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# BioXcel Therapeutics Announces Award of Grant by U.S. Department of Defense's ("DoD") Congressionally Directed Medical Research Programs ("CDMRP") for Development of BXCL501

*Expands clinical development of BXCL501 program for treatment ofalcohol and substance use disorders ("ASUD"), related to post-traumatic stress disorder ("PTSD") and traumatic brain injury ("TBI")*

*CDMRP aims to fund the study and development of new medications to improve treatment outcomes for ASUD comorbidities associated with PTSD and TBI*

NEW HAVEN, Conn., Aug. 20, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI), a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology, today announced that the U.S. DoD's CDMRP has awarded a planning grant[1] as a part of its Alcohol and Substance Abuse Disorders Research Program ("ASADRP") related to the development of the Company's product candidate, BXCL501. The grant will support the development of a clinical study to evaluate the use of BXCL501 for the treatment of ASUD, particularly related to PTSD and TBI. This study will be led by principal investigator John Krystal, M.D., the Robert L. McNeil, Jr. Professor of Translational Research, the Chairman of the Yale Department of Psychiatry, the Chief of Psychiatry at Yale-New Haven Hospital and the Director of Clinical Neurosciences, National Center for PTSD.

The Company recently announced positive top-line results from its adaptive Phase 1b, randomized, double-blind, placebo-controlled, multi-center, U.S. trial, evaluating multiple doses of BXCL501, an investigational selective and potent alpha2-adrenergic receptor agonist, for acute treatment of agitation in 135 patients with schizophrenia. Based on these results, the Company is planning to initiate Phase 3 pivotal trials in Q4 2019. The results from this study of BXCL501 suggest that it may merit further evaluation in patients with ASUD correlated with PTSD and TBI, experiencing hyper-arousal and high sympathetic nervous system activity. BXCL501 may be evaluated for use as an adjunct therapeutic before, during or after exposure therapy ("ET"), a gold standard therapy for PTSD, for treatment of ASUD patients comorbid with PTSD or TBI.

"The awarding of this grant is an important step forward that will allow BTI to advance the development of BXCL501 as a potential adjunct therapy to treat ASUD especially related to PTSD and TBI," commented Dr. Krystal. "BXCL501's unique properties and mechanism of action make it a potentially promising choice for further clinical evaluation in this area of high

unmet medical need. I am excited to develop BXCL501 as a potential treatment option for this patient population."

"We are honored to be selected for this award from the U.S. DoD, which we believe provides an important validation for our lead neuroscience candidate, BXL501. This grant will enable us to advance the clinical development of BXCL501 a potential adjunct therapy for ASUD related to PTSD and TBI," said Frank Yocca, Ph.D., Chief Scientific Officer of BTI. "BioXcel Therapeutics is dedicated to finding a solution to this critical problem that faces our civilian and military populations alike. We look forward to strengthening our existing relationship with the DoD."

"Using exposure therapy as a standard treatment approach that can lead to the recollection of traumatic events leading to autonomic responses such as elevated heart rate, increased blood pressure, and other symptoms like gooseflesh, palpitations, sweating, hypervigilance, paranoia, irritability and hostility," commented Jeff Sabados, MBA, MPP, former Navy SEAL, and BTI Advisor. "Unfortunately, a number of veterans have described how these recurrent hyperarousal symptoms often result in even more alcohol and substance abuse, discontinuation from exposure treatment and disengagement from providers and healthcare. We believe that BXCL501, if successfully developed and approved, could make a major impact on the health and wellness of service members and veterans as an adjunct therapeutic to lower the autonomic responses and enable patients to better engage in the ET for PTSD."

**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 is currently being developed for agitation associated with schizophrenia and bi-polar disorders followed by Alzheimer's/dementia.

**About Dr. John Krystal:**

Dr. Krystal is the Robert L. McNeil, Jr. Professor of Translational Research and Professor of Psychiatry, Psychology and of Neuroscience; Chair of Department of Psychiatry and Chief of Psychiatry at the Yale-New Haven Hospital. He also serves as the Director of National Alcohol Abuse and Alcoholism Advisory Council Center for the Translational Neuroscience of Alcoholism; Director, Clinical Neuroscience Division, VA National Center for PTSD. He is a leading expert in the areas of alcoholism, post-traumatic stress disorder, schizophrenia, and depression and has been crucial in the discovery of the rapid antidepressant effects of ketamine in depressed patients. Dr. Krystal is a clinical advisor to the Company.

**About Alcohol and Substance Use Disorder (ASUD) comorbid with Post-traumatic Stress Disorder (PTSD) or Traumatic Brain Injury (TBI):**

The relationship between ASUD and PTSD is well documented, where up to one third of people who survive traumatic accidents or disasters report drinking problems. These accidents may include traumatic brain injury which results in physical, cognitive, and emotional symptoms that overlap with PTSD. Suffering from both ASUD and PTSD can

make both issues worse, thus treatment for alcohol and substance abuse must often be part of PTSD treatment. Pharmacotherapies for ASUD can have significant side effects including but not limited to nausea, vomiting, dizziness and abdominal pain. Therapies for PTSD include exposure therapy which involves re-experiencing the traumatic events and such therapy has high dropout rates. These issues limit the willingness of patients with ASUD or PTSD to seek treatment and limit compliance with treatment regiments. PTSD is a condition that is caused by either experiencing or witnessing a traumatic event such as military combat, vehicle accidents, assault, abuse, natural disasters, and others. It is characterized by flashbacks, nightmares, severe anxiety, and also uncontrollable thoughts about the event. TBI is a form of acquired brain injury, which occurs when a sudden trauma causes damage to the brain either externally or internally.

**About BioXcel Therapeutics, Inc.:**

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence approaches to identify and advance the next wave of medicines 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 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 Company’s clinical development initiatives and trials for BXCL501, its efficacy in treatment of ASUD, in particular related to PTSD and TBI, and the timing of Phase 3 pivotal 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 June 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.

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.

**Contact Information:**

BioXcel Therapeutics, Inc.
www.bioxceltherapeutics.com
The Ruth Group
Janhavi Mohite
646-536-7026
jmohite@theruthgroup.com

[1] Information about the funding opportunity pursuant to which the grant was awarded is available at https://pasa.rti.org/Resources/RFA4/PASA_SRPP_RFA4a_Planning_Grant_FINAL.pdf.

Source: BioXcel Therapeutics, Inc.