BioXcel Therapeutics Announces Late-Breaking Abstract on Preliminary Findings from Phase 2 Investigator-Sponsored Trial of BXCL701 and KEYTRUDA® in Metastatic Pancreatic Ductal Adenocarcinoma (PDAC) Selected for Presentation at 2024 ASCO Annual Meeting

April 24, 2024

Poster presentation scheduled for June 1, 2024, 1:30-4:30 PM CT /2:30-5:30 PM ET

Trial being led by Georgetown University's Lombardi Comprehensive Cancer Center

NEW HAVEN, Conn., April 24, 2024 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology, today announced that a late-breaking abstract with preliminary findings from the Phase 2 investigator-sponsored trial of BXCL701 in combination with KEYTRUDA® (pembrolizumab) in previously treated metastatic pancreatic ductal adenocarcinoma (PDAC) has been selected for presentation in a poster session at the 2024 American Society of Clinical Oncology (ASCO) Annual Meeting. The meeting will take place from May 31 to June 4, 2024 in Chicago, IL.

The trial, which is led by investigators at Georgetown University's Lombardi Comprehensive Cancer Center, is evaluating BXCL701, an investigational, oral innate immune activator designed to inflame the tumor microenvironment and thereby augment the activity of checkpoint inhibitors. On February 6, 2024, BioXcel Therapeutics announced the completion of patient enrollment in the safety lead-in portion of the trial. Through its OnkosXcel Therapeutics immuno-oncology subsidiary, BioXcel Therapeutics is collaborating with Dr. Louis M. Weiner, director of the Lombardi Comprehensive Cancer Center, and Dr. Benjamin Weinberg, principal investigator of the study. BioXcel Therapeutics and Merck & Co. are providing BXCL701 and KEYTRUDA for the trial, respectively.

Poster Presentation*

Title: Phase 2 Trial of BXCL701 and Pembrolizumab in Patients with Metastatic Pancreatic Ductal Adenocarcinoma (EXPEL-PANC): Preliminary Findings

Presenter: Dr. Benjamin Weinberg, Ruesch Center for the Cure of Gastrointestinal Cancers, Lombardi Comprehensive Cancer Center

Abstract: LBA4132

Poster: 112

Session: Gastrointestinal Cancer—Gastroesophageal, Pancreatic, and Hepatobiliary

Date: June 1, 2024

Time: 1:30-4:30 PM CT /2:30-5:30 PM ET

*The poster abstract will be released at 7:00 AM CT /8:00 AM ET on the day of the presentation.

About BXCL701
BXCL701 is an investigational, oral innate immune activator designed to initiate inflammation in the tumor microenvironment. Approved and experimental immunotherapies often fail to address cancers that appear "cold." Therefore, BXCL701 is being evaluated to determine if it can render "cold" tumors "hot," making them more detectable by the adaptive immune system and thereby facilitating the development of a strong anticancer immune response. OnkosXcel Therapeutics' preclinical data support BXCL701's potential synergy with both current checkpoint inhibitors and emerging immunotherapies directed to activate T-cells. BXCL701 is currently being developed as a potential therapy for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. BXCL701 has received Orphan Drug Designation from the U.S. Food and Drug Administration in four indications: acute myelogenous leukemia, pancreatic cancer, stage IIb to IV melanoma, and soft tissue sarcoma. The U.S. Food and Drug Administration (FDA) designated as a Fast Track development program the investigation of BXCL701 in combination with a checkpoint inhibitor for treatment of patients with metastatic small cell neuroendocrine prostate cancer (SCNC) with progression on chemotherapy and no evidence of microsatellite instability. An 800+-subject clinical database, with data collected by the Company and others, supports the ongoing development of BXCL701.

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 (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, the Company's participation in and content of the Company's presentations at 2024 ASCO Annual Meeting and the potential benefits from treatment

with BXCL701. 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 and limited revenue generation; its incurrence of significant losses; its strategic reprioritization and related reduction in force may not achieve its intended outcome; its need for substantial additional funding and ability to raise capital when needed; 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 Company’s TRANQUILITY program; risks related to the limited clinical data supporting potential safety or efficacy of BXCL501 for use in the at-home setting; its dependence on the success and commercialization of IGALMI, BXCL501, BXCL502, BXCL701 and BXCL702 and other product candidates; interim “top-line” and preliminary data from its clinical trials may change and result in material changes in the final data; its ability to receive regulatory approval from the FDA and comparable foreign authorities for its product candidates; clinical trials are expensive, time-consuming, difficult to design, difficult to conduct, and involve an uncertain income; 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’s estimated number of episodes of agitation and its corresponding estimated total addressable market are subject to inherent challenges and uncertainties; 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 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; the Company is and may in the future be subject to legal proceedings, claims and investigations in or outside the ordinary course of business, which could be costly and time-consuming to defend and could result in unfavorable outcomes; risks related to unfavorable global political or economic events and conditions; 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, 2023, 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.

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