BioXcel's BXCL101, Receives Orphan Drug Designation from the U.S. FDA for the Treatment of Patients with Neurofibromatosis Type 2 (NF2)

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First therapeutic candidate to emerge from Company's R&D Engine to be granted Orphan Drug Designation

BRANFORD, Conn., Sept. 13, 2016 (GLOBE NEWSWIRE) -- BioXcel, a privately held biopharmaceutical company based in Connecticut, today announced that the U. S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to BXCL101 for the treatment of Neurofibromatosis Type 2 (NF2), an orphan disease with significant unmet medical need. BXCL101 is the first and only systemic therapy being developed to eliminate existing lesions and prevent the formation of new lesions by targeting the molecular mechanism of NF2 pathophysiology.

BXCL101 is a proprietary version of an approved drug, bortezomib, adapted for chronic use in NF2 patients with both a novel dosing regimen and delivery approach. NF2 is a rare disease associated with neurologic and ophthalmologic abnormalities caused by benign tumors of the brain, spinal cord and peripheral nerves. BXCL101 is the first drug candidate discovered using BioXcel's R&D Platform, to be granted orphan drug status by the FDA. BioXcel is advancing the clinical development of BXCL101 and is exploring global partnering opportunities.

"Securing Orphan Drug Designation from the FDA's Office of Orphan Products Development (OOPD) is an important milestone for BioXcel. Moreover, it provides a crucial regulatory validation of our business model, to identify clinical candidates that address areas of high unmet medical need and bring them quickly to confirmatory clinical trials," said **Dr. Vimal D. Mehta, CEO and Chairman of BioXcel.**

"BXCL101 represents a unique solution to address this rare and debilitating disease, where the current standard of care is not sufficient to satisfy patient needs," **added Dr. Krishnan Nandabalan, President and CSO of BioXcel.** "We believe that BXCL101 has the potential to significantly improve the lives of patients with NF2 and we look forward to advancing its development, as well as further growing our therapeutic pipeline with additional drug candidates."

Under the U.S. Orphan Drug Act, the FDA's Office of Orphan Products Development provides special status and incentives to encourage the development of drugs for diseases affecting fewer than 200,000 people in the U.S. This designation makes BXCL101 eligible for a seven year marketing exclusivity period. In addition, it provides certain incentives, including federal grants, prioritized consultation by the FDA on clinical studies, tax credits and a waiver of PDUFA filing fees. The orphan designation does not alter the standard regulatory requirements, timing and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies.

About Neurofibromatosis Type 2
Neurofibromatosis Type 2 is a rare disease with high morbidity, reduced lifespan and no systemic treatment. NF2 is caused by inactivation of the tumor suppressor gene, NF-2, which encodes the protein, merlin (m-NF2) also known as schwannomin. The prevalence of NF2 in the US may be as high as 1:25,000, translating to slightly more than 5, 000 people living with this disease. Typically, patients suffer from symptoms such as hearing loss, tinnitus, visual impairment, imbalance, and in many cases develop non-malignant brain tumors called meningiomas.

About BXCL101
BXCL101 is a reversible inhibitor of the chymotrypsin-like activity of the 26S proteasome in mammalian cells. The 26S proteasome is a large protein complex that degrades ubiquitinated proteins. By reversing the molecular effects of m-NF2, BXCL101 is expected to impact the lesions, schwannomas and meningiomas that are responsible for the debilitating aspects of NF2. BioXcel's R&D Platform powered by proprietary artificial intelligence and big data algorithms uncovered the complex relationship between NF-2 and the ubiquitination pathway, which was confirmed by preclinical studies and preliminary human data.

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 and neuroscience 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.

Contact Information:
Anita Ganjoo
Corporate Communications
203 957 2487

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