BioXcel Therapeutics Files Clinical Trial Application for the Development of BXCL701 and Pembrolizumab in Neuroendocrine Prostate Cancer (tNEPC)

March 25, 2019

Expands global footprint for lead immuno-oncology program, BXCL701

Patient recruitment in US tNEPC study on-track; additional sites being activated

Immuno-oncology Clinical Advisory Board expanded with addition of prominent U.K. medical oncologist, Professor Johann de Bono, M.D., Ph.D., who will serve as European Principal Investigator for trial

IND filing for BXCL701 in combination with NKTR-214 and avelumab (triple combination) in pancreatic cancer expected in 2Q 2019 following positive FDA feedback

NEW HAVEN, Conn., March 25, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI") (Nasdaq: BTAI) today announced that it has filed a Clinical Trial Application (CTA) with the U.K. health authorities for its lead immuno-oncology asset, BXCL701, an orally-available small molecule immune-modulator with dual mechanisms of action, in combination with pembrolizumab (Keytruda®), a checkpoint inhibitor, in tNEPC. BTI is a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology.

Following approval of the CTA, BTI plans to expand the Phase 1b/2 US study of BXCL701 and pembrolizumab in tNEPC to the U.K. Professor Johann de Bono, M.D., Ph.D., of The Royal Marsden NHS Foundation Trust and The Institute of Cancer Research, will serve as Principal Investigator for the European study of BXCL701 in tNEPC. Data from the open-label trial will support the ongoing global clinical development of BXCL701.

Additionally, BioXcel Therapeutics is preparing to file an Investigational New Drug (IND) application with the FDA for a clinical trial evaluating the triple combination of BXCL701, NKTR-214 and avelumab in the treatment of advanced pancreatic cancer.

Dr. Chetan D. Lathia, SVP & Head of Translational Medicine, Clinical Pharmacology and Regulatory Affairs of BTI said, "The filing of the CTA in the U.K., for the BXCL701 combination trial is an important regulatory milestone for BTI. It marks the beginning of our plans for the global development of our lead programs."

Dr. Vimal Mehta, Chief Executive Officer of BTI added, "We remain committed to translating the potential of BXCL701's novel mechanism of action into therapies that can fundamentally change the lives of cancer patients. The filing of this CTA is the first milestone in the expansion of our footprint into major markets outside the US. We believe that our pancreatic cancer clinical development partnership with Nektar, Pfizer and Merck KGaA, Darmstadt, Germany will also benefit the broader BXCL701 program."

The Company also announced that Professor de Bono has joined its Immuno-oncology Clinical Advisory Board. Professor De Bono is the Regius Professor of Cancer Research and a Professor in Experimental Cancer Medicine at The Institute of Cancer Research and The Royal Marsden NHS Foundation Trust. He is also the Director of the Drug Development Unit, overseeing the Phase I trials, with a particular interest in innovative trial designs, circulating biomarkers and prostate cancer. Additionally, he leads the Prostate Cancer Targeted Therapy Group and the Cancer Biomarkers laboratory team. He has served as chief investigator on trials for multiple approved drugs, including ZYTIGA®, JEVTANA® and XTANDI®.

Professor de Bono is a clinical investigator in the KEYNOTE- 199 trial, a study evaluating Pembrolizumab in docetaxel pre-treated castrate-resistant metastatic prostate cancer patients. He graduated with a medical degree and a postdoctoral degree from University of Glasgow. He also trained in medical oncology and was awarded a master's degree in cancer sciences from the University of Glasgow. Professor de Bono has received multiple awards including one of the "World's Most Influential Scientific Minds," the European Society for Medical Oncology (ESMO) award and the Royal Society of Chemistry award.

About BXCL701
BXCL701 is an orally-available systemic innate-immune activator with dual mechanisms of action. It 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 an important validation of the scientific rationale behind BXCL701.

About BioXcel Therapeutics, Inc.:
BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence to identify the next wave of medicines across neuroscience and immuno-oncology. BTI'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. BTI'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 BXCL501 and 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 Form 10K for the period ending December 31, 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.

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