March 11, 2021

BioXcel Therapeutics Submits New Drug Application to U.S. Food and Drug Administration for BXCL501 for the Acute Treatment of Agitation Associated with Schizophrenia and Bipolar Disorders

First New Drug Application for the Company's lead neuroscience program

NEW HAVEN, Conn., March 11, 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 Company has completed the rolling submission of its New Drug Application ("NDA") to the U.S. Food and Drug Administration ("FDA") for BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders I and II. BXCL501 is the Company's proprietary, investigational, orally dissolving thin film formulation of dexmedetomidine ("Dex"). The FDA has a 60-day filing review period to determine whether the NDA is complete and acceptable for filing.

"This submission is a significant achievement for BioXcel, having taken this program from its first-in-human trial to NDA submission in just over 2 years," commented Vimal Mehta, Chief Executive Officer of BioXcel. "BXCL501 was discovered using our innovative AI platform and we believe, if approved, could provide health professionals and patients with a fast acting, orally dissolving treatment option. We look forward to hearing back from the FDA and continuing with our commercial preparations to potentially bring a novel product that is designed to treat agitation associated with schizophrenia and bipolar disorders I and II to the U.S. market."

The submission 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 studies, BXCL501 was well tolerated and met the primary and secondary endpoints at the 120 mcg and 180 mcg doses, showing statistical significance versus placebo in mean change 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. 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 schizophrenia related agitation and bipolar disorder related agitation, respectively, and in a Phase 1b/2 trial (TRANQUILITY) for the acute treatment of dementia related agitation. This product candidate is also currently being evaluated in a Phase 1b/2 trial (RELEASE) for the treatment of opioid withdrawal symptoms and in a Phase 2 trial (PLACIDITY) for the treatment of delirium related agitation.

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 clinical benefits of BXCL501 to treat agitation. 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 BioXcel's current expectations and various assumptions. BioXcel believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BioXcel 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 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 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 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 our 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.

Contact Information:

BioXcel Therapeutics, Inc.

www.bioxceltherapeutics.com

Investor Relations:

Mary Coleman
BioXcel Therapeutics, VP of Investment Relations
MColeman@bioxceltherapeutics.com
1.475.238.6837

John Graziano
Solebury Trout
jgraziano@soleburytrout.com
1.646.378.2942

Media:

Julia Deutsch
Solebury Trout
jdeutsch@soleburytrout.com
1.646.378.2967

Source: BioXcel Therapeutics