BioXcel Therapeutics Receives FDA Breakthrough Therapy Designation for BXCL501 for the Acute Treatment of Agitation Associated with Dementia

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Designation offers the potential for expedited development and review, highlighting the urgent need for new treatment options for dementia related agitation

NEW HAVEN, Conn., March 15, 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 BXCL501, the Company's investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine ("Dex"), has been granted Breakthrough Therapy designation from the U.S. Food and Drug Administration ("FDA") for the acute treatment of agitation associated with dementia. The Breakthrough Therapy designation is intended to expedite the development and review of certain product candidates designed to treat serious or life-threatening diseases or conditions, and the designation includes increased interaction and guidance from the FDA.

“Managing dementia related agitation, specifically in elderly patients, represents a significant challenge for physicians and caregivers, as there are currently no FDA-approved therapies and off-label drugs come with black box warnings," stated Vimal Mehta, Chief Executive Officer of BioXcel. "The FDA's decision to grant Breakthrough Therapy designation further underscores the significant unmet need for a new treatment for this underserved patient population, as well as highlights BXCL501’s potential in becoming the first therapeutic option, if approved, to address this debilitating medical condition. We look forward to working closely with the FDA to advance BXCL501 into a pivotal dementia program, in hopes of quickly bringing this therapy to the millions of patients across treatment settings that lack alternative options.”

The Breakthrough Therapy designation for BXCL501 was supported by the positive topline data from the Phase 1b/2 TRANQUILITY study for the acute treatment of agitation associated with dementia, including Alzheimer's disease. BXCL501 demonstrated statistically significant reductions in agitation measures at 2 hours post-dose with both the 30 and 60 mcg doses as measured by multiple scales. The dose dependent response observed has the potential to support the Company’s plans to evaluate BXCL501 for use across the full range of dementia care settings.

About FDA Breakthrough Therapy Designation

Breakthrough Therapy designation is an FDA program intended to expedite the development and regulatory review of investigational therapies that are designed to address serious or life-threatening conditions. The criteria for Breakthrough Therapy designation requires preliminary clinical evidence that indicates that the candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. This designation provides the Company with more intensive FDA guidance on an efficient drug development program, and eligibility for other actions to expedite the FDA review, such as a rolling review of a New Drug Application (“NDA”), where the FDA may review sections of the NDA before the complete application is submitted. An NDA for a product candidate receiving breakthrough designation may also be eligible for priority review if the relevant criteria are met. Breakthrough Therapy designation does not change the standards for approval. For more information, please visit the FDA website at www.fda.gov.

About Dementia Related Agitation

Dementia is a neurocognitive condition caused by damage to brain cells that leads to a decline in cognitive abilities and independent function. It affects approximately 6 million individuals in the United States, with Alzheimer's disease accounting for up to 80% of these cases. During the course of the disease, patients with dementia often suffer from psychological and behavioral symptoms, such as agitation, which has been reported in up to 70% of patients. Agitation associated with dementia can negatively affect both the patient and caregiver's quality of life. Caregiver burden can contribute significantly to burnout, which can result in premature institutionalization of the patient. Treating agitation associated with dementia has been a challenge for providers as there are currently no FDA-approved therapies for the treatment of dementia-related agitation, and off-label therapies have black box warnings associated with their use.

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 potential for BXCL501 to treat dementia-related agitation and the timing of the planned pivotal Phase 3 trial of BXCL501 in dementia-related 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 Annual Report on Form 10-K for the year ended December 31, 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.

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