BioXcel Therapeutics Announces Positive Results from Correlation Study Supporting SERENITY At-Home Exploratory Efficacy Outcomes

October 14, 2025

Completes clinical trials required by FDA for sNDA submission planned for the first quarter of 2026

NEW HAVEN, Conn., Oct. 14, 2025 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience, today announced positive results from the correlation study related to exploratory efficacy outcomes from the SERENITY At-Home trial. The results, along with the data from the SERENITY At-Home trial, will be included in the supplemental New Drug Application (sNDA) submission that is planned for the first quarter of 2026.

The standard method for measuring acute agitation associated with schizophrenia and bipolar disorder is the Positive and Negative Syndrome Scale – Excited Component (PEC) administered by a trained clinician, which was used in the Serenity I & II Pivotal Trials. In order to evaluate BXCL501 for continued clinical effect with repeat dosing in the at-home setting using an exploratory efficacy measurement, the Company, in consultation with FDA, developed the modified CGI-S (mCGI-S) scale, which can be scored by patients and/or caregivers. The study assessed the correlation between PEC and mCGI-S in this prospective, open label, in-clinic trial in 33 patients.

The results demonstrated a strong correlation between the clinician assessments and the patient or caregiver (informant) rated outcomes, providing support for using mCGI-S to assess efficacy in the outpatient setting. A statistically significant and strong correlation between the PEC and mCGI-S with a correlation of $\rho=0.89$; $p<0.0001$ for patients and $\rho=0.88$; $p<0.0001$ for informants was observed.

“We are pleased with the strong and significant correlation observed with clinician assessment for both patients and informants, providing support for our exploratory efficacy outcomes in the SERENITY At-Home study.” said Dusan Kostic, Ph. D. Senior Vice President Medical Affairs and Clinical Development. “We plan to include these findings as a part of our upcoming sNDA package.”

There were no serious adverse events reported and the safety profile remains consistent with the IGALMI® label.

Additional data and results will be presented at upcoming medical meetings and conferences.

About Modified Clinical Global Impression – Severity Scale (mCGI-S)
The original clinician rated CGI-S scale describes an 8-point rating of agitation symptoms where zero means there was no assessment performed and scores of 1-7 rate increasing severities of the symptoms being assessed. For ease of implementation by the patient and informant in the home setting this scale was modified in consultation with FDA. The modified CGI-S is a 4-point scale where 0 is no agitation, and scores of 1-3 describe increasing severities of agitation (mild, moderate, or severe).

About the Positive and Negative Syndrome Scale - Excited Component (PEC)
The PEC is a 5-item assessment for rating acute psychomotor agitation within the following domains: excitement, hostility, tension, uncooperativeness, and poor impulse control (Montoya et al. 2011). Each item utilizes a 7-point scale, with domain-specific descriptions of the signs the rater may observe for each severity rating: 1 = Absent, 2 = Minimal, 3 = Mild, 4 = Moderate, 5 = Moderate Severe, 6 = Severe, and 7 = Extreme. The total PEC score is the sum of the five domain scores, ranging from 5 to 35. A PEC score of 14 is the threshold needed to treat an acute agitation episode.

About BXCL501
Outside of its approved indication by the U.S. Food and Drug Administration as IGALMI® (dexmedetomidine) sublingual film, BXCL501 is an investigational proprietary, orally dissolving film formulation of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist. BXCL501 is under investigation by BioXcel Therapeutics for the acute treatment of agitation associated with Alzheimer’s dementia and for the acute treatment of agitation associated with bipolar I or II disorder or schizophrenia in the at-home setting. The safety and efficacy of BXCL501 for these investigational uses have not been established. BXCL501 has been granted Breakthrough Therapy designation by the FDA for the acute treatment of agitation associated with dementia and Fast Track designation for the acute treatment of agitation associated with schizophrenia, bipolar disorders, and dementia.

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. (Nasdaq: BTAI) is a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience. Its wholly owned subsidiary, OnkosXcel Therapeutics, is focused on the development of medicines in 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 indications. For more information, please visit bioxceltherapeutics.com.

Forward-Looking Statements

This press release includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements related to: the Company’s planned advancement of its SERENITY program; potential market opportunity for BXCL501; release of data from the SERENITY At-Home trial; the submission of an sNDA to the FDA; the supply of IGALMI® through existing distribution channels; the potential for the results from the Company’s

completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates and change the treatment paradigm for agitation. When used herein, words including “anticipate,” “believe,” “can,” “continue,” “could,” “designed,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “might,” “plan,” “possible,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. 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 the Company’s current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. The Company 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; the impact of the reprioritization; its significant indebtedness, ability to comply with covenant obligations and potential payment obligations related to such indebtedness and other contractual obligations; the Company has identified conditions and events that raise substantial doubt about its ability to continue as a going concern; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY program; its dependence on the success and commercialization of IGALMI®, BXCL501, BXCL502, BXCL701 and BXCL702 and other product candidates; the number of episodes of agitation and the size of the Company’s total addressable market may be overestimated, and approval that the Company may obtain may be based on a narrower definition of the patient population; its lack of experience in marketing and selling drug products; the risk that IGALMI® or the Company’s product candidates may not be accepted by physicians or the medical community in general; the Company still faces extensive and ongoing regulatory requirements and obligations for IGALMI®; 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 the Company’s product candidates; its novel approach to the discovery and development of product candidates based on EvolverAI; the significant influence of and dependence on BioXcel LLC; its exposure to patent infringement lawsuits; its reliance on third parties; its ability to comply with the extensive regulations applicable to it; impacts from data breaches or cyber-attacks, if any; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; risks associated with federal, state or foreign health care “fraud and abuse” laws; and its ability to commercialize its product candidates, as well as the important factors discussed under the caption “Risk Factors” in its Annual Report on Form 10-K for the fiscal year ended December 31, 2024, 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 the Company’s 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 the Company 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 the Company’s views as of any date subsequent to the date of this press release.

Contact Information

Corporate/Investors
Russo Partners
Nic Johnson
nic.johnson@russopartnersllc.com
1.303.482.6405

Media
Russo Partners
David Schull
david.schull@russopartnersllc.com
1.858.717.2310

Source: BioXcel Therapeutics, Inc.