenter, randomized, double-blind, placebo-controlled, ascending dose Phase 1b/2 trial designed to evaluate the safety, pharmacokinetics, tolerability, and efficacy of BXCL501 administered twice daily for seven days — in patients experiencing symptoms of opioid withdrawal. In March 2021, BioXcel Therapeutics announced RELEASE top line results. BXCL501 was generally well tolerated, with no severe or serious adverse events reported across all doses evaluated (30 mcg, 60 mcg, 90 mcg, 120 mcg, 180 mcg and 240 mcg). After further post-hoc analysis, the 240mcg BID dose significantly reduced both subjective ratings of insomnia and clinician ratings of anxiety or irritability among enrolled patients.

"We are pleased to continue testing BXCL501 in patients with opioid withdrawal with support from the NIH," said Dr. Frances Levin, Professor of Psychiatry at Columbia University and Chief of the Division on Substance Use Disorders at New York State Psychiatric Institute/Columbia University, Multiple Principal Investigator of the study and Project Director for the grant.

Dr. Sandra Comer, Multiple Principal Investigator of the study and Professor of Neurobiology in the Department of Psychiatry at Columbia University, noted that "BXCL501 shows promise in treating opioid withdrawal symptoms, which may facilitate transition of patients with opioid use disorder to treatment medications such as buprenorphine and naltrexone."

"The NIDA/NIH grant to Columbia University provides important support for BXCL501's development as a potential for opioid treatment withdrawal, a significant national challenge," said Frank Yocca, Ph.D., Chief Scientific Officer of BioXcel Therapeutics. "We believe the results observed thus far in multiple dose regimens within the RELEASE trial provide valuable insights to support investigation across additional indications and treatment settings."

This research is supported by the National Institute on Drug Abuse of the National Institutes of Health under award number UG3DA056247. The content presented in this release is solely the responsibility of the authors and does not necessarily represent the official views of the National Institutes of Health.

About BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc. is a commercial-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. The Company'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. The Company's commercial product, IGALMI™ (developed as BXCL501) is a proprietary, sublingual film formulation of dexmedetomidine approved by the FDA for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. The safety and effectiveness of IGALMI has not been established beyond 24 hours from the first dose. BXCL501 is being evaluated for the acute treatment of agitation associated with Alzheimer's disease, and as an adjunctive treatment for major depressive disorder. The Company is also developing BXCL502 as a potential therapy for chronic agitation in dementia. Under its subsidiary, OnkosXcel Therapeutics, the Company is developing BXCL701, an investigational, orally administered, systemic innate immunity activator for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. 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 future clinical trial results, future funding provided by NIDA/NIH, the potential value of BXCL501 as a treatment option for opioid withdrawal symptoms, and the Company's future strategy for BXCL501 for the treatment of opioid

withdrawal symptoms and other indications. When used herein, words including “anticipate,” “will,” “plan,” “may,” “continue,” “intend,” “designed,” “goal” 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 the Company’s current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. The Company 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 IGALMI™, BXCL501, BXCL502 and BXCL701 and other product candidates; the Company has no experience in marketing and selling drug products; IGALMI™ or the Company’s product candidates may not be accepted by physicians or the medical community in general; 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 the Company’s product candidates; its novel approach to the discovery and development of product candidates based on EvolverAI; 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, 2022, 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 the Company 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 the Company’s views as of any date subsequent to the date of this press release.

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[1] Centers for Disease Control and Prevention

[2] White House Release March 28, 2022