



BioXcel Expands Into CNS Orphan Disease Therapeutics

February 8, 2016

-Novel Approach for Treatment of Rare and Ultra-Rare CNS Diseases-

BRANFORD, Conn., Feb. 08, 2016 (GLOBE NEWSWIRE) -- BioXcel Corporation, a global leader in cloud-based big data platforms and developer of transformative patient treatments, today announced its expansion into CNS Orphan Diseases that require novel treatment solutions for neurological manifestations, including neurodegenerative disorders, motor dysfunction disorders and lysosomal storage disorders. BioXcel will utilize its Big Data Innovation Lab and PharmGPS® Orphan Disease Suite to discern common pathophysiological mechanisms in Central Nervous System (CNS) related disease clusters and identify potential disease modifying agents. These CNS related disease clusters account for approximately one third of the 9,000 currently documented rare and ultra-rare diseases, more than 250 of which have a significant need for transformative care.

Vimal Mehta, Ph.D., BioXcel's Chairman and Chief Executive Officer, commented, "The CNS orphan disease space represents an underserved therapeutic area due to the high unmet medical need and lack of innovative treatments. We believe that our unique approach is capable of yielding multiple clinical programs leading to human proof-of-concept studies and, potentially, an accelerated path to market for our therapeutic candidates."

Through its big data approach, the Company will assess all currently marketed drugs, as well as late stage pharmacological agents which have not met primary clinical end points but offer repositioning potential, to decipher therapeutic value by linking novel connections between pathophysiology of these diseases, mechanisms of action and the pharmacological space.

"We are entering into partnerships with large pharmaceutical companies with a repertoire of clinical agents and leveraging the capabilities of our first-in-class big data-powered platform to discover breakthrough therapeutics," said BioXcel's **Senior Vice President, CNS R&D, Frank D. Yocca, Ph.D.** "The extension of our platform into CNS orphan disease therapeutics represents an important step forward in our evolution into a big data-driven drug development company. We believe that our unique approach could transform the treatment landscape for a wide range of serious CNS conditions."

BioXcel's therapeutic pipeline includes a drug candidate with established safety and tolerability, repositioned for treatment of symptoms of hyperarousal and agitation in neurodegenerative disorders associated with dementia. The mechanism of action overlaps with the pathophysiology of hyperarousal and agitation in various neurodegenerative disorders, a discovery made possible through BioXcel's Big Data Innovation Lab; exponents of the lab include industry leaders like Takeda, Alexion, Pronutria and many others.

Big Data Innovation Lab (BDI-Lab) and PharmGPS® Orphan Disease Suite:

Our BDI-Lab is built on three key pillars – PharmGPS®, a live cloud-based big data analytics platform built on proprietary algorithms and predictive analytics, multi-disciplinary teams of researchers based in our Integrated Center of Xcellence and a highly experienced and therapeutically aligned drug development team. Our live and multi-dimensional PharmGPS® platform leverages BioXcel's proprietary Orphan Disease Suite that encompasses more than 9,000 rare and ultra-rare diseases, 4,000-5,000 associated genes, 1,500 disease pathways, and distinct target-indication tiles for antibody, protein, RNA, small molecule and gene therapy modalities. Partners can make informed decisions related to rare disease indications, and associate the ideal mode of pharmacotherapy, factoring in strategic, medical, scientific and commercial considerations. The suite enables the commercialization of orphan drugs via de novo drug discovery, or repurposing and reformulation of clinical assets (drug rescue) based on disease severity, gene ontology, disease pathways, proteinopathy, standard of care, emerging innovation, enabling technologies, and current drug pipeline – all of which are crucial when addressing the high unmet need of patients with rare and ultra-rare diseases.

About BioXcel

BioXcel Corporation is a global leader in cloud-based big data platforms and developer of transformative patient treatments. Our first in class Big Data Innovation Lab is focused on the development of transformative patient treatments for strategic portfolio growth and leverages; PharmGPS® - a live analytics platform; an Integrated Center of Xcellence housing multidisciplinary scientific, commercial and medical teams; and an executive team that offers deep domain expertise. 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:

BioXcel Corporation
Anita Ganjoo
Corporate Communications
Email: aganjoo@bioxcel.com
Tel: 203 957 2487

Source: BioXcel Corporation