BioXcel Therapeutics Announces Expansion of Phase 2 Trial of BXCL701 in De Novo and Treatment-Emergent Small-Cell Neuroendocrine Prostate Cancer

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Initial findings from Phase 1b/2 trial of BXCL701 in combination with KEYTRUDA met efficacy threshold to support expansion of existing cohort of de novo and treatment-emergent Small-Cell Neuroendocrine Prostate Cancer (SCNC)

High unmet medical need with no FDA approved therapies for patients presenting with SCNC

Data from the trial, including the expansion cohort, anticipated by Q1 2022

NEW HAVEN, Conn., Oct. 18, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop potentially transformative medicines in neuroscience and immuno-oncology, today announced that it has expanded its ongoing Phase 1b/2 trial of BXCL701, the Company's investigational, orally administered innate immune activator, in heavily pre-treated metastatic castration-resistant prostate cancer (mCRPC) patients with either de novo or treatment-emergent small-cell neuroendocrine carcinoma (SCNC). The Company's decision to advance BXCL701 into the second stage of the Phase 2a efficacy portion of the study follows the achievement of the protocol-specified efficacy threshold for cohort expansion, meaning observation of three composite responses.

"There is currently no standard of care for mCRPC patients presenting with the SCNC phenotype," said Vincent J. O'Neill, M.D., Senior Vice President and Chief Medical Officer of BioXcel. "SCNC is a particularly rare and difficult to treat variation of mCRPC. The decision to expand the SCNC cohort builds upon positive interim safety and efficacy data from the adenocarcinoma cohort presented last month at ESMO and moves us into the next stage of the evaluation lifecycle. With three composite responses observed among ten evaluable patients, and what we believe is a manageable side effect profile, we continue to be encouraged by BCXL701's potential to generate an immune response in 'cold' tumor types. We intend to continue recruitment of additional patients for our SCNC cohort, and we look forward to the further evaluation of BXCL701 in this mCRPC patient population, as well as in our adenocarcinoma cohort, which also continues enrollment."

The Phase 1b/2 trial is an open-label, multicenter study to evaluate the safety and efficacy of BXCL701 in combination with pembrolizumab for men with mCRPC presenting with either SCNC or adenocarcinoma phenotypes. Eligibility criteria include progression as defined by PCWG3* criteria and at least one prior line of cytotoxic chemotherapy for inclusion in the SCNC cohort, or one or two androgen signaling inhibitors and at least one line of taxane chemotherapy for inclusion in the adenocarcinoma cohort. mCRPC patients with SCNC 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 trial endpoint is a composite response rate, defined as either RECIST 1.1 objective response or PSA_{50} or CTC count conversion, with a target of achieving >15% response rate. Provided there are at least three composite responses observed in any patient cohort in the first stage of the Phase 2a portion of the study, additional patients may be accrued within that cohort during the second stage, for a total of up to 28 patients. Secondary endpoints include duration of response, progression-free survival, and overall survival. As of October 18, three of ten SCNC evaluable patients achieved composite response, satisfying the protocol-specified threshold for continued expansion.

*Prostate Cancer Working Group 3 (PCWG3) is an international working group of clinical and translational experts in prostate cancer who issued Consensus Guidelines on key principles of trial conduct for trials in castration-resistant prostate cancer

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 (mCRPC) 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, trial design 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.

Contact Information

Corporate

BioXcel Therapeutics
Erik Kopp
1.203.494.7062
ekopp@bioxceltherapeutics.com

Media

FTI Consulting
Helen O’Gorman
1.718.408.0800
helen.ogorman@fticonsulting.com

Investor Relations

FTI Consulting
Matt Ventimiglia
1.212.850.5624
matthew.ventimiglia@fticonsulting.com

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