BioXcel Therapeutics Showcases Data on BXCL701 in Combination with OX40 Agonist in Late-breaking Poster Presentation at AACR Annual Meeting

April 1, 2019

Novel combination achieves synergistic anti-tumor effect and survival benefit

Demonstrates BXCL701's potential ability to bridge innate and adaptive immune systems

Clinical Advisors Louis Weiner, MD and Daniel Von Hoff, MD Receive 2019 AACR Distinguished Service Award

NEW HAVEN, Conn., April 01, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI) today presented promising preclinical data demonstrating potential for combining the Company's BXCL701, an oral immunomodulator, and an OX40 agonist antibody as a possible combination therapy for certain solid tumors at the American Association for Cancer Research (AACR) Annual Meeting. This year's meeting is being held from March 29 to April 3, 2019 in Atlanta, Georgia. BTI is a clinical stage biopharmaceutical development company utilizing novel artificial intelligence to identify the next wave of medicines across neuroscience and immuno-oncology.

Results from this preclinical study demonstrate that BXCL701, a dipeptidyl peptidase (DPP) and fibroblast activation protein (FAP) inhibitor, in combination with an anti-OX40 antibody resulted in synergistic anti-cancer activity and a statistically significant improvement in median survival compared to vehicle, as well as BXCL701 or the OX40 agonist alone. These results support the Company's belief that the combination of BXCL701 and an OX40 agonist represents a new potential treatment approach against multiple cancer types, and provide rationale for a clinical efficacy study of the combination therapy. There are currently multiple OX40 agonists in various stages of clinical development.

Dr. Vincent J. O'Neill, Chief Medical Officer of BTI, commented, "We are pleased to report the findings from this study, which support the therapeutic rationale for combining BXCL701 with an OX40 agonist. This combination achieved a significant increase in anti-cancer activity in tumor models, as compared to control. These results further validate BXCL701 as a potentially versatile and effective immuno-oncology agent, due to its ability to stimulate both innate and adaptive immunity. Based on these results, we plan to explore further studies to enhance our understanding of this particular therapeutic approach and its potential in a clinical setting."

Prior studies have shown that BXCL701 inhibits tumor growth and up-regulates immuno-stimulatory cytokines as well as tumor infiltrating immune cells by targeting DPP 8/9 and FAP. Treatment with BXCL701 induces pyroptosis in macrophages, and results in the production of several pro-inflammatory factors important for a robust, anti-tumor adaptive immune response mediated by T-cells.

Full details of the accepted AACR late breaking poster presentation are below:

Abstract #077 / Poster #22**: Dipeptidyl Peptidase Inhibitor BXCL701 synergizes with an OX40-agonist antibody resulting in synergistic anti-tumor response and survival in an animal model of colorectal cancer by bridging the innate and adaptive arms of the immune system**

Date:	Monday, April 01, 2019
Time:	8:00 AM-12:00 PM ET
Session:	Late Breaking Research- Immunotherapy 1
Location:	Georgia World Congress Center, Exhibit Hall B, Section 41

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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