BioXcel Therapeutics Announces FDA Closed its Inspection of Site for Phase 3 TRANQUILITY II Trial for Acute Treatment of Agitation Associated with Alzheimer's Dementia

March 3, 2025

FDA concludes site inspection is closed under 21 C.F.R.20.64(d)(3); Voluntary Action Indicated

Company believes this further supports reliability of data from TRANQUILITY II trial of BXCL501

NEW HAVEN, Conn., March 03, 2025 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience, today announced that the U.S. Food and Drug Administration (FDA) has concluded that the inspection of a single site in its TRANQUILITY II Phase 3 trial is closed under 21 C.F.R.20.64(d)(3) and released the Establishment Inspection Report. The FDA has designated "Voluntary Action Indicated" for the site.

"We believe this report and the closing of the investigation, together with the positive findings from the independent audit we announced in October 2023, further reaffirm the data integrity from the single site and the body of clinical evidence we intend to include in a potential sNDA submission," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "We have already received FDA feedback on the protocol for our TRANQUILITY In-Care Phase 3 trial and look forward to advancing this program with our lead neuroscience asset BXCL501."

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 the TRANQUILITY In-Care Phase 3 Trial

The TRANQUILITY In-Care trial is designed as a double-blind, placebo-controlled study to evaluate the efficacy and safety of a 60 mcg dose of BXCL501 over a 12-week period for agitation associated with Alzheimer's dementia in the care setting. The trial is expected to enroll approximately 150 patients 55 years and older who have mild, moderate, or severe dementia with mini-mental state examination scores of 0 to 25 and who reside in skilled nursing facilities, memory care units, or assisted living facilities. Patients will self-administer 60 mcg of BXCL501 or placebo when episodic agitation episodes occur over the trial period. The primary endpoint is expected to be a change from baseline in the Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score at two hours post-first dose. Additional PEC and Clinical Global Impressions – Improvement Scale measurements will also be obtained during the trial.

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: our submission of an sNDA for the treatment of agitation associated with Alzheimer's dementia in the care setting and advancing our Tranquility program and initiating our TRANQUILITY In-Care pivotal Phase 3 trial, and other statements that are not historical fact. 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 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
BioXcel Therapeutics
Erik Kopp
1.203.494.7062

Media
Russo Partners
David Schull
1.858.717.2310

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