BioXcel Therapeutics Presents Results from Ongoing Phase 2 Trial of BXCL701 in Combination with KEYTRUDA® in Aggressive Forms of Prostate Cancer at ESMO

September 15, 2021

BXCL701 plus KEYTRUDA® (pembrolizumab) demonstrates encouraging anti-tumor activity in heavily pre-treated mCRPC patients with adenocarcinoma

BXCL701 combination continues to exhibit favorable safety profile; patient enrollment continues as per protocol

NEW HAVEN, Conn., Sept. 15, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced data from its ongoing Phase 1b/2 trial of BXCL701, the Company's investigational, oral innate immunity activator, in metastatic castration-resistant prostate carcinoma (mCRPC) in a poster presentation at the 2021 European Society for Medical Oncology (ESMO) Congress.

“These data lay a strong foundation for the broad potential of BXCL701 in combination with pembrolizumab, including for heavily pre-treated mCRPC patients with adenocarcinoma, an aggressive tumor for which there are few available treatment options,” said Vincent J. O’Neill, M.D., Senior Vice President and Chief Medical Officer of BioXcel. “The study demonstrated that 26% of patients in the adenocarcinoma cohort achieved a composite response, and all responders experienced a decrease in tumor size. Further, the majority of these responders who received BXCL701 in combination with pembrolizumab did not have strong predictive markers of pembrolizumab response. We believe BXCL701 has the potential to be the most advanced orally available innate immune activator and to inflame the tumor micro-environment, thus making tumors more responsive to immunotherapies, including the PD-1 inhibitor, pembrolizumab. We look forward to the continued evaluation of BXCL701 in mCRPC in our adenocarcinoma cohort, as well as in our small-cell neuroendocrine carcinoma (SCNC) cohort, which both continue to enroll patients.”

The Phase 1b/2 trial is an open-label, multicenter study to evaluate the safety and efficacy of BXCL701 in combination with pembrolizumab, in men with mCRPC adenocarcinoma and SCNC phenotypes. Eligibility criteria include progression as defined by PCWG3 criteria and at least 1 line of taxane chemotherapy for inclusion in the adenocarcinoma cohort, or at least 1 prior line of chemotherapy for inclusion in the SCNC cohort. 32 eligible mCRPC 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 endpoint of the trial is composite response rate, with a target of achieving >15% response. Secondary endpoints include duration of response, progression-free survival, and overall survival.

KEY FINDINGS – ADENOCARCINOMA

- In the response evaluable patient cohort (n=23), 6 (26%) achieved a composite response; all responders experienced a decrease in tumor size from baseline.
- In patients with measurable disease (n=19), RECIST-defined partial response was 16%.
- The disease control rate (defined as PR + SD + non-CR / non-PD) was 63%.
- In the 29 patients who had at least 1 post-baseline PSA measurement, the PSA_{50} was 17% including 3 patients who had a PSA decrease of around 90%.
- From historic data, single agent pembrolizumab saw an objective response rate of approximately 5%, a disease control rate of 12% and a PSA_{50} response of 6%.[1]
- Treatment-related adverse events (AEs) observed with the combination were generally low-grade and consistent with the side effect profiles of each agent. The most common AEs included fatigue (16%), hypotension (13%), and pruritus and rash (13%). There was no evidence that BXCL701 increased immune-related AEs associated with checkpoint inhibitors.

POSTER PRESENTATION DETAILS

Title: BXCL701—1st-in-class oral activator of systemic innate immunity—combined with pembrolizumab, in men with metastatic castration-resistant prostate cancer (mCRPC): Phase 2 results

Poster Session: Genitourinary Tumors, Prostate

Date/Time: September 15, 2021 at 6:05 PM EST

Poster Number: 610P

Additional information can be found at www.esmo.org.

About BXCL701

BXCL701 is an investigational orally administered innate immune activator designed to initiate inflammation in the tumor microenvironment. Approved and experimental immunotherapies often struggle to address cancers that appear “cold” or uninflamed. Therefore, BXCL701 may 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. BioXcel's preclinical data supports BXCL701's synergy with both current checkpoint inhibitor-based therapies and emerging immunotherapies directed to activate T-cells, such as IL-2.

BXCL701 is currently being developed as therapy for metastatic castration-resistant prostate cancer of adenocarcinoma and small-cell neuroendocrine carcinoma (SCNC) phenotypes (both "cold" tumors) and other advanced solid cancers that are "hot" or have become resistant to checkpoint inhibitors.

About BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc. is a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. BioXcel'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 indices. BioXcel's two most advanced clinical development programs are BXCL501, an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine for the treatment of agitation associated with psychiatric and neurological disorders, and BXCL701, an investigational, orally administered, systemic innate immunity activator in development for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. For more information, please visit www.bioxceltherapeutics.com.

Forward-Looking Statement

This press release includes "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this press release include but are not limited to the timing and data from clinical trials for BXCL701 and the potential benefits of treatment with BXCL701,. When used herein, words including "anticipate," "being," "will," "plan," "may," "continue," and similar expressions are intended to identify forward-looking statements. 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 limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501 and BXCL701 and other product candidates; 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 approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; impacts from the COVID-19 pandemic; 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, 2021, 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. 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] Antonarakis et al. "Pembrolizumab for Treatment-Refractory Metastatic Castration-Resistant Prostate Cancer: Multicohort, Open-Label Phase II KEYNOTE-199 Study." *Journal of Clinical Oncology* 38, no. 5 (February 10, 2020) 395-405. DOI: 10.1200/JCO.19.01638.