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

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

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 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.
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.