BioXcel Therapeutics Provides Update on the Clinical Advancement of BXCL501 for the Acute Treatment of Agitation

October 30, 2018

On track to initiate BXCL501 first-in-human pharmacokinetic (bioavailability) and safety study

Data readout expected first half 2019

NEW HAVEN, Conn., Oct. 30, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI") (Nasdaq: BTAI), a clinical stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology, today provided an update on the Company's progress advancing its lead neuroscience program, BXCL501, a proprietary, first-in-class sublingual thin film formulation of dexmedetomidine hydrochloride ("Dex") being developed for the acute treatment of agitation.

BTI is on track to initiate an IND-opening Phase 1 pharmacokinetic ("PK") and safety study with BXCL501 by the end of this year, following approval of its IND application. Prior to its recently completed pre-IND meeting with the Food and Drug Administration ("FDA"), BTI submitted a pre-IND briefing book containing regulatory, chemistry, manufacturing, controls ("CMC") and non-clinical work, along with a proposed clinical study synopsis and a broader development plan. During the pre-IND meeting with the FDA, BTI received valuable feedback that will help guide further clinical development of BXCL501.

Vimal Mehta, Ph.D., Chief Executive Officer of BTI commented, "We have made a number of advancements in the development of BXCL501 including the recent successful completion of CMC. The planned initiation of the first-in-human PK and safety trial of BXCL501, a proprietary sublingual film formulation of Dex, will be a significant milestone. We expect to report data from this study in the first half of 2019, laying the foundation for a registration trial that will follow."

Building on the positive data from the Phase 1 study of the intravenous ("IV") formulation of Dex in healthy middle-aged and elderly participants announced earlier this year, BTI expects to report top-line data from the ongoing study of IV Dex in patients with schizophrenia and senile dementia of the Alzheimer's type (SDAT) in the fourth quarter of this year.

BTI plans to explore expanding the range of target indications for BXCL501 outside its current focus areas. Treatment of agitation remains a significant global healthcare challenge in patients with drug & alcohol withdrawal, delirium and post traumatic stress disorder ("PTSD"), as the currently available treatment options are suboptimal, invasive, difficult to administer and often pose safety issues.

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 has demonstrated anti-agitation effects in preclinical and clinical studies. It has a well-established regulatory and reimbursement path in schizophrenia and bipolar disorder, as demonstrated by a previously-approved drug, Adasuve.

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 1.2 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 immuno-oncology. The Company'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. The Company's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation of Dex designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology 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.

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 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 10-Q for the Quarter ended June 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

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