September 4, 2019

BioXcel Therapeutics Receives FDA Orphan Drug Designation for BXCL701 for the Treatment of Acute Myeloid Leukemia (AML)

Expansion of clinical development for BXCL701 program planned in hematological malignancies, beyond solid tumors (Pancreatic and tNEPC)

Confirmation and further extension of key findings in Nature Medicine Publication for BXCL701 selectivity for targeting AML by pyroptotic programmed cell death

Third Orphan Drug Designation received for BXCL701 from the FDA in addition to Melanoma and Pancreatic Cancer

NEW HAVEN, Conn., Sept. 04, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI), a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology, today announced that the U.S. Food and Drug Administration ("FDA") has granted Orphan Drug Designation ("ODD") for BXCL701, an investigational orally-available systemic innate immunity activator with dual mechanisms of action, for the treatment of Acute Myeloid Leukemia ("AML").

BXCL701 is designed to activate innate immune cells, specifically macrophages, by inhibiting the dipeptidyl dipeptidases DPP8 and DPP9, a novel mechanism, and exerts immune stimulatory activity through a pro-inflammatory form of programmed cell death known as pyroptosis. The Company is currently leveraging the pro-inflammatory mechanism of action of BXCL701 for treatment-Emergent Neuroendocrine Prostate (tNEPC) cancer and Pancreatic cancer. However, recent preclinical work by Darren C. Johnson, et al, published in the July 2018 Nature Medicine publication, "DPP8/DPP9 inhibitor-induced pyroptosis for treatment of acute myeloid leukemia," has also pointed to AML as a potential indication for BXCL701, since AML cells are of the same lineage as macrophages. Targeting AML represents a novel application of BXCL701's intended mechanism of action of driving programmed cell death. While BXCL701 potentially acts as an activator of the innate immune system in Prostate and Pancreatic cancer, it may act as a direct cytotoxic agent in AML.

Vincent O'Neill, M.D., Senior Vice President & Chief Medical Officer of BTI, said, "AML is an aggressive form of cancer, originating in the myeloid line of blood cells. For patients who don't respond adequately to induction therapy or who quickly relapse (about 50% of all patients), outcomes can be particularly poor. Newer, more effective therapeutic options are therefore needed for this indication. BXCL701 has been observed to directly attack and kill AML cells in multiple preclinical studies, corroborating our belief that BXCL701 presents an

opportunity to address this rare and deadly disease. We plan to further investigate BXCL701 as a single agent and/or in combination for specific patient segments with high unmet medical needs. We believe that an active agent that is orally available offers important advantages in the clinical setting, and could combine well with other oral drugs, potentially providing patients with an all oral combination option. We look forward to exploring BXCL701 as a therapeutic option for AML as we finalize development plans and continue discussions with a number of leading academic centers."

Dr. Chetan D. Lathia, Senior Vice President & Head, Translational Medicine, Clinical Pharmacology & Regulatory Affairs for BTI, commented, "The FDA grants ODD to investigational therapies for conditions affecting fewer than 200,000 people in the US and the potential for seven years of marketing exclusivity if the drug is approved in the orphan designated indication. We believe that receiving the third ODD for BXCL701 validates our commitment to transforming drug development in oncology, including orphan indications. We are extremely excited to evaluate BXCL701's potential as a therapeutic option for AML."

About Acute Myeloid Leukemia:

AML is an aggressive hematologic malignancy in which myeloid lineage cells of the bone marrow cease to differentiate appropriately, resulting in a marked increase in the number of circulating immature blast cells. As a consequence, the counts of mature red blood cells, platelets, and normal white blood cells decline, causing fatigue, shortness of breath, bleeding, and increased susceptibility to infection. In 2019, 21,450 new cases of AML are estimated in the United States. The median age at diagnosis is 68 years old, with rising age associated with a progressively worsening prognosis.

About Orphan Drug Designation:

The FDA Office of Orphan Products Development grants Orphan Drug Designation to drugs and biologics that are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases and disorders that affect fewer than 200,000 people in the U.S. The designation allows BioXcel to qualify for a number of incentives, including the potential for seven years of market exclusivity upon regulatory approval in the orphan designated indication, if received; waiver from FDA marketing application user fees for AML; and tax credits for qualified clinical trials.

About BXCL701:

BXCL701 is an investigational orally-available systemic innate immunity activator with dual mechanisms of action. It has shown single agent activity in melanoma and safety has been evaluated in more than 700 healthy subjects and cancer patients. Designed to stimulate both the innate and acquired immune systems, BXCL701 is designed to inhibit dipeptidyl peptidase (DPP) 8/9 and block immune evasion by targeting Fibroblast Activation Protein (FAP). BXCL701 is currently being developed for treatment of a rare form of prostate cancer and for pancreatic cancer in combination with other immuno-oncology agents.

About BioXcel Therapeutics, Inc.:

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence approaches to identify and advance the

next wave of medicines in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically evaluated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI's two most advanced clinical development programs are BXCL501, an investigational sublingual thin film formulation in development for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an investigational orally administered systemic innate immunity activator in development for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer in combination with other immuno-oncology agents. For more information, please visit www.bioxceltherapeutics.com.

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 clinical development initiatives and trials for BXCL701, the benefits to the Company of ODD, and the efficacy of BXCL701 in the treatment of AML. 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 BTI's current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BTI 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; 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; 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, 2019 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 BTI 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 BTI’s views as of any date subsequent to the date of this press release.

Contact Information:

BioXcel Therapeutics, Inc.
www.bioxceltherapeutics.com
The Ruth Group
Carol Ruth / James Salierno
646-536-7004 / 7028
cruth@theruthgroup.com
jsalierno@theruthgroup.com

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