



BioXcel Therapeutics Reports Positive Results from Phase 1b Trial of Intravenously-dosed Dexmedetomidine Supporting BXCL501 Development

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Optimal exposure levels for developing BXCL501 sublingual thin film identified

Results support clinical evaluation of dexmedetomidine in acute treatment of agitation resulting from neuropsychiatric disorders and dementia

BRANFORD, Conn., June 27, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI") (Nasdaq:BTAI), a clinical stage biopharmaceutical development company utilizing novel artificial intelligence to identify the next wave of medicines across neuroscience and immuno-oncology, today announced positive data from its Phase 1b study evaluating intravenous (IV) administration of dexmedetomidine (Dex). Data from this study will aid in establishing the optimal dose for BXCL501, a sublingual formulation of Dex, for the acute treatment of agitation.

Sheldon Preskorn, MD, a leading Psychiatric Drug Development Expert and BTI's Neuroscience Clinical Advisor, commented, "The development of a medication that treats agitation without the various safety concerns of existing options is a large unmet medical need – a topic addressed at a symposium¹ conducted on June 14th in New York City by the New York Academy of Sciences. The current Phase 1b study was designed to evaluate the safety and establish an optimal exposure range. This information will be used to determine the ideal dosing strategy for the continued clinical development of BXCL501."

BTI completed the first part of its Phase 1b, randomized, placebo-controlled, dose ranging, single-center study of the IV formulation of Dex in healthy middle-aged and elderly participants. The study enrolled 16 healthy volunteers aged 55-75. The primary endpoint of the study was the dose and drug exposure levels required to produce mild sedation, which can serve as a surrogate endpoint for treating agitation using the Richmond Agitation-Sedation Scale (RASS)² score. The goal of the study was to achieve mild sedation without any clinically meaningful cardiovascular side effects. In this study:

- Dose escalation was performed by infusing 0.1 mcg to 0.6 mcg of the IV formulation of Dex over 30 min.
- An optimal starting dose of 0.3 mcg was determined in cohort 1 and subsequently used in cohort 2.
- The IV formulation of Dex was found to achieve mild sedation or a RASS score of -1 in patients at a Dex exposure level without producing any clinically meaningful effects on blood pressure and/or heart rate.
- This effect was evident in 11/12 subjects on the IV formulation of Dex and occurred within 30 minutes of starting the dose which produced the desired effect. In contrast, a mild sedating effect was seen in only 1 out of 4 individuals on placebo. The mild sedative effects of the IV formulation of Dex persisted for 1.5-2 hours, a clinically relevant duration.
- Inter-individual variability of the IV formulation of Dex was limited by dose titration.
- The IV formulation of Dex was well tolerated.

"Treatment of agitation remains a significant global healthcare challenge in patients with various neuropsychiatric disorders and dementia, as the currently available treatment options are suboptimal, invasive, difficult to administer and often pose safety issues especially in the elderly. The positive data from this Phase 1b study of the IV formulation of Dex represents an important step forward in the clinical development of BXCL501," said Dr. Vincent J. O'Neill, Chief Medical Officer of BTI. "These results clearly demonstrate doses and exposure levels of the IV formulation of Dex that have calming effects in healthy subjects without clinically meaningful changes in blood pressure or heart rate. With these findings in hand, we plan to evaluate the selected dose and exposure levels identified in this ongoing Phase 1b study in patients with senile dementia of the Alzheimer type (SDAT)."

¹ Psychiatric Symptoms in Alzheimer's Disease and Dementia symposium

² RASS is a 10-point (+4 "combative" to -5 "unarousable") medical scale used to measure the agitation or sedation level of a patient.

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 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.

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 prospectus dated March 7, 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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