NEWS RELEASE
For Immediate Distribution

October 19th, 2021

Phoenix Nest Biotech-Awarded a $3.4 million National Institute of Health Grant

Brooklyn, NY- Phoenix Nest Inc., is on a mission to treat the ultra-rare disease, Sanfilippo syndrome types C and D. This is the 4th in a series of NIH grants totaling $10.7 million awarded to Phoenix Nest relating to the research and development of an enzyme replacement therapy (ERT) for Sanfilippo syndrome type D, a rare lysosomal storage disorder.

The current $3.4 million grant (Award Number UB1NS122644) from the National Institute of Neurological Disorders and Stroke of the National Institute of Health will help Phoenix Nest execute a retrospective and prospective observational study in the global Sanfilippo type D patient population. Patient enrollment is expected to begin March 2022.

This study will use a novel application to capture real world patient video recordings to supplement the clinical data obtained in the hospital setting. Our goal is to identify endpoints to assess clinical benefits from experimental therapies during an interventional trial.

"Our MPS IIID ERT program is making steady progress in nonclinical and manufacturing development. We are excited to initiate this clinical trial funded by NINDS, it puts us a step closer to bringing our promising experimental therapy to the patients". - Srikanth Singamsetty PhD, Scientific Director, Phoenix Nest Inc.

"*I couldn't be more thrilled to be a part of this study. MPS IIID has been ignored for too long, these patients are in desperate need of a treatment. Phoenix Nest has done an incredible job in designing an observational study that encompasses all aspects of daily living activities, disease progression and clinical assessments for a disease that has yet to establish a natural history. I look forward to executing the study with my esteemed coPI, Dr. Pramod Mistry at Yale University" -Heather Lau, MD Yale Lysosomal Disease center.*

Patient families and physicians interested in hearing more about this study are encouraged to contact the study coordinator, Ruby Yang at Yale Lysosomal Disease center: Ruhua.yang@yale.edu or call 203-785-3197.

About Phoenix Nest:

Phoenix Nest is a bespoke biotech focused on treatments for Sanfilippo syndrome specifically subtype's C and D. Co-Founded by the parent of a child suffering from Sanfilippo syndrome type C, we believe that no disease should be deemed too rare to treat. We are open to new opportunities to collaborate. If interested, please contact jwood@phoenixnestbiotech.com
Visit us at https://www.phoenixnestbiotech.com

About Sanfilippo syndrome:

Sanfilippo syndrome, is an inherited mutation in one of the four enzymes required to catabolize heparan sulfate (HS). The four different affected enzymes create four subtypes of the disease denoted as A, B, C and D. Pathogenic mutations in one of the four enzymes, results in a progressive accumulation of undegraded HS-GAGs in all cells of the body. The primary target organ for this disease is the central nervous system (CNS). Sanfilippo syndrome is an insidious disease, children appear normal at birth; achieving developmental milestones including the ability to walk and talk. The central pathologic features of Sanfilippo syndrome are neurologic; there is a stagnation of cognitive development, severe behavioral problems, progressive cognitive decline, prolonged periods of sleeplessness, dementia, and decline in motor skills that steadily lead to immobility, unresponsiveness, and death.