BioXcel Therapeutics Announces Extension of FDA Review Period of its NDA for BXCL501 for the Acute Treatment of Agitation Associated with Schizophrenia and Bipolar Disorders

December 1, 2021

PDUFA date extended by three months to April 5, 2022

No Additional Data Requested Following Meeting with FDA

NEW HAVEN, Conn., Dec. 01, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) date for its review of the New Drug Application (NDA) of BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders I and II.

In connection with the FDA's ongoing review, BioXcel Therapeutics responded to the agency's information request pertaining to analyses of clinical data, and was recently informed the application would require additional time for review. As a result, the FDA extended the previously disclosed PDUFA date of January 5, 2022 to April 5, 2022. BioXcel Therapeutics and the FDA met on November 30. No additional data has been requested.

"Following our meeting with the FDA, we remain committed to working closely with the agency to facilitate a review of our NDA for BXCL501 and excited about making this potential therapy available to patients and caregivers as soon as possible," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "As we prepare for anticipated FDA approval, we are confident in BXCL501's broad potential to treat the millions of patients suffering from agitation associated with schizophrenia and bipolar disorders."

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 associated with neuropsychiatric disorders. BioXcel believes that BXCL501 potentially targets a causal agitation mechanism, and the Company has observed anti-agitation results in multiple clinical studies across several neuropsychiatric disorders, including schizophrenia related agitation (SERENITY I), bipolar disorder related agitation (SERENITY II) and dementia related agitation (TRANQUILITY). BXCL501 has been granted Breakthrough Therapy designation for the acute treatment of agitation associated with dementia and Fast Track designation for the acute treatment of agitation associated with schizophrenia, bipolar disorders and dementia. The safety and efficacy of BXCL501 has not been established.

About Schizophrenia and Bipolar Disorder Related Agitation

Agitation is a common and difficult to manage symptom associated with multiple neuropsychiatric conditions, including schizophrenia and bipolar disorders I and II. These two disease states alone have an estimated U.S. prevalence of approximately 9 million adults with more than 3 million experiencing agitation each year. On average, patients with these conditions experience more than a dozen episodes per year, the majority requiring pharmacologic treatment. Early identification and prompt intervention to relieve agitation are essential to avoid symptomatic escalation and the emergence of aggression. Expert consensus best-practice guidelines have recommended that agitation should be treated by a combination of behavioral calming techniques, verbal de-escalation, and medications that are voluntarily accepted by patients without coercion, with the pharmacologic goal of "calming without excessive sedation." A non-invasive therapy that causes rapid and sustained symptom relief may be helpful to avoid the costly and traumatic use of coercive techniques, like physical restraint and seclusion, which may result in admission and prolonged hospitalization.

About BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc. is a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. BioXcel'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. BioXcel Therapeutics' two most advanced clinical development programs are BXCL501, an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine for the treatment of agitation associated with psychiatric and neurological disorders, 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 anticipated commercial approval of BXCL501 for the acute treatment of schizophrenia and bipolar disorders and the potential value of BXCL501 as a treatment option. 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 BXCL501, BXCL502 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 the Company'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, 2021, 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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