Phoenix Nest Initiates Natural History Study of Sanfilippo Syndrome type C

First comprehensive natural history study in Sanfilippo syndrome type C approved for enrollment

Brooklyn, New York- October 30th, 2024
Phoenix Nest Inc., a niche biotech focused on developing treatments for Sanfilippo syndrome announces today that the French Committee for the Protection of Persons and CNIL has approved a natural history study of patients with Sanfilippo syndrome type C.

JLK-447 is the first comprehensive natural history study of patients affected by Sanfilippo syndrome type C (NCT05825131). This is an observational study; no drug will be investigated. JLK-447 will document and follow the symptoms of the disease. The data collected will be used to better define clinical outcomes and potential treatment effects in Phoenix Nest's future gene therapy clinical trial. To date there are no approved treatments for this disease.

Phoenix Nest CEO and parent of a child living with the disease, Jill Wood said: "I could not be more thrilled to announce the launch of this long-awaited natural history study of children affected by Sanfilippo syndrome type C. Sanfilippo is caused by a single gene defect which lends itself well to gene therapy. Unfortunately, due to the rarity of Sanfilippo it's been up to the patient caregivers to financially drive the science towards a treatment and initiate natural history studies to support the development of these treatments. Parents shouldn't be told that their child has a terminal genetic disease that has no treatment. The science is here now. These rare genetic diseases can be treated, it's just a matter of funding. I thank the National Institute of Health and the patient organizations that have supported this study."

"Sanfilippo syndrome is a devastating disorder that robs children of their skills and causes premature death. I have cared for several patients affected by Sanfilippo syndrome over the years and have seen first-hand the destructive cognitive and physical decline in these children. It is heartbreaking. My team and I are honored to be a part of this unprecedented study and wish to make a difference in the lives of these children and their families." Said Dr. Nathalie Guffon head of the reference center for inherited metabolic diseases at Hospices Civils de Lyon, France and one of two principal investigators conducting the study.

About Sanfilippo syndrome type C

Sanfilippo syndrome type C is a lysosomal storage disorder and part of the larger group of mucopolysaccharidosis (MPS) disorders. Sanfilippo syndrome has 4 subtypes A-D and is also known as MPS IIIC, each subtype refers to one of the four missing genes responsible for breaking down and recycling a sugar molecule called Heparan Sulfate

(HS). Sanfilippo syndrome type C is estimated to occur in 1 in 1 million births. HS builds up in all cells causing a catastrophic cascade of defects, the brain being most affected. Children with Sanfilippo appear normal at birth making it difficult to diagnose them in early childhood. Children present with behavioral issues and developmental delays and are often diagnosed as autistic. They may remain under the autism diagnosis for many years until they start to regress. The children lose their ability to talk, walk, and eat on their own eventually succumbing to a premature death.

Dr. Steven Gray, the co-director of the UTSW Gene Therapy Program and principal investigator of Phoenix Nest's gene therapy program said: "I have been working with patient organizations and biotech in developing treatments for ultra-rare diseases for 2 decades now. While we have seen gene therapy approvals for the rapidly progressing diseases like Batten and SMA, slower progressing diseases like Sanfilippo type C and GAN have been more challenging, in part due to gaps in natural history data and FDA-appropriate surrogate biomarkers. The data collected from the JLK-447 study will be integral to the success of measuring endpoints for clinically meaningful benefit in any future clinical trials for Sanfilippo type C."

About JLK-447

The studies defining clinical outcome assessment is a novel video application named C-RARE Recording Application of Real-world Evidence for Sanfilippo syndrome type C. The app is propriety technology licensed from Aparito, a wholly owned subsidiary of Eli Lilly and Company. Patient caregivers download the app onto their smart phone. From there they are given instructions on how to use C-RARE to film videos of their children performing activities of daily living. Such as eating a snack, playing a game and washing hands. The C-RARE video activities resemble the clinical outcome assessments and questionnaires given in the clinic. Children with Sanfilippo syndrome have difficult behaviors, short attention span, brain damage and limited to no speech. Making typical assessments used in a clinical setting problematic in assessing the child's actual capabilities. These videos will be completed at home in the child's natural environment.

JLK-447 will have two sites. Hospices Civils de Lyon is anticipated to start enrolling next month. The second site is planned to take place at UT Southwestern, Texas and is funded by a generous grant of $2,995,000 from the National Institute of Neurological Disorders and Stroke, Small Business Innovation Research program award # 1R44NS137892-01. Time of enrollment has not been determined yet. Funding for the French site activities was provided by French patient organizations: Vaincre les Maladies Lysosomales and Sanfilippo Sud. You can find more about the study on clinicaltrials.gov. (NCT05825131).

About Phoenix Nest

Phoenix Nest is a niche biotech focused on the treating Sanfilippo syndrome. The name Phoenix Nest comes from the mythological bird. The Phoenix made her nest out of

medicinal herbs: frankincense and myrrh. Like the Phoenix, parents of chronically ill children line their homes with medicinal treatments. In hopes that their children will grow up healthy enough to leave their nest someday. To date Phoenix Nest has only received funding through NIH/NINDS STTR/SBIR federal grants and patient organizations. Learn more about the company through direct contact- Jill Wood, CEO, jwood@phoenixnestbiotech.com 917-909-0553.

Forward Looking Statements

This press release contains “forward-looking statements.” Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “anticipates,” “expects,” “believes,” “plans,” “will,” “intended,” “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, 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.