# BioXcel Therapeutics Advances Process Development for BXCL501 Thin Film Formulation

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*Automated, scalable process developed for manufacturing clinical and commercial supply*

BXCL501 thin film architecture is proprietary and flexible for combination therapies

*Multiple subjects treated with BXCL501 in ascending dose Phase 1 clinical trial*

*Appoints industry veteran Dr. Pascal Borderies as the Vice President, Commercial Development and Medical Affairs to lead commercialization strategy*

NEW HAVEN, Conn., May 02, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (“BTI” or “Company”) (Nasdaq: BTAI), today announced that the Company has finalized the formulation development for BXCL501 and has transitioned to automated manufacturing for its sublingual thin film containing dexmedetomidine (Dex). The manufacturing process scale-up is on track to support the Company’s Phase 3 clinical trial expected to be initiated in the second half of 2019. 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.

The Company is currently assessing the intended commercial formulation of BXCL501 in a Phase 1 pharmacokinetic (bioavailability) and safety study of multiple dosing strengths, with data expected in the near term. BTI’s proprietary sublingual thin film has been designed for both robustness and stability. The production of the sublingual thin film has been transitioned to an automated, robot-aided platform that is scalable and provides flexibility to produce a wide range of dose strengths. The manufacturing process and proprietary film structure are designed to allow for dosing flexibility and potential use in combination therapy.

Dr. David Hanley, Vice President and Head of Global Pharmaceutical Development and Operations commented, “We believe that we have optimized BXCL501 thin film properties for acute treatment of agitation across multiple disease states. We are excited to have transitioned to automated manufacturing for scale-up and initiation of Phase 3 clinical study and commercial supply.”

Dr. Vimal Mehta, Chief Executive Officer of BTI said, “We are pleased with the rapid advancement of the BXCL501 program as a potential non-invasive treatment of acute agitation in patients suffering from schizophrenia, bipolar disorder and Alzheimer’s disease. We anticipate that the upcoming results from our Phase 1 pharmacokinetic (bioavailability) and safety study will help establish a solid foundation as we work toward our goal of initiating Phase 2/3 trials and filing BTI’s first New Drug Application with the FDA in 2020.”

**BXCL501 Program Update:**
BTI has completed dosing subjects in a Phase 1 placebo-controlled, single dose, dose-escalation study of BXCL501. The clinical trial data is currently undergoing analyses with topline results expected to be announced shortly. The results are anticipated to encompass pharmacokinetics, safety, tolerability, and therapeutic exposures. The Company plans to initiate its first registration trial in the second half of 2019 based on findings from this study.

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

**BTI Management Update:**
Additionally, the company has appointed Dr. Pascal Borderies as the Vice President, Commercial Development and Medical Affairs. Dr. Borderies is responsible for designing and executing BTI’s global commercialization and medical affairs strategy, including sales and marketing. Dr. Borderies has more than 25 years of experience in commercial development and medical affairs at companies including, Novartis Consumer Health and Schering Plough, among others. Dr. Borderies holds a medical degree from the Paris 12 Val de Marne University School of Medicine.

Dr. Borderies commented, “I am thrilled to be joining the BioXcel Therapeutics leadership team to spearhead the Company’s commercialization and medical affairs initiatives. The BTI team has made remarkable progress in developing an innovative drug portfolio. I look forward to supporting the team to maximize the potential of the entire pipeline and bringing these therapies to the market.”

**About BXCL501:**
BXCL501 is a first in class, sublingual film of dexmedetomidine, a selective alpha 2a receptor agonist for the treatment of acute agitation. BTI believes that BXCL501 directly targets a causal agitation mechanism and using IV (intravenous) Dex has demonstrated anti-agitation effects in both preclinical and clinical studies. There is precedent for FDA approval and reimbursement of a non-invasive therapy for the acute treatment of agitation in patients with schizophrenia and bipolar disease, evidenced by regulatory approval of Adasuve, an inhaled version of the antipsychotic loxapine.

**About BioXcel Therapeutics, Inc.:**
BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence to identify the next wave of medicines across neuroscience and immuno-oncology. 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 neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed for treatment of a rare

form of prostate cancer and for treatment of pancreatic cancer in combination with other immuno oncology agents. 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, statements that relate to the advancement and development of BXCL501 and BXCL701, the commencement of clinical trials, the availability of data from clinical trials, BTI’s submission of its first New Drug Application with the FDA 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 BTI's current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BTI 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; it ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; 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; 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 fiscal year ended December 31, 2018 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 BTI 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 BTI’s views as of any date subsequent to the date of this press release.

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