BioXcel Therapeutics Receives FDA Fast Track Designation for BXCL701 for Treatment of Small Cell Neuroendocrine Prostate Cancer (SCNC)

February 12, 2024

Designation for BXCL701 in combination with a checkpoint inhibitor (CPI) for treatment of patients with metastatic SCNC with progression on chemotherapy and no evidence of microsatellite instability

BXCL701 is an investigational, oral innate immune activator designed to inflame the tumor microenvironment and augment CPI activity

Company to discuss registration path at upcoming meeting with FDA

NEW HAVEN, Conn., Feb. 12, 2024 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology, today announced that the U.S. Food and Drug Administration (FDA) has designated as a Fast Track development program the investigation of BXCL701 in combination with a CPI for the treatment of patients with metastatic small cell neuroendocrine prostate cancer (SCNC) with progression on chemotherapy and no evidence of microsatellite instability. The FDA grants Fast Track designation to facilitate the development and expedite the review of medicines to treat serious conditions, fill unmet medical needs, and bring promising medicines to patients more quickly. Therapies granted this designation are given the opportunity for more frequent interactions with the FDA, a rolling review, and potential eligibility for accelerated approval and priority review.

“The FDA’s Fast Track designation for the investigation of BXCL701 in SCNC is an important recognition of our most advanced immuno-oncology asset and an acknowledgment of its potential to address the considerable unmet medical need in these patients. At the same time, it further validates the unique AI-based drug re-innovation approach that we used to discover this asset,” said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. “BXCL701 has already demonstrated considerable potential in our clinical trials to date, and we plan to further define its development path while exploring strategic options for our OnkosXcel Therapeutics subsidiary.”

SCNC, classified as a “cold” tumor, represents an underserved, growing patient population, with cases increasing due to earlier and more widespread use of androgen receptor inhibitors. In 2024, the American Cancer Society estimates that 299,010[1] men will be diagnosed with prostate cancer in the United States, with approximately 20% expected to progress to the more aggressive metastatic castration-resistant form, including an estimated 11,960 patients expected to progress to SCNC[2].

“SCNC is characterized by poor prognosis and a low survival rate, and current treatment options are suboptimal,” said Vincent J. O’Neill, M.D., Executive Vice President, Chief of Product Development and Medical Officer of BioXcel Therapeutics. “We are encouraged by the potential of BXCL701, which has demonstrated clinical proof of concept in both SCNC and adenocarcinoma. Following the positive survival results from our Phase 2 trial that we reported at the end of last year, we look forward to further discussing the registration path at an upcoming meeting with the FDA.”

About 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 anticancer 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 and Drug Administration in five indications: SCNC, 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. Its wholly owned subsidiary, OnkosXcel Therapeutics, is focused on the development of medicines in 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, the potential benefits from treatment with BXCL701 and from fast track designation, potential meeting with the FDA and strategic options for the OnkosXcel Therapeutics subsidiary. 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 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; and its novel approach to the discovery and development of product candidates based on EvolverAI, as well as 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, 2023, 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

Investor Relations
BioXcel Therapeutics
Brennan Doyle
1.475.355.8462
bdoyle@bioxceltherapeutics.com

Media
Russo Partners
David Schull
T: 858-717-2310
David.schull@russopartnersllc.com
Scott Stachowiak
T: 646-942-5630
Scott.stachowiak@russopartnersllc.com

Source: BioXcel Therapeutics, Inc.

References

1 American Cancer Society. Key Statistics for Prostate Cancer. Accessed February 8, 2024 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
2. B.R. Alabi, S. Liu and T. Stoyanova, Current and emerging therapies for neuroendocrine prostate cancer, *Pharmacology and Therapeutics* (2022), https://doi.org/10.1016/j.pharmthera.2022.108255