BioXcel Therapeutics Announces Formation of OnkosXcel Therapeutics to Develop Medicines Focused in Oncology

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OnkosXcel, a Company subsidiary, to utilize proprietary AI-powered platform to progress therapies to address difficult-to-treat cancers with high unmet need

New structure to unlock growth opportunities and maximize value of neuroscience and immuno-oncology franchises

Lead pipeline asset, BXCL701, has shown clinical proof-of-concept in aggressive forms of prostate cancer and is currently in Phase 2 clinical trials

NEW HAVEN, Conn., April 19, 2022 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI) (the "Company" or "BioXcel Therapeutics"), a commercial-stage biopharmaceutical company utilizing artificial intelligence (AI) approaches to develop transformative medicines in neuroscience, today announced the formation of wholly-owned subsidiary OnkosXcel Therapeutics, Inc. ("OnkosXcel") to develop transformative medicines in oncology.

OnkosXcel is focused on the sustained expansion and optimization of the oncology franchise, while providing maximum strategic and financial flexibility. OnkosXcel plans to progress the development of BXCL701, an investigational orally administered innate immune activator designed to initiate inflammation in the tumor microenvironment.

BXCL701 is being evaluated in combination with KEYTRUDA® (pembrolizumab) in an ongoing Phase 2 trial in metastatic castration-resistant prostate cancer (mCRPC) patients with either adenocarcinoma or small cell neuroendocrine carcinoma (SCNC) phenotype. Most recently, the Company announced positive efficacy and safety data at the 2022 ASCO Genitourinary Cancers Symposium, reinforcing the broad potential of BXCL701 to extend the activity of checkpoint inhibitor (CPI) therapy into "cold" tumor settings. BXCL701 is also being evaluated in combination with KEYTRUDA in advanced, "hot" and CPI-resistant tumors in an investigator-initiated, Phase 2 study led by MD Anderson Cancer Center, with additional efficacy data expected in the second half of 2022. BioXcel Therapeutics believes BXCL701 is one of the most advanced orally available, innate immune activators in the clinic and it has been evaluated in approximately 800 healthy subjects and cancer patients. OnkosXcel plans to explore the BXCL701 clinical development program in hematological malignancies and other solid tumors.

"We believe the formation of OnkosXcel will unlock significant value for both our neuroscience and immuno-oncology franchises and build a foundation for sustainable growth," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "For BioXcel Therapeutics, this is the logical next step as we advance our five-year vision of becoming the premier AI-driven neuroscience company, exemplified by the recent FDA approval of IGALMI™ for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. For our immuno-oncology assets, we believe this new structure will sharpen our strategic focus and help us further maximize the value of our oncology portfolio with increased flexibility in the future."

The Company plans to provide additional details regarding OnkosXcel in the second half of 2022.

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 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 therapy for mCRPC of adenocarcinoma and SCNC phenotypes (both "cold" tumors) and other advanced solid cancers that are "hot" or have become resistant to checkpoint inhibitors. BXCL701 has received Orphan Drug Designation (ODD) from the U.S. Food & Drug Administration (FDA) in four indications.

About BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc. is a commercial-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience. 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 indices. The Company's commercial product, IGALMI™ (developed as BXCL501) is a proprietary, sublingual film formulation of dexmedetomidine approved by the FDA for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. BXCL501 is also being evaluated for the acute treatment of Alzheimer's disease, and as an adjunctive treatment for major depressive disorder. For more information, please visit www.bioxceltherapeutics.com.

About OnkosXcel Therapeutics, Inc.

OnkosXcel Therapeutics, Inc. is a clinical-stage, independent, private subsidiary of BioXcel Therapeutics, Inc., focused on developing transformative medicines utilizing artificial intelligence approaches in oncology. The subsidiary was formed to develop BXCL701, a Phase 2, investigational, orally administered, systemic innate immunity 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.

Forward-Looking Statements

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 Company's plans for its OnkosXcel subsidiary; clinical development plans, including the ongoing development of BXCL701 and data from clinical trials of BXCL701 and BXCL701's status as one of the most advanced orally available, innate immune activators in the clinic; and the Company's future growth plans;. When used herein, words including "anticipate," "will," "plan," "potential," "may," "continue," "intend," "designed," "goal" 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; the Company's need for substantial additional funding and ability to raise capital when needed; the Company's dependence on the success and commercialization of IGALMI, 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; the regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, expensive and inherently unpredictable; the Company has limited experience in drug discovery and drug development; regulatory agencies, may not accept or agree with the Company's assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and the Company in general; the Company has no experience in marketing and selling drug products and has not entered into arrangements for the sale and marketing of IGALMI™; IGALMI™ or the Company's other product candidates may not be accepted by physicians or the medical community in general; the Company may not be able to obtain marketing approvals for BXCL501 or BXCL701; the Company may need substantial additional funding to develop and conduct clinical trials with respect to its product candidates and support its operations; the Company must comply with extensive regulations applicable to it; and healthcare reform could adversely impact future commercial success. These and other important factors are discussed under the caption "Risk Factors" in its Annual Report on Form 10-K for the year ended December 31, 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

Investor Relations

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

Media

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

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