BioXcel Therapeutics Announces Grant by U.S. Department of Defense to Evaluate BXCL501 in Patients Suffering from PTSD

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Grant will support clinical studies in patients with post-traumatic stress disorder related to alcohol and substance abuse

NEW HAVEN, Conn., Dec. 07, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (“BTI” or the “Company”) (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology, today announced that it will be collaborating with the VA Connecticut Healthcare System and the Yale University Medical School on a grant awarded from the U.S. Department of Defense’s (“DOD”) Congressionally Directed Medical Research Programs (“CDMRP”) to evaluate BXCL501 in patients suffering from post-traumatic stress disorder (“PTSD”) related to alcohol and substance abuse disorder (“ASUD”). BXCL501 is the Company’s proprietary, orally dissolving thin film formulation of dexmedetomidine (“Dex”) in late stage development for the treatment of agitation and opioid withdrawal symptoms.

The funding will be used for studies of patients diagnosed with PTSD related to ASUD and will be the first time the Company is investigating BXCL501 as a potential chronic treatment. Following an initial safety assessment that is being funded by the U.S. Department of Defense, a follow-on assessment study is anticipated that will include an evaluation of patients self-administering BXCL501 daily at-home over a 28 day period. Principal investigators will be Ismene Petrakis, M.D., Chief of Psychiatry Services at VA Connecticut Healthcare System and Professor of Psychiatry at Yale University School of Medicine, as well as John Krystal, M.D., Director of the Clinical Neuroscience Division of the National Center for PTSD of the Department of Veterans Affairs and the Robert L. McNeil, Jr. Professor of Translational Research, Chair of the Yale Department of Psychiatry.

“We’re pleased that the DOD has recognized the potential of BXCL501 to improve care for men and women suffering from PTSD related to alcohol and substance abuse,” commented Frank Yocca, Ph.D., Chief Scientific Officer of BTI. “For the first time, we will be exploring BXCL501 as a daily treatment to address a chronic disorder. We believe that this product candidate, when used chronically, has the potential to reduce and alleviate the frequent hyperarousal symptoms that individuals with PTSD can experience on a daily basis. The U.S. federal government’s support of these studies will help us achieve our goal of advancing the evaluation of BXCL501 in more patient populations where there is significant need for better treatments.”

Dr. Kerry Ressler M.D., Ph.D., Professor of Psychiatry, Harvard Medical School, and a leader in PTSD research, commented, “In anxiety-related disorders, such as PTSD, norepinephrine is a major driver of the stress-related agitation and nightmares that can result due to hyperarousal of the sympathetic nervous system. Dex is a full agonist with higher CNS penetration and higher intrinsic activity at the receptor compared to the other alpha-2 receptor agonists, including clonidine and guanfacine, that are only partial agonists. BXCL501 could be a valuable therapeutic option to reduce the hypersympathetic response in patients with PTSD and has the potential, upon further study and approval, of becoming a first line chronic treatment for PTSD patients.”

The views expressed in this news release are those of the author and may not reflect the official policy or position of the Department of the Army, Department of Defense, or the U.S. Government.

About BXCL501:

BXCL501 is an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine, a selective alpha-2a receptor agonist for the treatment of agitation and opioid withdrawal symptoms. BTI believes that BXCL501 directly targets a causal agitation mechanism, and the Company has observed anti-agitation effects in multiple clinical studies across several 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 has been studied in two Phase 3 trials (SERENITY I and II) for the acute treatment of agitation associated with schizophrenia and bipolar disorders. Also, the product is being evaluated in a Phase 1b/2 trial (TRANQUILITY) for the acute 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.

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 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, an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine for the treatment of agitation and opioid withdrawal symptoms, and BXCL701, an investigational, orally administered, systemic innate immunity activator in development 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 the timing and data from clinical development initiatives and trials for BXCL501, including studies involving patients suffering from PTSD related to ASUD. 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 September 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 the Investors section 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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