First observational study initiated in Sanfilippo syndrome type D

Potential lead-in trial for interventional study of novel enzyme replacement therapy

New York, January 10, 2023- Phoenix Nest, a company founded on the love of parents and caregivers to treat their children affected with Sanfilippo syndrome announces today the launch of ALL-127, a Natural History Study (NHS) of Sanfilippo syndrome type D. This will be a single-center site performed at Columbia University Irving Medical Center. The study will evaluate patients with Sanfilippo syndrome type D to assess the course of the syndrome and may act as a lead-in to an interventional trial of ALL-027, an enzyme replacement therapy in Phoenix Nest's pipeline.

Sanfilippo syndrome type D is a rare, fatal neurodegenerative genetic disorder Children appear normal at birth and present with speech and cognitive delays in early childhood, followed by hyperactivity and behavioral disorders. Prior to receiving a Sanfilippo diagnosis children are usually diagnosed with Autism. Sanfilippo syndrome is often dubbed as childhood Alzheimer's.

"*This is an underserved patient population in desperate need of treatments. There are no approved treatments and very few drugs available to alleviate the symptoms. This study will help us further understand the course of the disease and define appropriate endpoints for our planned interventional study.* Says: Jill Wood, CEO of Phoenix Nest and parent of a child affected by Sanfilippo syndrome

About the Natural History Study

ALL-127 encompasses a range of assessments chosen to capture the patients' cognitive abilities, mobility, and clinical changes. Up to 10 participants from around the world are expected to enroll and come to the site for 3 annual visits. These participants will also provide video capture remotely via their smart phone. Video capture will be completed on the 'RARE' app every 6 months over the course of 2 years. The natural history study is designed to capture the disease progression for each child and is intended to act as the lead-in for an interventional trial of Phoenix Nest's lead therapy, ALL-027. The study will also determine clinically meaningful outcome measures to be used in future interventional trials.

"Natural history studies in rare diseases like Sanfilippo syndrome are a critical preparatory step to provide a baseline against which to compare outcomes in studies of new treatments. Data

from this study will provide critical information to plan a future clinical trial," says: Wendy Chung, MD, PhD, a clinical geneticist at Columbia University Irving Medical Center and principal investigator of the study.

The study is supported by a $3.4 million grant from the National Institute of Neurological Disorders and Stroke of the National Institute of Health (NIH/NINDS) under award number UB1NS122644.

About the Recording Application for Real-world Evidence (RARE) app
The RARE app will assess 9 different tasks videotaped by the patient's caregiver via their smart phone. The tasks include activities of daily living and designed to capture small changes that would otherwise be hard to document in a clinical setting. Sanfilippo syndrome causes hyperactivity, behavioral problems, and the loss of sight and hearing making it difficult to impossible for clinicians to assess the child's medical condition and cognitive functions during study visits. For more information about ALL-127 you can find it at clinicaltrials.gov

About Sanfilippo syndrome type D
Sanfilippo syndrome more formally known as mucopolysaccharidosis type III (MPS III), is part of a family of rare and fatal hereditary lysosomal storage disorders. Sanfilippo syndrome leads to accumulation of toxic material in cells throughout the body, particularly the brain. Sanfilippo syndrome type D is one of the four subtypes of Sanfilippo A-D and is thought to be the rarest form. Individually Sanfilippo syndromes are ultra-rare but combined have an incidence of 1 in 70,000 births. There are no approved treatments for any form of Sanfilippo syndrome.

About Phoenix Nest
Phoenix Nest is focused on developing life-changing medicines for patients and their families affected by Sanfilippo syndrome. We partner with leading academic researchers, patient advocacy organizations, and caregivers to bring therapies for Sanfilippo syndrome to fruition. To date Phoenix Nest has been the recipient of 6 NIH/NINDS grants and is actively seeking partnerships to help bring their gene therapy program for Sanfilippo type C, JLK-247 and enzyme replacement therapy for type D, ALL-027 to the clinic. For more information, visit phoenixnestbiotech.com

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"potential," "possible," and other similar expressions are intended to identify forward-looking statements. These forward-looking statements include without limitation statements regarding Phoenix Nest's pipeline of development candidates and observational studies. These forward-looking statements involve risk and uncertainties, many of which are beyond Phoenix Nest's control. Known risks include, among others: Phoenix Nest may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory or grant milestones and timelines, its reliance on third-parties, clinical development plans, manufacturing processes and plans, and bringing its product candidates to market, including the ongoing COVID-19 pandemic, dependence on outside funding sources such as the National Institute of Health funding mechanisms, unexpected manufacturing setbacks that may not be resolved in a timely manner, potential disagreements or other issues with our third-party partners and collaborators, and regulatory, court or agency feedback or decisions, such as feedback and decisions from the United States Food and Drug Administration or the United States Patent and Trademark Office. Any of the foregoing risks could adversely affect Phoenix Nest's business plans and drug development. You should not place undue reliance on the forward-looking statements contained in this press-release.