

April 3, 2017



BioXcel Presents Promising Data Demonstrating BXCL701 / Anti-PD1 Combination Increases Anti-Tumor Cytokines and Tumor Growth Inhibition at 2017 AACR Annual Meeting

Next-Generation Small Molecule Checkpoint Inhibitor Targeting Castration-Resistant Prostate Cancer

BRANFORD, Conn., April 03, 2017 (GLOBE NEWSWIRE) -- BioXcel, a biopharmaceutical company integrating big data analytics and machine learning-based artificial intelligence (AI) with drug development expertise to advance the next wave of medicines, today announced that it will present a poster supporting the clinical development of its lead candidate BXCL701 in castration-resistant prostate cancer (CRPC) at the upcoming [American Association for Cancer Research \(AACR\) Annual Meeting](#), being held April 1-5, 2017 in Washington, D.C.

Dr. Luca Rastelli, BioXcel's VP, Oncology R&D, commented "BXCL701, our first-in-class small molecule immuno-modulator stimulates a pro-inflammatory anti-tumor cellular response that is complementary to the one mediated by immune-checkpoints. In addition, genomic data analysis of patients with CRPC showed that BXCL701 molecular targets are upregulated in patients previously treated with the androgen deprivation therapies enzalutamide or abiraterone. These data support our plan to develop BXCL701 as a monotherapy and in combination with immune-checkpoint inhibitors (ICI) in advanced CRPC."

Dr. Rastelli added: "We are highly encouraged by the results obtained from this study being presented at the prestigious AACR 2017 meeting. These findings show that BXCL701 in combination with an anti-PD1 agent, may demonstrate clinical benefit in patients that do not respond to ICI therapy. Moreover, BXCL701 has broad potential to combine with other checkpoint inhibitors and therapeutic modalities including vaccines and cellular therapies."

The topline data and logistical details of the poster presentation includes:

[Abstract #2629 / Poster #18](#): The synergy between BXCL701, a DPP inhibitor, and immune checkpoint inhibitors discovered using AI and Big Data analytics

Date: Monday, April 3, 2017
Time: 1 – 5 p.m. EDT
Location: Section 25

This preclinical study confirmed the ability of BXCL701 to synergistically enhance the anti-tumor activity of ICI. An *in-vivo* study of BXCL701 in combination with an anti-PD1 agent in the syngeneic MC38 colon adenocarcinoma mouse model demonstrated an increase in anti-tumor cytokines including IL2 and IL12, as well as increased tumor growth inhibition.

These data confirm BXCL701's novel MoA that converts "non-inflamed tumors" to "inflamed tumors" by inhibiting dipeptidyl peptidase 8-9 (DPP8-9), next-generation immune checkpoints covered in a recent *Nature* publication¹, and fibroblast activation protein (FAP), a major immune-suppressive mediator.

Over 700 patients have been treated with BXCL701. It has well-characterized pharmacokinetic, pharmacodynamic and safety profiles that do not overlap with those seen with immune-checkpoint inhibitors.

This study confirms the ability of the Company's proprietary AI-powered discovery engine to identify synergistic combinations to optimize the clinical benefit of ICI therapies.

Additionally the BXCL701 poster was selected by other investigators in the field as being of high interest for interaction, and Dr. Rastelli was invited to discuss the abstract at the "[Targeted Immunotherapeutics Interactome: Beyond Checkpoint Inhibitors](#)" session. The session will take place on Monday, April 3, 2017 from 4:45-6:00 p.m. EDT in the Marquis Ballroom Salons 1-2, Meeting Level 2 Marriott Marquis DC.

¹ Okondo, M. C. et al. DPP8 and DPP9 inhibition induces pro-caspase-1-dependent monocyte and macrophage pyroptosis. *Nature Chemical Biology* 13, 46–53 (2016).

About BioXcel

BioXcel is a biopharmaceutical company pioneering the integration of big data analytics and machine learning-based artificial intelligence with drug development expertise to advance the next wave of medicines, impacting the probability of success of drugs. Our focus is to develop innovative medicines that address immuno-oncology, neuroscience and rare diseases with high unmet medical need. Committed to innovation, product excellence and partner success, BioXcel's global collaborations span the biopharmaceutical ecosystem. We are headquartered in Branford, CT, USA with operations in Asia.

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