

BioStrategies LC awarded NIH grant to develop an enzyme therapeutic for GM1 Gangliosidosis

November 1, 2014

The National Institute of Neurological Disease and Stroke (NINDS) has awarded a Small Business Innovative Research Phase I Grant to BioStrategies LC to develop a targeted enzyme replacement therapy for the neurodegenerative rare disease, GM1 Gangliosidosis. Principle investigators on the project are BioStrategies' Drs. David N. Radin (lead) and Carole L. Cramer, and GM1 Gangliosidosis expert, Dr. Alessandra d'Azzo of St. Jude Children's Research Hospital.

GM1 Gangliosidosis is a "lysosomal storage disease" caused by an inherited gene defect. Patients with the disease are unable to break-down certain complex lipids, which then accumulate to pathological levels particularly in cells of the central nervous system. The disease is marked by progressive neurodegeneration and the infantile forms are particularly devastating to patients and their families. Enzyme replacement therapies (ERTs) have been effective in treating several lysosomal storage diseases. However, no ERT exists for GM1 Gangliosidosis, with efforts hindered by the significant technical challenge of getting the corrective protein delivered into the brain. According to Radin, "BioStrategies LC has developed a novel protein carrier technology that we believe will enable enzyme delivery across the blood-brain-barrier. Our goal in this project is to produce carrier – enzyme fusions and determine whether they access the brain and reduce the lysosomal pathology in the mouse model of GM1 gangliosidosis." This project will also test the feasibility of BioStrategies' plant-based bioproduction platform for producing this complex protein drug. Plants bring key advantages in safety and the speed and cost of large-scale production of complex human proteins.

About BioStrategies

BioStrategies LC is a biotech company focused on the development of innovative protein-based therapeutics for human health and veterinary applications. The company has received multiple grant awards from NIH and other federal and state agencies and received the prestigious SBA Tibbett's Award in 2012 for innovative research excellence in the national Small Business Innovative Research Program (SBIR). This award recognized the company's pioneering research to develop novel enzyme replacement therapeutic (ERT) technology, which employs biotechnology approaches for production of the human enzyme in a safe, low cost plant-based production system for the rare genetic condition, Gaucher's Disease. This innovative technology was the foundation for Protalix' plant-produced ERT drug for Gaucher's, Eleyso, which in 2012 became the first plant-produced biologic drug to receive FDA approval and is currently marketed worldwide by a partnership between Pfizer and Protalix. BioStrategies continues to bring novel technologies to therapeutics for rare disease with the development of new lectin-based protein delivery systems. The company's lectin-ERT fusion proteins are designed to increase ERT drug effectiveness by enhancing delivery to cells and tissues of the body that are not treated adequately by existing ERT drugs and drug delivery mechanisms.

Contacts:

David Radin, Ph.D.
Managing Director, BioStrategies LC
870-8978-7310; www.biostrategies-lc.com