# BioXcel Therapeutics Announces FDA Acceptance for Filing of NDA for BXCL501 for the Acute Treatment of Agitation Associated with Schizophrenia and Bipolar Disorders I and II

May 19, 2021

*FDA sets PDUFA action date for January 5, 2022*

*If approved, BXCL501 would represent the first major advancement in the acute treatment of agitation associated with schizophrenia and bipolar disorders in almost a decade*

NEW HAVEN, Conn., May 19, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BioXcel" or the "Company") (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 accepted for filing the New Drug Application (“NDA”) for BXCL501, the Company’s proprietary, investigational, orally dissolving thin film formulation of dexmedetomidine, for the acute treatment of agitation associated with schizophrenia and bipolar disorders I and II. The FDA has assigned a Prescription Drug User Fee Act (“PDUFA”) target action date of January 5, 2022. At this time, the FDA is not planning to hold an advisory committee meeting to discuss the application.

“The filing of our NDA marks an important milestone toward our goal to provide a new treatment option for the millions of patients with schizophrenia and bipolar disorders struggling with acute agitation,” said Vimal Mehta, Chief Executive Officer of BioXcel. “We believe that BXCL501, if approved, would represent a significant improvement in the care and management of agitation in these patients, potentially easing the burden for physicians and allied caregivers. While the FDA reviews our application, we will continue to execute on our comprehensive commercial strategy to ensure we are well positioned for potentially bringing BXCL501 – which is designed to address an important unmet need – to both patients and health care providers across the U.S.”

The application is supported by data from two randomized, double-blinded, placebo-controlled, parallel group Phase 3 studies (SERENITY I & SERENITY II) of BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders I and II, respectively. In both trials, BXCL501 was well-tolerated and met the primary and secondary endpoints at the 120 mcg and 180 mcg doses, demonstrating statistically significant, rapid and durable improvements from baseline versus placebo across multiple agitation scales.

**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 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. 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 Company recently received acceptance of its New Drug Application for BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders. The safety and efficacy of BXCL501 has not been established.

**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’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 of FDA’s review of BioXcel’s NDA submission, the potential commercialization of BXCL501 and BXCL501’s ability to treat those suffering from agitation associated with schizophrenia and bipolar disorders I and II. 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.

These forward-looking statements are based on management's current expectations and beliefs. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause BioXcel's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: 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 BioXcel'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 March 31, 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 and Investors sections 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 BioXcel 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 its views to change. These forward-looking statements should not be relied upon as representing BioXcel's views as of any date subsequent to the date of this press release.

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