Family Foundations Fund Critical Study Necessary For Clinical Trials And Enter Into Agreement With Nationwide Children's Hospital To Ensure Data Access For Lysosomal Storage Disease MPS III

Boston, MA – May 7, 2013
Nearly 50 years to the day since Dr. Sylvester Sanfilippo first characterized the disease Mucopolysaccharidosis (MPS) III in a presentation to the American Pediatric Society, three parent-run foundations have awarded investigators at Nationwide Children's Hospital in Columbus, OH with a $550,000 grant to support a natural history study (NHS) of Sanfilippo Syndrome Types A & B. This is a crucial step, led by Dr. Kevin Flanigan, Center for Gene Therapy, and co-investigator Dr. Kim McBride, Center for Human & Molecular Genetics, towards bringing the gene therapy research of Drs. Haiyan Fu and Douglas McCarty, Center for Gene Therapy, to human clinical trials targeted for 2014.

Natural history studies are an important tool for understanding the untreated course and progression of the disorder, determining clinical outcome measures and identifying potential participants for clinical trials. Data collected from these types of studies are not typically shared between researchers other than through published papers, which often lag the studies by months or years. Since rare diseases like Sanfilippo Syndrome have small patient populations, participating in multiple studies places a burden on affected children and their families. That is why the Sanfilippo Research Foundation (Ben's Dream), The Children's Medical Research Foundation, Inc. (A Cure for Kirby) and The Sanfilippo Children's Research Foundation (A Life for Elisa) worked closely with Nationwide Children's to ensure that data collected from this study would be available to other qualified institutions.

"We traveled with our daughter Blair from Florida to Minnesota to participate in a study sponsored by industry and while I am very excited by the progress of research, the idea of traveling and putting her through those tests again is daunting. I am so pleased that Nationwide has agreed to share the data collected in their study. I think this will ease the burden on families, and quicken the pace of research worldwide." Susan Chapin, Orlando, Florida

Sanfilippo Syndrome, is a group of four devastating lysosomal storage diseases, each caused by a deficiency in an essential enzyme. Children with Sanfilippo Syndrome appear normal at birth, but develop severe progressive developmental delays and neurological disorders. Sanfilippo Syndrome is a fatal disease and there is currently no treatment available. Drs. Fu and McCarty have developed novel, non-invasive gene therapy approaches that involve delivery of the corrected gene with a simple intravenous injection.

"Although neurological impact is the major problem, Sanfilippo Syndrome affects the entire body," said Dr. Fu. "Therefore we need to treat the entire nervous system and as many organs as possible in order to have the best outcome. Our gene therapy technique is minimally invasive and shows widespread expression throughout the whole body. Without the funding from these organizations we wouldn't be on the edge of clinical trials—more is still needed to do all the work necessary to ensure we're successful in treating the disease."

In addition to the grant for the NHS, the three foundations have collaborated since 2010 to provide over $610,000 in funding to advance the gene therapy research at Nationwide Children's for Sanfilippo Syndrome. This support enabled the Nationwide Children's team to garner two additional NIH grants totaling $3.75M to advance their gene therapy work to the point of submission for an Investigational New Drug (IND) application to the FDA for clinical trials for which a NHS is required.

About the Sanfilippo Research Foundation, Inc.
The Sanfilippo Research Foundation, Inc. was formed by Jennifer and Stuart Siedman in honor of their son Benjamin's fifth birthday. Ben's Dream has raised over $1.2 million dollars for research and has been supporting the work of Dr. Haiyan Fu at Nationwide Children's since 2004. The Sanfilippo Research Foundation commits 96 cents of every dollar raised to research.

For more information: www.bensdream.org
Stuart Siedman, stuart@bensdream.org or 617-899-4158

About The Children's Medical Research Foundation, Inc.
The Children's Medical Research Foundation, Inc. was formed in 1995, shortly after Kirby, the daughter of founders, Sue and Brad Wilson, was diagnosed with Sanfilippo Syndrome. To date, this non-profit foundation has awarded $3.7 million dollars to research at seven universities, with more than 95% of all receipts appropriated to funding research.

For more information: www.curekirby.org
Sue Wilson, curekirby@sbcglobal.net or 708-784-0631

About The Sanfilippo Children's Research Foundation
Formed in 1998, The Sanfilippo Children's Research Foundation, located in Canada is a volunteer organization which has raised and committed over $4 million dollars towards Sanfilippo research; funding 30 research projects in five different countries. Nineteen year old, Elisa Linton, daughter of founders, Elizabeth and Randall Linton suffers from Sanfilippo Syndrome.

For more information: www.alifeforelisa.org
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