BioXcel Therapeutics to Host BXCL701 Key Opinion Leader Day to Highlight Company's Investigational, Oral Innate Immune Activator for Rare Form of Prostate Cancer

February 8, 2023

NEW HAVEN, Conn., Feb. 08, 2023 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced that it will host a Key Opinion Leader (KOL) Day focused on its lead immuno-oncology program BXCL701 for the investment community on Tuesday, February 21, 2023 from 1:00 to 3:00 p.m. ET.

Vimal Mehta, Ph.D., Chief Executive Officer, and Vincent J. O'Neill, M.D., Chief R&D Officer, OnkosXcel Therapeutics, a wholly owned subsidiary of BioXcel Therapeutics, Inc., will be joined by a panel of leading oncology experts to review top-line Phase 2 data results being presented at the 2023 American Society of Clinical Oncology Genitourinary Cancers Symposium (ASCO GU), and the development path and future opportunities for BXCL701, which is currently being developed for the potential treatment of small cell neuroendocrine prostate cancer (SCNC), an aggressive variant of prostate cancer.

SCNC represents a rare, underserved, growing patient population, with SCNC cases increasing due to earlier and more widespread use of androgen receptor inhibitors. In 2022, there were an estimated 268,500[1] new prostate cancer patients, with approximately 10,740 patients progressing to SCNC.

Participating KOLs and discussion topics will include:

- **Daniel P. Petrylak, M.D.:** *Prostate Cancer Overview and Challenges with Current Immuno-therapy*

  Daniel P. Petrylak, M.D., is Professor of Medicine and Urology at Yale School of Medicine and a pioneer in the research and development of new drugs and treatments to fight prostate, bladder, kidney, and testicular cancer. At the Smilow Cancer Hospital, Dr. Petrylak's position as a national leader on clinical trials for men with prostate and bladder cancer has opened up a world of treatment options for patients in New England. Dr. Petrylak currently serves as either the principal investigator or co-principal investigator on seven Southwest Oncology Group (SWOG) clinical trials for genitourinary cancers. To date, he has authored more than 100 peer-reviewed articles on prostate and bladder cancer research.

- **Louis M. Weiner, M.D.**: *BXCL701 Mechanism of Action*

  Dr. Louis M. Weiner is Director of Georgetown Lombardi Comprehensive Cancer Center, one of 53 National Cancer Institute (NCI)-designated comprehensive cancer centers in the United States. He holds the Francis L. and Charlotte G. Gragnani Chair and is Professor of Oncology and Chair of the Department of Oncology at Georgetown University Medical Center. Dr. Weiner also serves as Director of the MedStar Georgetown Cancer Institute, a cancer service line for patients in the Washington, D.C. and Baltimore metropolitan areas. Dr. Weiner is known for his laboratory and clinical research focusing on new therapeutic approaches that mobilize the patient's immune system to fight cancer using monoclonal antibodies and related modalities of therapy. His current research focuses on identifying and therapeutically exploiting mechanisms employed by malignant cells to combat immune destruction, with a focus on pancreatic cancer.

- **Rahul Aggarwal, M.D.**: *Results of Phase 2 Trial of BXCL701 in Small Cell Neuroendocrine Prostate Cancer (SCNC)*

  Rahul Aggarwal, M.D., is Associate Director for Clinical Sciences, Helen Diller Family Comprehensive Cancer Center, and Associate Professor of Medicine at the University of California San Francisco (UCSF). His clinical practice focuses on patients with advanced solid tumor malignancies with a particular emphasis on genitourinary malignancies including prostate, kidney, bladder, and testicular cancer. Dr. Aggarwal serves as the Co-Leader for the GU Medical Oncology program at UCSF. His research focus is on developing novel therapies and imaging modalities for patients with advanced solid tumor malignancies, with a focus on patients with advanced prostate cancer and neuroendocrine prostate cancer in particular. Dr. Aggarwal serves as the principal investigator for the Phase 2 study of BXCL701 in SCNC.

Investors interested in attending the in-person event should RSVP by contacting Brennan Doyle at bdoyle@bioxceltherapeutics.com. To access a live webcast, please visit "News/Events" within the Investors & Media section of the Company's website 15 minutes prior to the start time of the presentation. A webcast replay and accompanying presentation materials will be available on the website following the event.

About Metastatic Castration-Resistant Prostate Cancer (mCRPC) and SCNC
mCRPC is a form of advanced prostate cancer that is no longer responding to testosterone-lowering hormone treatments and has spread to other

areas of the body such as the lymph nodes, bones, the bladder, rectum, liver, or lungs. SCNC is a particularly difficult-to-treat histologic subtype of mCRPC that emerges in approximately 20% of mCRPC patients, though this number is increasing due to earlier and more widespread use of androgen blockers.

About BXCL701
BXCL701 is an investigational, oral 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. BioXcel Therapeutics’ preclinical data supports BXCL701’s synergy with both current checkpoint inhibitor-based therapies and emerging immunotherapies directed to activate T-cells. BXCL701 is currently being developed as a potential therapy for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. BXCL701 has received Orphan Drug Designation from the U.S. Food & Drug Administration in four indications: acute myelogenous leukemia, pancreatic cancer, stage IIb to IV melanoma, and soft tissue sarcoma. An 800-patient clinical database supports the development of BXCL701.

About OnkosXcel Therapeutics, LLC
OnkosXcel Therapeutics, LLC is a wholly owned subsidiary of BioXcel Therapeutics, Inc., focused on developing transformative medicines in oncology utilizing artificial intelligence approaches. The subsidiary was formed in 2022 to develop BXCL701, a Phase 2, investigational, oral innate immune activator for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors, as well as other immuno-oncology focused assets.

About BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc., is a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. The Company’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 indications. The Company’s commercial product, IGALMI™ (developed as BXCL501), is a proprietary, sublingual film formulation of dexmedetomidine approved for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. The safety and effectiveness of IGALMI have not been established beyond 24 hours from the first dose. For more information, please visit IGALMIhcp.com and also see the IGALMI full Prescribing Information. BXCL501 is under evaluation for at-home use for the acute treatment of agitation in bipolar and schizophrenia patients, for acute treatment of Alzheimer’s-related agitation, and as an adjunctive treatment for major depressive disorder. The safety and efficacy of BXCL501 for these uses have not been established. The Company is also developing BXCL502 as a potential therapy for chronic agitation in dementia. Under its subsidiary, OnkosXcel Therapeutics LLC, the Company is developing BXCL701, an investigational, oral innate immune activator for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. The safety and efficacy of BXCL502 and BXCL701 have not been established. For more information, please visit bioxceltherapeutics.com.

Forward-Looking Statements

This press release includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the “Securities Act”) and Section 21E of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, the date, time and content of the BXCL701 Key Opinion Leader Day. When used herein, words including “anticipate,” “believe,” “can,” “continue,” “could,” “designed,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “might,” “plan,” “possible,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. 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 the Company’s current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. The Company 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, the important factors discussed under the caption “Risk Factors” in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2022, 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 the Company 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 the Company’s views as of any date subsequent to the date of this press release.

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Source: BioXcel Therapeutics, Inc.

1 American Cancer Society's estimates for prostate cancer in the United States for 2022