

# BioXcel Therapeutics Announces FDA Acceptance of IND for Lead Neuroscience Candidate, BXCL501 for the Acute Treatment of Agitation

*First-in-human pharmacokinetic (bioavailability) study with sublingual thin-film formulation  
Patient recruitment initiated and data read-out expected in 1H 19  
Results to support dose selection for registration trial expected to initiate in 2019*

NEW HAVEN, Conn., Dec. 12, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or "Company") (Nasdaq: BTAI), today announced that the U.S. Food and Drug Administration ("FDA"), has accepted its Investigational New Drug (IND) application for lead neuroscience candidate, BXCL501. BTI plans to evaluate BXCL501, a proprietary sublingual thin-film formulation of dexmedetomidine (Dex) in a first-in-human pharmacokinetic (bioavailability) and safety study in healthy volunteers. BTI is a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology.

Dr. Chetan D. Lathia, Senior Vice President & Head of Translational Medicine, Clinical Pharmacology & Regulatory Affairs of BTI commented "Clinical supplies with multiple dosing strengths of BXCL501 are now available to initiate clinical trials. With the FDA acceptance of the IND, we are on-track to dose subjects by year-end, with data expected in the first half of 2019. The results of this study will help in the determination of doses for our planned registration study."

Dr. Vimal Mehta, Chief Executive Officer of BTI added, "As a result of this IND acceptance, our second clearance this quarter, we have two active clinical studies for our lead programs in neuroscience and immuno-oncology. The initiation of these studies further validates our unique approach to drug development, which leverages the power of artificial intelligence to optimize research and development economics, improve development efficiency and speed to market. Given the ease of administration and other advantages of our proprietary sublingual thin-film formulation, we believe that BXCL501 has the potential to address the large unmet medical need and significant healthcare burden associated with the acute treatment of agitation."

The IND-opening Phase 1 study is a placebo-controlled, single-dose, dose-escalation study of BXCL501 that is expected to enroll up to 60 healthy adult volunteers across various dosing groups. The primary endpoints are pharmacokinetics and safety, with secondary endpoints including assessment of pharmacodynamics (PD) and the relationship between BXCL501 concentrations and PD endpoints. The Company expects to report top-line data from the study in the first half of 2019 that will provide a path for BTI to launch the anticipated registration studies.

BTI continues to explore expanding the range of target indications for BXCL501 beyond its current focus areas of acute treatment of agitation in schizophrenia, bipolar disorder and dementia. Treatment of agitation remains a significant global healthcare challenge in patients with drug and alcohol withdrawal, delirium and post-traumatic stress disorder, as the currently available treatment options are suboptimal, invasive, difficult to administer and often pose safety issues.

## **About BXCL501:**

BXCL501 is a first in class, proprietary sublingual film of dexmedetomidine, a selective alpha 2a receptor agonist intended for the treatment of acute agitation. BTI believes that BXCL501 directly targets a causal agitation mechanism and has demonstrated anti-agitation effects in preclinical and clinical studies. It has a well-established regulatory and reimbursement path for the acute treatment of agitation in schizophrenia and bipolar disorder, as demonstrated by Adasuve, a drug previously-approved by the FDA.

## **About Treatment of Agitation**

Agitation, including the acute treatment of agitation, remains a growing global healthcare burden. The Company estimates the total direct financial cost of all aspects of care for agitation in Alzheimer's disease to be approximately \$40 billion per year. The Company believes approximately 5.0 million patients with Alzheimer's disease, schizophrenia and bipolar disorder experience agitation in the U.S. Approximately 1.1 million of these patients experience mild to moderate agitation and represent a potential patient population for treatment with BXCL501.

**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 the next wave of medicines across neuroscience and immunology. 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, a sublingual thin film formulation designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immunology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer. For more information, please visit [www.bioxceltherapeutics.com](http://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, statements that relate to the advancement and development of BXCL501 and BXCL701, the commencement of clinical trials, the availability of data from clinical trials and other information that is not historical information. When used herein, words such as "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, market conditions and the factors described under the caption "Risk Factors" in BioXcel's Form 10Q for the period ending September 30, 2018, and BioXcel's other filings made with the Securities and Exchange Commission. Consequently, forward-looking statements should be regarded solely as BioXcel's current plans, estimates and beliefs. Investors should not place undue reliance on forward-looking statements. BioXcel cannot guarantee future results, events, levels of activity, performance or achievements. BioXcel does not undertake and specifically declines any obligation to update, republish, or revise any forward-looking statements to reflect new information, future events or circumstances or to reflect the occurrences of unanticipated events, except as may be required by law.

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