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BioXcel Therapeutics Announces FDA Acceptance of IND for Lead Immunology Candidate, BXCL701, in Treatment Emergent Neuroendocrine Prostate Cancer

First-in-human Phase 1b / 2 combination trial of BXCL701 and pembrolizumab (Keytruda®) expected to initiate in 4Q 2018

Efficacy study with objective response rate endpoint to enroll up to 40 patients at multiple clinical sites

NEW HAVEN, Conn., Nov. 05, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or "Company") (Nasdaq: BTAI), today announced that the U.S. Food and Drug Administration ("FDA"), has accepted its Investigational New Drug ("IND") application for its lead immuno-oncology candidate, BXCL701. BTI plans to evaluate BXCL701 in combination with pembrolizumab (Keytruda®) as a potential therapy for treatment-emergent neuroendocrine prostate cancer ("tNEPC"), with the trial expected to initiate in the fourth quarter of 2018. BTI is a clinical stage biopharmaceutical development company that utilizes novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology.

Dr. Vincent J. O'Neill, Senior Vice President and Chief Medical Officer of BTI, commented, "FDA approval of this IND is a significant milestone for our BXCL701 program, as we are now able to commence the Phase 1b/2 combination study in tNEPC patients. We believe that the combination of BXCL701 and pembrolizumab has the potential to meaningfully improve the lives of patients with this highly aggressive, rare form of prostate cancer, and to succeed where current checkpoint inhibitor monotherapies have demonstrated limited clinical benefit. We have an obligation to tNEPC patients to find a viable treatment and look forward to evaluating the combination in this trial."

Dr. Vimal Mehta, Chief Executive Officer of BTI added, "The FDA approval of this IND and initiation of this trial provides an important validation of our AI-powered approach to drug development, which enables us to develop therapeutic candidates more quickly, at a lower cost and with a higher probability of success than with traditional drug development approaches."

The Phase 1b/2 study is expected to enroll up to 40 subjects at multiple trial sites. The goal of this single arm, Simon 2-stage open label study is to examine the safety, pharmacokinetics and anti-tumor activity of the combination of BXCL701 and pembrolizumab in tNEPC patients with the efficacy endpoint of objective response rate. Data

readouts are expected throughout 2019.

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 Treatment-emergent neuroendocrine prostate cancer (tNEPC)

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.

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. 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 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.

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