BioXcel Therapeutics Announces Positive Findings from Independent Third Party Audit of Data Integrity at TRANQUILITY II Phase 3 Trial Site

October 25, 2023

No evidence of misconduct or fraud found beyond instance previously reported[1]

No findings identified that impact data integrity

Company believes audit findings support reliability of positive TRANQUILITY II trial data and potential sNDA submission

NEW HAVEN, Conn., Oct. 25, 2023 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience, today announced positive findings from an independent third party audit of the data from a single site[1] in its TRANQUILITY II Phase 3 trial.

Conducted by a well-regarded regulatory and quality consulting firm, the independent audit consisted of a comprehensive review of records from over 50% of subjects enrolled at the single trial site to identify any additional instance of misconduct or fraud[2] and to evaluate data integrity and reliability for eligibility, safety, and efficacy data. This sample size provides 95% confidence that the data reviewed is a representative sample. Following an extensive review, the team of auditors did not identify any findings that they believe impact the data reliability or integrity, nor did they find any evidence of additional misconduct or fraud. Based on these findings, BioXcel Therapeutics believes that the positive, statistically significant TRANQUILITY II trial data announced in June 2023 potentially support a supplemental new drug application (sNDA) for BXCL501 for the acute treatment of agitation associated with dementia in probable Alzheimer's disease.

"We believe these results of an audit by a respected, independent firm validate the integrity of data from the single site in question and add to the body of clinical evidence we intend to include in our sNDA submission," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "We recently had a Type B/Breakthrough meeting with the FDA to discuss our plans for the development of BXCL501 for the acute treatment of agitation associated with dementia in probable Alzheimer's disease. We expect to receive the FDA meeting minutes in the first half of November, and intend to provide an update on additional steps for the TRANQUILITY program and a potential sNDA in our upcoming third quarter financial results."

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. (Nasdaq: BTAI) is a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience. 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 and Disclaimers
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 (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements regarding: the expected receipt of the meeting minutes from FDA; additional steps for the TRANQUILITY program and to support an sNDA for BXCL501 for the acute treatment of agitation associated with dementia in probable Alzheimer's disease; the Company's advancement of its product candidates for regulatory approval; the representativeness of the patient data audited; and the potential for the results from the Company's completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates, including the results from the TRANQUILITY II clinical trial. The words "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; its ability to successfully negotiate amended terms under the financing agreements to be able to access funding and to obtain relief under financial covenants; its significant indebtedness and potential payment obligations related to such indebtedness and other contractual obligations; risks associated with the strategic reprioritization; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY II Phase 3 trial and related audit; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502, BXCL701 and BXCL702 and other product candidates; 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 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; impacts from the COVID-19 pandemic; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; 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 June 30, 2023, which are accessible on the SEC's website at www.sec.gov. 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.

The Company cannot guarantee that the FDA will accept or agree with the Company's or its auditors' conclusions or analyses, and the FDA may interpret or weigh their importance differently. Further, if the FDA determines that there are issues with data integrity and/or compliance with good clinical practice requirements in connection with the TRANQUILITY II trial, the Company may be unable to use some or all of the subject data generated to support a marketing application, which could result in inadequate powering of the TRANQUILITY II trial and failure to reach statistical significance.

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Source: BioXcel Therapeutics, Inc.

[1] For additional information, see the Company's Current Report on Form 8-K, filed with the Securities and Exchange Commission on June 29, 2023.

[2] For purposes of the audit, (1) misconduct was defined to mean the negligent or intentional non-compliance with relevant standards and regulations, or the deliberate or intentional disregard for the protocol, policies and procedures, and regulatory requirements or otherwise engaging in practices that seriously deviate from commonly accepted research principles and compromise the safety of subjects or the quality and/or integrity of data or documents generated in the study, to include falsification, fabrication, and forgery and (2) fraud was defined to mean the deliberate reporting of false or misleading data or information, or the deliberate withholding of reportable data.