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Abeona Therapeutics Raises Foundation-led \$3.6M to Develop Therapies for Children Suffering from Rare Sanfilippo Diseases

CLEVELAND, Dec. 1, 2014 /PRNewswire-USNewswire/ -- Biotechnology startup Abeona Therapeutics announced today it closed \$3.6 million to complete preclinical development of therapies for children with Sanfilippo syndrome (mucopolysaccharidosis type III, MPS III), a rare terminal genetic disorder that results in death in children before they reach their mid-teens. Investors include U.S.-based Cure Sanfilippo Foundation, Sanfilippo Research Foundation (SRF), Team Sanfilippo, the Abby Grace Foundation, and the National MPS Society. Abeona also generated strong support from the international Sanfilippo community, with investments from Spain-based Stop Sanfilippo and Sanfilippo B Foundation, Geneva, Switzerland-based Fondation Sanfilippo and Mexico-based Red Sanfilippo Foundation. Support for the program has also been provided by The Children's Medical Research Foundation, Inc. (CMRF) and Canadian led Sanfilippo Children's Research Foundation (SCRF).



"For over a decade, our Sanfilippo research has been supported by Ben's Dream – SRF, and Cure Kirby – CMRF, and jointly by A Life for Elisa – SCRF, Team Sanfilippo and LivLife Foundation since 2010. We are very thankful that our joint efforts have led to our planned clinical trials in 2015," said Drs. Douglas McCarty and Haiyan Fu, Abeona's scientific founders and investigators at Nationwide Children's Hospital."

Sanfilippo syndrome is a group of 4 deadly genetic diseases resulting from the body's inability to properly break down certain sugars. Symptoms often appear in the first year of life, and the disease causes progressive muscular and cognitive decline in children after the age of two. There is no cure and currently no approved treatments for Sanfilippo syndrome. As such, children afflicted with Sanfilippo syndrome experience progressive loss of speech, the ability to eat and walk, and rarely live past their second decade of life. Abeona

Therapeutics is focusing initially on gene therapies for Sanfilippo syndrome types A and B.

"This funding helps support our global development of gene therapies for treating patients with Sanfilippo syndrome. We have formed a very special, international, collaborative effort where Abeona has become the voice of over a dozen patient advocacy groups desiring accelerated access to potential therapies," explained Tim Miller, Ph.D, Abeona's President/CEO. "We are pleased to collaborate with Nationwide Children's Hospital in the development of these therapies as we approach clinical trials."

"We are excited to be working with Abeona and the impressive world-wide collaboration of Sanfilippo foundations they have organized. Their emphasis on developing minimally-invasive treatments is reassuring as a parent. The dedication and approachability of the team has built confidence in the program and allows foundations to feel connected to the pursuit of a cure," said Kathleen Buckley, President of the Team Sanfilippo Foundation.

Time is a critical factor for children with Sanfilippo syndrome: 70% of the children with the disease will not reach their 18th birthday. "We are poised to initiate clinical trials in early-to-mid 2015 for these much needed therapies," said Kevin Flanigan, MD, principal investigator, Center for Gene Therapy, and neurologist at Nationwide Children's Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. He is also principal investigator for the ongoing Sanfilippo Natural History Study and upcoming gene transfer trials at Nationwide Children's.

The collaborative efforts of Abeona, the supporting Sanfilippo foundations, and Nationwide Children's Hospital were recognized by receipt of the Global Genes "RARE Champion of Hope" Collaborations in Advocacy award in late 2013 and by recent support from the National MPS Society. "The mission of the National MPS Society is to support promising therapies for these devastating, rare diseases, and we are pleased to support Abeona's progress towards clinical trials, " said Barbara Wedehase, Executive Director.

Cleveland-based Abeona Therapeutics is a start-up company focused on developing gene therapy based treatments for patients with lysosomal storage diseases, with lead products focusing on MPS IIIA and IIIB. Abeona Therapeutics received Orphan Product Designations for MPS IIIA and IIIB in 2014 and clinical trials are anticipated to begin in early-to-mid 2015.

About Abeona Therapeutics

Abeona Therapeutics - named after the Roman Goddess who is the protector of children - was formed in early 2013 to help focus the search for a cure for Sanfilippo Syndrome and provide a unifying voice between patient advocate groups, researchers, clinicians and investors. Abeona Therapeutics is the result of collaborative efforts between Nationwide Children's Hospital and multiple international patient advocate groups for developing Sanfilippo therapies. The collaboration has helped focus parents and caregivers on a leading therapy with broad potential to provide long-term benefits to children with Sanfilippo Syndrome. Clinical trials for Sanfilippo types A and B are anticipated to begin in early-to-mid 2015.

About Nationwide Children's Hospital

Ranked 7th of only 10 children's hospitals on *U.S. News & World Report's* 2014-15 "America's Best Children's Hospitals Honor Roll" and among the Top 10 on *Parents* magazine's 2013 "Best Children's Hospitals" list, Nationwide Children's Hospital is one of the

nation's largest not-for-profit freestanding pediatric healthcare networks providing care for infants, children and adolescents as well as adult patients with congenital disease. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's faculty train the next generation of pediatricians, scientists and pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the Top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational and health services research at Nationwide Children's. The Research Institute encompasses three research facilities totaling 525,000 square feet dedicated to research. More information is available at NationwideChildrens.org/Research.

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