BioXcel Therapeutics, Inc. (BTI) Is Launched with Phase 2-ready Programs Addressing Unmet Needs in Immuno-oncology and Neuroscience

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BTI is launched by BioXcel Corp. to advance BXCL701 as a single agent and in synergy with PD-1/Checkpoint Inhibitors for Castration Resistant Prostate Cancer and BXCL501 for acute agitation in schizophrenic and bipolar patients

BRANFORD, Conn., May 10, 2017 (GLOBE NEWSWIRE) -- BioXcel Corporation, a privately held biopharmaceutical company pioneering the integration of big data analytics and machine learning-based artificial intelligence (AI) with drug development expertise to advance the next wave of medicines, today announced the formation of BioXcel Therapeutics, Inc. (BTI), to focus on the development of lead programs in immuno-oncology, BXCL701, and in neuropsychiatric and neurodegenerative diseases, BXCL501.

Using its proprietary AI-powered R&D engine, BioXcel has identified a number of highly promising therapeutic candidates with applications across the therapeutic spectrum. BTI was established as a biotechnology company to capitalize on these promising therapeutic candidates with the mission of developing innovative treatments to address unmet medical needs in immuno-oncology and neuroscience. BTI's drug re-innovation approach has significantly de-risked both BXCL701 and BXCL501, and this will further result in future drug candidates with the potential to provide therapeutic benefit to patients, while carrying lower development and regulatory risks.

Vimal Mehta, Ph.D., co-founder, Chairman and Chief Executive Officer of BioXcel has been appointed Chairman and Chief Executive Officer of BTI, Frank Yocca, Ph.D., has been appointed Chief Scientific Officer and Luca Rastelli, Ph.D., has been appointed Vice President of Oncology and Translational Sciences of BTI. In addition to Dr. Mehta, BioXcel Therapeutics' Board of Directors includes accomplished pharmaceutical industry executives:

- **Peter Mueller, Ph.D.**, joins the BTI Board of Directors with more than 30 years of pharma and biotech expertise through senior-level positions at leading companies such as Axcella Health, Inc., where he served as Chief Scientific Officer and President of Research & Development, Vertex Pharmaceuticals, Inc. and Boehringer Ingelheim Pharmaceuticals, Inc. During his career, Dr. Mueller has been responsible for the development and approval of several drug products including Spiriva®, Combivent®, Atrovent®, Incivek® and Kalydeco®, as well as the development and NDA/MAA submission for Orkambi®. Dr. Mueller is President of The Mueller Health Foundation, an organization he created, dedicated to the eradication of global, lethal infectious diseases, with a primary focus on tuberculosis.

- **Eric Rowinsky, M.D.**, brings to BTI's Board of Directors nearly 30 years of experience in oncology drug development. As Chief Medical Officer of ImClone Systems, he led the development of Erbitux® (cetuximab), and has contributed to the development programs for several oncology drugs such as ramucirumab, necitumumab, paclitaxel, docetaxel, topotecan, erlotinib, irinotecan, lapatinib, and cixutumumab, among others. Dr. Rowinsky currently serves as a Director of a number of biotechnology and pharmaceutical companies including Biogen, Inc. (NASDAQ:BIIB), Fortress Biotech, Inc. (NASDAQ:FBIO) and Verastem, Inc. (NASDAQ:VSTM).

- **Krishnan Nandabalan, Ph.D.**, is co-founder, President and Chief Scientific Officer of BioXcel Corporation. Dr. Nandabalan is a pioneer and innovator in the application of AI to biopharma R&D. In addition to his work at the intersection of technology and biopharmaceutical R&D, Dr. Nandabalan has played important roles in licensing, discovery and development of oncology and neuroscience therapeutics and diagnostic products.

"We have formed an incredibly talented leadership team and Board of Directors to support the development of BTI's lead clinical programs. Each member of the organization has played a critical role in the successful expansion and advancement of clinical pipelines within leading pharmaceutical and biotechnology companies. This leadership team brings a deep understanding of both immuno-oncology and neuroscience, making them ideally suited to maximize the potential of BTI's pipeline and accelerate the company's strategy," said Dr. Vimal Mehta, Chairman and CEO of BTI.

BXCL701 is a first-in-class DPP8-9/FAP inhibitor with a well-established mechanism of action, PK/PD and safety profile. BTI is initially developing BXCL701 in Castration Resistant Prostate Cancer (CRPC), both as monotherapy and in combination with other immuno-oncology agents. BXCL701 has broad immuno-modulatory properties, making it suitable for combination with other therapeutic modalities, vaccines, CAR-T and CAR-NK and targeted therapies. The Company believes that BXCL701 has the potential to be applied to a variety of other tumor types with high unmet medical need, including pancreatic cancer, non-small cell lung cancer (NSCLC) and head and neck cancers, among others.

BXCL501 is a first in class selective alpha 2a receptor agonist for treating acute agitation in schizophrenic and bipolar patients. BXCL501 is a proprietary sublingual formulation of an FDA-approved anesthetic that has demonstrated anti-agitation effects both pre-clinically and clinically. Given the established profile of BXCL501, BTI plans to pursue regulatory approval of BXCL501 via the 505(b)(2) pathway. BTI believes BXCL501 addresses a very important need in the treatment of agitation, and is both patient and caregiver centric.

"The establishment of BTI is a significant milestone for BioXcel Corporation, and an important step toward achieving our goal of maximizing the value

of these highly promising therapeutic programs. Our lead programs are clinically-validated drug candidates targeting areas of high unmet medical need, each with a clear path to market," added Dr. Mehta. "We are on-track to initiate human proof-of-concept trials of both BXCL701 and BXCL501, and are excited for the opportunities that lie ahead as we advance the development of these emerging therapeutic candidates."

About BioXcel Therapeutics, Inc. (BTI):
BioXcel Therapeutics, Inc. is engaged in the development and advancement of the next wave of medicines, initially targeting the treatment of immuno-oncology and neuroscience diseases. The company's lead therapeutic candidates are BXCL701, a first-in-class DPP8-9/FAP inhibitor with broad potential application in oncology indications, both as a monotherapy and in combination with immuno-oncology agents, and BXCL501, a proprietary sublingual formulation of an anesthetic for the treatment of acute agitation, with the potential to expand into other neuropsychiatric and neurodegenerative disorders. The company's strategy is to apply a drug re-innovation approach to develop therapeutic candidates with a high probability of clinical and regulatory success. For more information, please visit www.bioxceltherapeutics.com.

About BioXcel Corporation:
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. The company's 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 Corporation's global collaborations span the biopharmaceutical ecosystem. Headquartered in Branford, CT, USA, the company is supported by a R&D Center of Excellence in India. For more information, please visit www.bioxcel.com.

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