BioXcel Therapeutics Announces Promising Top-Line Results from Phase 2 Trial of BXCL701 in Aggressive Form of Rare Prostate Cancer

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Results demonstrate encouraging response rate of BXCL701 plus KEYTRUDA® (pembrolizumab) in patients with SCNC

Full data will be presented at the 2023 ASCO Genitourinary Cancers Symposium in February

NEW HAVEN, Conn., Jan. 11, 2023 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced promising top-line data from its Phase 2 trial of BXCL701, the Company's investigational, oral innate immune activator, in combination with KEYTRUDA® (pembrolizumab) in small cell neuroendocrine metastatic castration-resistant prostate cancer (SCNC) patients. Full data have been submitted to the 2023 American Society of Clinical Oncology Genitourinary Cancers Symposium (ASCO GU).

“We are pleased that BXCL701 in combination with pembrolizumab has demonstrated an encouraging response rate in this difficult-to-treat cancer with no currently approved FDA therapies,” said Vincent J. O’Neill, M.D., Chief R&D Officer, OnkosXcel Therapeutics, a wholly owned subsidiary of BioXcel Therapeutics. “BXCL701’s promising profile as an oral innate immune activator with a large safety dataset and novel mechanism of action further supports OnkosXcel’s pipeline and use of BioXcel’s AI platform, and reinforces our confidence in BXCL701’s potential to enable checkpoint inhibitor therapy in traditionally cold cancers.”

SCNC represents a rare, underserved, growing patient population, with SCNC cases increasing due to earlier and more widespread use of androgen receptor inhibitors. In 2022, there were an estimated 268,500[1] new prostate cancer patients, with approximately 10,740 patients progressing to SCNC.

The Phase 2a trial is an open-label, multicenter study to evaluate the safety and efficacy of BXCL701 in combination with pembrolizumab in men with SCNC. Eligibility criteria include histologically confirmed *de novo* or treatment-emergent SCNC, progression as defined by PCWG3 criteria, and at least 1 prior line of chemotherapy for locally advanced or metastatic prostate cancer. 28 evaluable SCNC 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 a composite response rate defined as RECIST 1.1 and/or PSA_{50} and/or CTC count conversion. Secondary endpoints 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.

About BXCL701
BXCL701 is an investigational, oral 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 Therapeutics’ preclinical data supports BXCL701’s synergy with both current checkpoint inhibitor-based therapies 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.

About OnkosXcel Therapeutics, LLC
OnkosXcel Therapeutics, LLC is a wholly owned subsidiary of BioXcel Therapeutics, Inc., focused on developing transformative medicines in oncology utilizing artificial intelligence approaches. The subsidiary was formed in 2022 to develop BXCL701, a Phase 2, investigational, oral innate immune activator for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors, as well as other immuno-oncology focused assets.

About Metastatic Castration-Resistant Prostate Cancer (mCRPC) and Treatment-Emergent SCNC
mCRPC is 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, the bladder, rectum, liver, or lungs. Treatment-emergent SCNC is a particularly difficult-to-treat histologic subtype of mCRPC that emerges in approximately 20% of mCRPC patients, though this number is increasing due to earlier and more widespread use of androgen blockers.

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc., is a biopharmaceutical company utilizing artificial intelligence approaches 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. The Company’s commercial product, IGALMI™ (developed as BXCL501), is a proprietary, sublingual film formulation of dexmedetomidine approved for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. The safety and effectiveness of IGALMI have not been established beyond 24 hours from the first dose. For more information, please visit IGALMIhcp.com and also see the IGALMI full Prescribing Information. BXCL501 is under evaluation for at-home use for the acute treatment of agitation in bipolar and schizophrenia patients, for acute treatment of Alzheimer’s-related agitation, and as an adjunctive treatment for major depressive disorder. The safety and efficacy of BXCL501 for these uses have not been established. The Company is also developing BXCL502 as a potential therapy for chronic agitation in dementia. Under its subsidiary, OnkosXcel Therapeutics LLC, the Company is developing BXCL701, an investigational, oral innate immune activator for the treatment of

aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. The safety and efficacy of BXCL502 and BXCL701 have not been established. 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 presentation at the 2023 ASCO GU, the expected timing of, trial design and benefits of BXCL501, BXCL502 and BXCL701 and potential market opportunities for BXCL501. 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 experience in drug discovery and drug development; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502 and BXCL701 and other product candidates; its lack of experience in marketing and selling drug products; 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; and the other important factors discussed under the caption "Risk Factors" in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2022, 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 American Cancer Society's estimates for prostate cancer in the United States for 2022