April 28, 2020

BioXcel Therapeutics Announces Initiation of a Phase 2 Study of BXCL701 in Combination with a PD-1 Inhibitor for Treatment Emergent Neuroendocrine Prostate Cancer

Completed the Phase 1b safety lead-in, identifying recommended Phase 2 dose of BXCL701 in combination with pembrolizumab (KEYTRUDA®) *in advanced prostate cancer*

Initial interim data readout expected in the fourth quarter of 2020

NEW HAVEN, Conn., April 28, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics ("BTI" or "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 the initiation of the Phase 2 efficacy portion of the Phase 1b/2 trial of BXCL701 in combination with pembrolizumab (KEYTRUDA®) for treatment emergent Neuroendocrine Prostate Cancer (tNEPC). Initial interim data from this study is expected in the fourth quarter of 2020.

Results from the Phase 1b safety assessment of BXCL701, an investigational oral innate immunity activator, indicated that a split dose totaling 0.6 mg per day is the recommended dose when used in combination with KEYTRUDA®, a PD-1 inhibitor. The 0.6 mg total daily dose has shown on-target side effects consistent with cytokine activation. Splitting the daily dose can be associated with an improved safety profile and will be used in the efficacy portion of the clinical program.

"Identifying the recommended Phase 2 dose of BXCL701, in combination with KEYTRUDA®, is a critical step for our immuno-oncology program, as it allows us to evaluate the anti-tumor activity in patients with tNEPC and other cancer types," commented Vincent J. O'Neill, M.D., Senior Vice President and Chief Medical Officer of BTI. "tNEPC is a rare and highly aggressive form of prostate cancer, the most common malignancy in men, with no current standard treatments. With the DPP8/9 target amplified in some tNEPC tumors, we believe BXCL701 has the potential to facilitate a strong adaptive immune response, making tumors more responsive to immunotherapies, including the PD-1 inhibitor pembrolizumab. We believe BXCL701 has potential to treat this subpopulation, and if successfully developed and approved, could fill the gap in prostate cancer treatment."

The Phase 1b/2 trial is an open-label, multicenter study to evaluate the safety and efficacy of BXCL701 administered orally and daily, combined with pembrolizumab (KEYTRUDA®), in men with tNEPC. With the initial assessment of safety and the identification of the recommended Phase 2 BXCL701/PEMBRO dose schedule complete, the Company can

now initiate the Phase 2 efficacy portion of the trial. Approximately 30 eligible tNEPC patients will receive 0.3 mg of BXCL701 twice daily (BID) on days 1 through 14 of a 21-day cycle plus 200 mg of pembrolizumab administered intravenously on day 1 and every subsequent 21 days. The primary endpoint of the trial is the composite response rate, with a target of achieving a greater than 15% composite response rate. Secondary endpoints include duration of response, overall survival and progression-free survival.

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

About Treatment-Emergent Neuroendocrine Prostate Cancer (tNEPC)

tNEPC is a 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 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 uninflamed. 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 preclinical data supports BXCL701’s synergy with both current checkpoint inhibitor-based therapies and emerging immunotherapies directed to activate T-cells, such as IL-2.

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.

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 and for treatment of pancreatic cancer in combination with other immuno-oncology agents. For more information, please visit http://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 the timing of and data readouts from the trials involving BXCL701 and the ability for BXCL701 to support the treatment of advanced prostate cancer. 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; impacts from the COVID-19 pandemic; its ability to commercialize its product candidates; and the other important factors discussed under the caption “Risk Factors” in its Annual Report on Form 10-K for the fiscal year ended December 31, 2019, as supplemented by its Current Report on Form 8-K filed on April 14, 2020, 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 and the Investors page of its website at www.bioxceltherapeutics.com*.

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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