

January 3, 2019



BioXcel Therapeutics Reports Positive Human Proof-of-Concept Data for Acute Treatment of Agitation in Patients with Alzheimer's Disease

Primary endpoint (safety and tolerability) met, with clinical benefit observed in 7 of 10 patients

Proof-of-Concept established in agitated patients across multiple underlying disorders including Alzheimer's Disease, Schizophrenia, and Delirium

Trial data, coupled with upcoming results of pharmacokinetic (bioavailability) BXCL501 study to support anticipated registration trial in 2019

Appoints Industry Veteran Dr. Robert Risinger as Vice President, Clinical Development to lead BXCL501 development

Conference call to discuss results and BXCL501 program on January 3, 2019 at 11:30 ET

NEW HAVEN, Conn., Jan. 03, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI") (Nasdaq: BTAI) today announced proof-of-concept data from its Phase 1 study of IV (intravenous) dexmedetomidine (Dex) for acute treatment of agitation in patients with Senile Dementia of the Alzheimer's Type (SDAT). The positive data from this Phase 1 trial provides evidence to support the continued clinical development of BXCL501 for the acute treatment of agitation under the accelerated Fast Track regulatory process. Agitation is common across all severities of Alzheimer's Disease, with an increasing prevalence as the disease progresses¹. 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 SDAT trial met its primary endpoint by identifying a safe dose of IV Dex that produced a mild arousable sedation, defined by a RASS² (Richmond Agitation Sedation Scale) score of -1. Data from this study, along with data from previously completed Phase 1 studies of IV Dex in agitated patients with schizophrenia and healthy elderly volunteers, is valuable in determining the optimal dose of BXCL501, a sublingual thin film formulation of Dex, being developed for the acute treatment of agitation.

This study enrolled a total of 14 SDAT patients. Ten patients in the treatment arm received IV Dex therapy, while 4 patients received placebo. In accordance with study designs used in previous participant populations, Dex treatment was begun at 0.1 mcg/kg/h and dose escalation occurred every 30 minutes by increasing the infusion rate by 0.1 mcg/kg/h to a maximum infusion of 0.5 mcg/kg/h. Such dosing allowed for the efficient determination of the

optimal dose in each participant. The study demonstrated that 7 out of 10 patients in the treatment arm achieved arousable sedation (RASS score of -1), with only 1 of 4 patients in the placebo arm. The drug was well tolerated without any clinically significant adverse events.

Vincent O'Neill, MD, Chief Medical Officer of BTI, commented, "The results of this study further validate our belief that BXCL501 represents a product with the potential to treat acute agitation arising from a number of neuropsychiatric indications. IV Dex has now demonstrated that this selective and safe alpha 2a receptor agonist can benefit patients across multiple pathologies where acute agitation is an issue. We look forward to evaluating BXCL501, which recently received Fast Track designation from the U.S. Food and Drug Administration (FDA), in our ongoing first-in-human pharmacokinetic (bioavailability) study for which we expect top-line data in the first half of 2019."

Sheldon Preskorn, MD, Professor in the Department of Psychiatry at the University of Kansas School of Medicine-Wichita and a member of the Company's Neuroscience Clinical Advisory Board added, "The results of this study demonstrate the ability of Dex to produce a rapid calming effect via its novel mechanism of action and support its potential as a safe and efficacious treatment for acute agitation. An easy-to-administer thin film formulation of BXCL501 could provide a much-needed treatment for agitation in patients across a range of neurological and psychiatric disorders."

BXCL 501 Program Update:

BTI is currently dosing subjects in a Phase 1 placebo-controlled, single dose, dose-escalation study of BXCL501. The study 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 this study in the first half of 2019.

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 dementia. Treatment of agitation remains a significant global healthcare challenge in patients suffering 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.

BTI Team Update:

Additionally, the Company announced the appointment of Robert Risinger, MD, as Vice President Clinical Development. Reporting to Dr. Vincent O'Neill, Dr. Risinger will lead the clinical development of BXCL501. He joins BTI with more than 10 years of neuropsychiatric drug development experience at companies including Alkermes, Bristol-Myers Squibb, Johnson & Johnson and most recently NeuroRx Pharmaceuticals. Prior to his industry career, Dr. Risinger was an Assistant Professor of Psychiatry and Behavioral Medicine at Medical College of Wisconsin. He also served as a Major and Staff Psychiatrist in the U.S. Air Force. He holds an MD from the University of Pittsburgh School of Medicine and completed his Psychiatric Residency at Emory University School of Medicine. The hiring of Dr. Risinger brings BioXcel Therapeutics' total employees to nearly 20.

Dr. Risinger commented, "I am thrilled to join BTI at such a critical juncture. The data we have generated to-date is extremely compelling and suggests that BXCL501 has the potential to demonstrate a superior therapeutic profile and route of administration compared to any other alpha 2a receptor agonist. The data from this study, coupled with previously-generated data in healthy volunteers, as well as agitated patients with schizophrenia, provides a strong rationale to launch a potential registration trial of BXCL501."

¹ <https://institute.progress.im/en/content/alzheimer%E2%80%99s-disease-and-agitation>

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

Conference Call:

BTI will host a conference call today, January 3, 2019, at 11:30 a.m. Eastern Time to discuss the results of this study and BXCL501 Program. Interested investors can access the call by dialing 800-239-9838 in the U.S. and Canada, or 323-994-2093 internationally. The call, along with a slide presentation to accompany the call, will be available via a live, listen-only webcast at <http://public.viavid.com/index.php?id=132677>, and archived for 30 days. For those unable to participate, a replay of the call will be available until February 3, 2019. To access the replay, please dial 844-512-2921 in the U.S. and Canada, or 412-317-6671 internationally, and enter passcode 2933928.

About BXCL501:

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