

June 4, 2018



BioXcel Therapeutics and Nektar Therapeutics Present Preclinical Pancreatic Cancer Data for BXCL701, NKTR-214 and Anti-PD1 Combination Therapy at ASCO 2018 Annual Meeting

BXCL701 in Combination with NKTR-214 and anti-PD-1 Therapy Leads to Complete and Sustained Tumor Regression and Immune Memory in Pre-Clinical Syngeneic Models

Results Support Further Development of triplet therapy

BRANFORD, Conn., June 04, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI") (Nasdaq:BTAI), a clinical stage biopharmaceutical development company utilizing novel artificial intelligence to identify the next wave of medicines across neuroscience and immuno-oncology, today announced data from preclinical studies of the Company's BXCL701 and Nektar Therapeutics' NKTR-214 as a potential combination therapy for pancreatic cancer patients at the 2018 American Society for Clinical Oncology (ASCO) Annual Meeting, being held June 1-5, 2018 in Chicago, IL.

Results from the preclinical study demonstrate that BXCL701, a dipeptidyl peptidase (DPP) and fibroblast activation protein (FAP) inhibitor, in combination with NKTR-214, a CD122-biased agonist, and an anti-PD-1 antibody represents a new treatment approach against pancreatic cancer. BTI plans to present additional data demonstrating BXCL701's potential to generate anti-cancer immunity.

Dr. Vincent J. O'Neill, Chief Medical Officer of BTI commented, "This study highlights the potential of BXCL701 to augment the effect of other immunotherapies such as NKTR-214 and anti-PD-1 to produce an improved, durable anti-cancer response. The triplet therapy leverages the power of both innate and adaptive immunity to attain complete tumor regression in these preclinical studies by recruiting increased levels of immuno-stimulatory cytokines, effector T cells and NK cells while reducing the cytokines involved in immune evasion. Most importantly, the combination also demonstrated functional evidence of memory T cell response, making the mice immune to re-challenge with the same cancer cells. We believe that this triple combination could offer a unique therapeutic approach in the treatment of immune checkpoint inhibitor resistant pancreatic cancer."

DPPs play a crucial role in tumorigenesis and tumor stromal remodelling. FAP is associated with immune system evasion and suppression in cancer biology. High levels of both DPP8/9 and FAP are expressed in pancreatic tumors. BXCL701's dual mechanism of action targets DPP8/9, stimulating effector T cells and NK cells while inhibition of FAP helps to clear an immune obstructive tumor microenvironment, thus promoting a robust pro-inflammatory

response.

NKTR-214 in combination with anti-PD-1 has shown a strong anti-cancer response in multiple murine cancer models and in combination with nivolumab in multiple human cancers^{1,2}.

This preclinical study aimed to utilize the triple combination of BXCL-701, NKTR-214 and anti-PD-1 to potentiate more robust and durable anti-tumor activity and help overcome an immune refractory tumor microenvironment. Data from the study displayed a significant improvement in complete tumor regression and development of anti-cancer immunity in the Pan02 mouse model. Treatment with the triple combination showed increases in Ly6G+ neutrophils, CD8+ T cell infiltrates and immune stimulatory cytokines that are responsible for generation of memory T cell responses. The combination therapy also achieved a notable reduction in FAP expressing cells and cytokines associated with tumor invasion and migration.

Full details of the accepted ASCO poster are below:

Abstract #3085 / Poster #299: Efficacy and immune modulation by BXCL701 a dipeptidyl peptidase inhibitor, NKTR-214 a CD122-biased immune agonist with PD1 blockade in murine pancreatic tumors

Presenter BioXcel Therapeutics' and Nektar Therapeutics' teams

Date: Monday, June 04, 2018

Time: 8:30 AM-11:30 AM CT

Session: Developmental Therapeutics- Immunotherapy

Location: Hall A

1. Charych, Deborah, et al. "Modeling the receptor pharmacology, pharmacokinetics, and pharmacodynamics of NKTR-214, a kinetically-controlled interleukin-2 (IL2) receptor agonist for cancer immunotherapy." PloS one 12.7 (2017): e0179431.

2. Adi Diab et al., Presented at SITC 2017, Maryland, Poster #O20

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.

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 BXCL701's advancement and development, the presentation of data at ASCO and other information that is not historical

information. When used herein, words such as “anticipate”, “being”, “will”, “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 factors, including, without limitation, market conditions and the factors described under “Risk Factors” in BioXcel’s prospectus dated March 7, 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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Source: BioXcel Therapeutics, Inc.