BioXcel Therapeutics Reports Positive Overall Survival Results from Single-Arm, Open-Label Phase 2 Trial of BXCL701 in Patients with Metastatic Castration-Resistant Prostate Cancer (mCRPC) of Adenocarcinoma Phenotype

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Median overall survival of 15.5 months with BXCL701 + KEYTRUDA® (pembrolizumab), compared to 9.6 months with checkpoint inhibitor monotherapy in late-line refractory patients in separate Phase 2 trial[1]

59% of studied patients alive at one year following treatment with BXCL701 + KEYTRUDA

Median progression-free survival of 4.2 months with BXCL701 + KEYTRUDA compared to 2.1 months with checkpoint inhibitor monotherapy in late-line refractory patients in separate Phase 2 trial[1]

Company plans to determine program development path following end of Phase 2 meeting with FDA scheduled for December

NEW HAVEN, Conn., Nov. 08, 2023 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology, today announced positive overall survival (OS) data from its Phase 2 trial of BXCL701, the Company's investigational oral innate immune activator, in combination with KEYTRUDA® (pembrolizumab) in patients with metastatic castration-resistant prostate cancer (mCRPC) of adenocarcinoma phenotype, the most common form of the disease. As of a September 6, 2023 data cutoff, evaluable patients with adenocarcinoma (n=29) showed a median OS of 15.5 months (95% CI 9.6–NR), and a 12-month survival rate of 59%.

“Patients with mCRPC who have failed androgen deprivation and taxane-based chemotherapy have few remaining treatment options and, unfortunately, KEYTRUDA to date has not shown additional benefit in this setting,” said Vincent J. O’Neill, M.D., Chief R&D Officer, OnkosXcel Therapeutics, a wholly owned subsidiary of BioXcel Therapeutics. “Therefore, we are highly encouraged by these combination data bearing in mind historical data with checkpoint inhibitor monotherapy. In addition, we now have a second positive dataset in a separate cold tumor histology, further increasing our belief that BXCL701 has the potential to inflame the tumor microenvironment of cold tumors, thereby sensitizing them to checkpoint inhibition. We believe the data warrant further evaluation of BXCL701 in this setting and look forward to determining the development path for this program following our end of Phase 2 meeting with the FDA scheduled for December.”

In 2023 in the United States, there are expected to be an estimated 288,300[2] new patients with prostate cancer, which is classified as a “cold” tumor. Of those, 20% are expected to advance to mCRPC, a form of advanced prostate cancer that is no longer responding to testosterone-lowering hormone treatments and has spread to other areas of the body such as the lymph nodes, bones, bladder, rectum, liver, or lungs. Approximately 80% of mCRPC cases are of the adenocarcinoma phenotype, which represent approximately 46,128 patients.

In addition to the new OS data, the Company recently presented an update on response rate data from the Phase 2 adenocarcinoma cohort at the Prostate Cancer Foundation Annual Scientific Retreat. The Company reported a RECIST partial response rate of 28% with a median duration of response of 19 months. This is in contrast to a RECIST response rate of 5% with a median duration of response of 16.8 months from the KEYNOTE-199 trial of pembrolizumab monotherapy in a similar patient population.

The Company’s Phase 2 trial is an open-label, multicenter study to evaluate the safety and efficacy of BXCL701 in combination with pembrolizumab in men with mCRPC of adenocarcinoma phenotype as well as in men with SCNC. Twenty-nine (29) evaluable adenocarcinoma patients received 0.3 mg of BXCL701 twice daily (BID) on days 1 through 14 of a 21-day cycle (0.2 mg BID the first week of Cycle 1) plus 200 mg of pembrolizumab administered intravenously on day 1 and every subsequent 21 days. The primary objective of the trial is a composite response rate defined as either objective response by RECIST 1.1 criteria and/or PSA50 and/or CTC count conversion. Secondary objectives include duration of response, progression-free survival, overall survival, and biomarker evaluation as measured by changes in circulating cytokines and correlation of outcome with baseline tumor characteristics.

The Company is continuing to actively evaluate strategic options for OnkosXcel Therapeutics, including potential financing, strategic partnership, or M&A.

About OnkosXcel Therapeutics, LLC and BXCL701
OnkosXcel Therapeutics, LLC is a wholly owned subsidiary of BioXcel Therapeutics, Inc., focused on developing transformative medicines in oncology utilizing artificial intelligence. The subsidiary was formed in 2022 to develop 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 anti-cancer 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 & Drug Administration in four indications: acute myelogenous leukemia, pancreatic cancer, stage IIb to IV melanoma, and soft tissue sarcoma. 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 and 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, statements regarding: the Company's expected timing of, trial design and data results from, future clinical trials of BXCL701 with pembrolizumab, potential benefits from treatment with BXCL701, the Company's planned discussions with FDA, the future clinical development of BXCL701, the Company's plans to evaluate strategic options for OnkosXcel Therapeutics and potential market size and opportunity for product candidates. 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.

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Source: BioXcel Therapeutics, Inc.

1. FOR ILLUSTRATIVE PURPOSES ONLY: Analysis based on results from Pembrolizumab KEYNOTE-199 study. Pembrolizumab for Treatment-Refractory Metastatic Castration-Resistant Prostate Cancer: Multicohort, Open-Label Phase II KEYNOTE-199 Study. Emmanuel S. Antonarakis et al. Journal of Clinical Oncology; published at ascopubs.org/journal/jco on November 27, 2019: DOI https://doi.org/10.1200/JCO.19.01638.

Pembrolizumab is not an approved therapy for the treatment of any form of mCRPC, and no head-to-head clinical trial has been conducted evaluating BXCL701 against pembrolizumab or other candidates or products. Notable differences exist between the Company's trial designs, conditions under study and subject characteristics as compared to the third-party results discussed above and caution should be exercised when comparing data across these studies.

2. American Cancer Society. Key Statistics for Prostate Cancer. Retrieved October 9, 2023. https://www.cancer.org/cancer/types/prostate-cancer/about/key-statistics.html#:~:text=The%20American%20Cancer%20Society's%20estimates,34%2C700%20deaths%20from%20prostate%20cancer