October 8, 2018

BioXcel Therapeutics Announces Submission of IND for Lead Immuno-oncology Candidate, BXCL701, in Treatment Emergent Neuroendocrine Prostate Cancer

First-in-human Phase 1b / 2 combination trial to treat rare form of prostate cancer, tNEPC, using BXCL701 and a PD-1 checkpoint inhibitor

NEW HAVEN, Conn., Oct. 08, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI") (Nasdaq: BTAI), a clinical stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology, today announced it has submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration ("FDA") to evaluate its lead immuno-oncology candidate, BXCL701, in combination with pembrolizumab (Keytruda®) as a potential therapy for treatment emergent neuroendocrine prostate cancer ("tNEPC"). BTI expects to initiate the trial, subject to IND approval, in the fourth quarter of 2018.

BXCL701 is a first in class oral immunotherapy with dual mechanisms of action that has demonstrated single agent activity in melanoma, with an established safety profile from 700 healthy subjects and cancer patients. Designed to stimulate both the innate and acquired immune systems, BXCL701 works by inhibiting dipeptidyl peptidase (DPP) 8/9 and blocking immune evasion by targeting fibroblast activation protein (FAP). Preclinical combination data evaluating BXCL701, a checkpoint inhibitor and other immuno-oncology agents has demonstrated encouraging anti-tumor activity in multiple tumor types and formation of functional immunological memory. BXCL701's primary mechanism of action has recently been highlighted in multiple peer reviewed journals, providing further validation of the scientific rationale behind BXCL701.

tNEPC is a rare hormone-refractory manifestation of prostate cancer occurring secondary to treatment with androgen deprivation therapies such as Zytiga® (Johnson & Johnson) and Xtandi® (Pfizer). This form of highly aggressive tumor, with no current treatment, is observed in approximately 20-30% of patients treated with androgen inhibitors and has a median survival time of less than one year. Single agent checkpoint inhibitor therapy produces very low response rates in hormone refractory prostate cancer, creating a major unmet medical need for tNEPC patients.

Dr. Vincent J. O'Neill, Senior Vice President and Chief Medical Officer of BTI, commented, "We believe that inhibition of DPP 8/9 and FAP can effectively target tNEPC, a recently characterized form of prostate cancer. We believe the combination of BXCL701 and

pembrolizumab can inhibit tumor growth and increase T-cell infiltration, potentially resulting in superior clinical responses. Submission of our IND for BXCL701 marks a significant milestone for BTI, and we look forward to further assessing its potential as a treatment for tNEPC and other forms of cancer. We are excited to initiate the trial of BXCL701 in combination with a checkpoint inhibitor to advance our mission of developing transformative medicines for patients."

The trial has been designed by BTI's clinical team, in consultation with its immuno-oncology clinical advisory board. BTI has selected a world-class oncology focused full service clinical research organization ("CRO") to develop BXCL701 as a combination therapy. The CRO will support BTI in selecting the trial sites as well as conducting and managing the clinical studies.

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 the next wave of medicines across 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 indices. The Company's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer. 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, statements that relate to the advancement and development of BXCL701, the commencement of clinical trials, the availability of data from clinical trials and other information that is not historical information. When used herein, words such as "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 BioXcel's current expectations and various assumptions. BioXcel believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. BioXcel 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, market conditions and the factors described under the caption "Risk Factors" in BioXcel's 10 Q for the Quarter ended June 30, 2018 and BioXcel's other filings made with the Securities and Exchange Commission. Consequently, forward-looking statements should be regarded solely as BioXcel's current plans, estimates and beliefs. Investors should not place undue reliance on forward-looking statements. BioXcel cannot guarantee future results, events, levels of activity, performance or achievements. BioXcel does not undertake and specifically declines any obligation to update, republish, or revise any forward-looking statements to reflect new information, future events or circumstances or to reflect the occurrences of unanticipated events, except as may be required by law.

Contact Information:
The Ruth Group
Lee Roth/ Janhavi Mohite
646-536-7012/ 7026
lroth@theruthgroup.com/ jmohite@theruthgroup.com

Source: BioXcel Therapeutics, Inc.