



BioXcel Therapeutics to Expand Study of BXCL701 into Multiple Advanced Solid Tumor Types

December 11, 2019

BXCL701 may improve patient response when combined with checkpoint inhibitors or re-sensitize patients who have failed checkpoint therapy

NEW HAVEN, Conn., Dec. 11, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology, today announced that the Company will advance the clinical evaluation of BXCL701 into multiple advanced solid tumors. BXCL701 is an orally active, systemic innate immunity activator with dual mechanisms of action.

"This new study allows us to accelerate the evaluation of BXCL701 in combination with a checkpoint inhibitor in patients with a range of tumor types where checkpoint inhibitors are standard of care," commented Vincent J. O'Neill, M.D., Senior Vice President and Chief Medical Officer of BTI. "We believe BXCL701 may have the potential to extend treatment responses to KEYTRUDA®, a PD-1 inhibitor, when used together to treat advanced solid cancers. This trial expands on our studies of BXCL701 in prostate and pancreatic cancers as we explore its full potential."

The open-label Phase 2 basket trial ([NCT04171219](https://clinicaltrials.gov/ct2/show/study/NCT04171219)) will take place at a leading cancer center in the U.S. The study is designed to evaluate the response rate of orally administered BXCL701, combined with Pembrolizumab (KEYTRUDA®) in patients with advanced solid cancers. If successful, the study would provide an option for patients who have failed or are refractory to checkpoint therapy. Outcome measures will include progression-free-survival, overall survival, and duration of response, as well as the safety of the combined treatment.

To learn more about the trial, visit <https://clinicaltrials.gov/>

About BXCL701:

BXCL701 is an investigational orally administered innate immune activator designed to initiate inflammation in the tumor microenvironment. Approved and experimental immunotherapies often struggle to address cancers that appear "cold" or uninfamed. Therefore, BXCL701 may render "cold" tumors "hot," making them more detectable by the adaptive immune system and thereby facilitating the development of a strong anti-cancer immune response. BTI's data supports BXCL701's synergy with both current checkpoint inhibitor-based therapies and emerging immunotherapies directed to activate T-cells, such as IL-2 and OX40 agonist antibodies.

This candidate is currently being developed as therapy for treatment emergent Neuroendocrine Prostate Cancer (tNEPC) and pancreatic cancer (both "cold" tumors) and other advanced solid cancers that are "hot" or have become resistant to checkpoint inhibitors. The safety escalation portion of the trial evaluating the double combination of BXCL701 and KEYTRUDA® for tNEPC is ongoing with data read-out expected in the first half of 2020. The FDA has also accepted an IND application for the triple combination of BXCL701, bempedegalesleukin (produced by Nektar Therapeutics, Inc., or Nektar) and BAVENCIO® (avelumab, Merck KGaA, Darmstadt, Germany and Pfizer) in pancreatic cancer. The triple combination trial is expected to begin following Nektar and Pfizer's safety run-in study of a double combination of bempedegalesleukin and avelumab and the outcome of that trial.

About BioXcel Therapeutics, Inc.:

BioXcel Therapeutics, Inc. is a clinical-stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically evaluated 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, an investigational sublingual thin film formulation in development for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an investigational orally administered systemic innate immunity activator in development for treatment of a rare form of prostate cancer, pancreatic cancer and advanced solid cancers in combination with other immuno-oncology agents. For more information, please visit www.bioxccltherapeutics.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 the timing and data from clinical development initiatives and trials for BXCL701 and the potential for BXCL701 to improve or extend treatment responses when combined with checkpoint inhibitors. When used herein, words including "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 BTI's current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BTI 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, its limited operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; its limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501 and BXCL701 and other product candidates; the failure of preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; its approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; its exposure to patent infringement lawsuits; its ability to comply

with the extensive regulations applicable to it; its ability to commercialize its product candidates; and the other important factors discussed under the caption "Risk Factors" in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2019 as such factors may be updated from time to time in its other filings with the SEC, which are accessible on the SEC's website at www.sec.gov.

These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While BTI may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing BTI's views as of any date subsequent to the date of this press release.

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