BioXcel Therapeutics Announces Initiation of Phase 2 PLACIDITY Trial of BXCL501 for the Treatment of Delirium Related Agitation

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Topline results are expected in Q1 2022

NEW HAVEN, Conn., Feb. 25, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (“BioXcel” or the “Company”) (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced the initiation of the Phase 2 PLACIDITY trial of BXCL501, the Company’s investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine (“Dex”), for the treatment of delirium related agitation.

“The initiation of PLACIDITY marks an important milestone in our efforts to showcase BXCL501’s ability to calm patients struggling with delirium related agitation, our fifth potential indication for this candidate,” commented Vimal Mehta, Chief Executive Officer of BioXcel. “Treating agitation associated with delirium remains a challenge for healthcare workers and patients as there are no FDA-approved therapies and off-label treatments are suboptimal, resulting in serious medical complications and extended hospital stays. We believe BXCL501, if approved, would be a welcomed therapy option for the approximately 4 million U.S. patients suffering from delirium related agitation annually, and we look forward to reporting topline results in the first quarter of 2022. Moreover, since delirium occurs across treatment settings within a hospital, this potential indication would be synergistic with the commercial infrastructure we are currently building to support our first New Drug Application.”

The PLACIDITY trial is a multicenter, randomized, double-blind, placebo-controlled, ascending dose-finding, adaptive Phase 2 study designed to evaluate the safety, efficacy, and pharmacokinetics of BXCL501 in intensive care unit adult patients experiencing delirium related agitation, including COVID-19 patients. Approximately 20 patients will be randomized into each sequential ascending dose cohort of BXCL501 (starting doses of 120 ug, 180 ug, 240 ug, or 300 ug), or matching placebos to determine an optimal starting dose that could effectively and safely reduce agitation. Elderly delirium patients (65 years or older) in these cohorts will receive half the dose. The primary endpoint is the reduction in agitation measured by at least a 2-point drop in the Richmond Agitation Sedation Scale (“RASS”) at two hours post BXCL501 administration. The secondary endpoint is the earliest time at which a 2-point drop is seen in RASS after BXCL501 administration. An exploratory endpoint of this trial will be to determine the overall clinical improvement after drug administration using the Clinical Global Impression – Improvement Scale (“CGI-I”).

About Delirium Related Agitation:
Delirium is a serious condition that occurs in a variety of hospital settings, including frequently in the intensive care unit. This condition may be caused by numerous underlying pathologic processes and disease states. Delirium is known to cause public health burden due to extended hospital stays, medical complications, increased financial costs and increased mortality. Delirium related agitation occurs in the majority of patients with this condition. Agitated patients with delirium are unable to calm themselves, rest or sleep and often self-extubate, remove catheters and IV lines thus complicating overall patient care. With no FDA-approved treatments for this condition, current guidelines recommend sedative medications to maintain a light level of sedation in adult patients, which is frequently not achieved with commonly used therapies. A therapy that quickly and effectively reduces agitation, without causing excessive sedation, is needed to speed up recovery time and improve patient outcomes.

About the Richmond Agitation Sedation Scale (“RASS”)
The most commonly used and recommended instrument for agitation assessment in the ICU is the Richmond Agitation-Sedation Scale (“RASS”). The Richmond Agitation–Sedation Scale was developed in a collaborative effort with practitioners representing critical care physicians, nurses, and pharmacists and its validation and reliability is well documented. RASS is a 10-point scale that ranges from +4 to -5. There are four point levels to assess agitation (+4 to +1), a single point level to denote a calm and alert state (0), and five point levels to assess sedation (-1 to -5). On one end of the RASS score, +4 represents a very combative, violent patient, who is dangerous to the staff. On the other end, -5 represents a patient who is unarousable, with no response to voice or physical stimulation.

About BXCL501
BXCL501 is an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine, a selective alpha-2a receptor agonist for the treatment of agitation and opioid withdrawal symptoms. BioXcel believes that BXCL501 directly targets a causal agitation mechanism, and the Company has observed anti-agitation results in multiple clinical studies across several neuropsychiatric disorders. BXCL501 has been granted Fast Track Designation by the U.S. Food and Drug Administration for the acute treatment of agitation in patients with schizophrenia, bipolar disorders, and dementia. BXCL501 has been studied in two Phase 3 trials (SERENITY I and II) for the acute treatment of schizophrenia related agitation and bipolar disorder related agitation, respectively, and in a Phase 1b/2 trial (TRANQUILITY) for the acute treatment of dementia related agitation. This product candidate is also currently being evaluated in a Phase 1b/2 trial (RELEASE) for the treatment of opioid withdrawal symptoms and in a Phase 2 trial (PLACIDITY) for the treatment of delirium related agitation..

BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. is a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. BioXcel’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. BioXcel’s two most advanced clinical development programs are BXCL501, an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine for the treatment of agitation and opioid withdrawal symptoms, and BXCL701, an investigational, orally administered, systemic innate immunity activator in development for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. 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 the timing and data from the Phase 2 PLACIDITY trial for BXCL501, synergy of the delirium indication with the Company’s planned commercial structure, value of BXCL501 as a treatment option, and the Company’s planned new drug application. When used herein, words including “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, 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; the failure of preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; undesirable side effects caused by BioXcel’s product candidates; 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; impacts from the COVID-19 pandemic; its ability to commercialize its product candidates; and the other important factors discussed under the caption “Risk Factors” in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2020, 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 and the Investors section of our website at www.bioxceltherapeutics.com.

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 BioXcel 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 BioXcel’s views as of any date subsequent to the date of this press release.

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