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Abeona Therapeutics Closes \$750,000 in Seed Round to Develop Therapies for Children Suffering from Rare Sanfilippo Diseases

Studies Show Therapy is Best Hope for Survival

CLEVELAND, Dec. 16, 2013 /PRNewswire-USNewswire/ -- Biotechnology startup Abeona Therapeutics announced today it closed \$750,000 in seed financing to complete preclinical development of therapies for children with Sanfilippo Syndrome, a rare terminal genetic disorder that kills afflicted children before they reach their mid-teens. With this funding, more than \$2 million has been raised to date for this initiative. Investors include U.S.-based The Children's Medical Research Foundation, Inc. and Team Sanfilippo as well as Madrid, Spain-based Stop Sanfilippo and Geneva, Switzerland-based Fondation Sanfilippo.

(Logo: <https://photos.prnewswire.com/prnh/20131009/DC94808LOGO>)

Sanfilippo Syndrome is a deadly genetic disease 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 Sanfilippo Syndrome Types A and B.

"The support from multiple international foundations with a mandate for identifying lead Sanfilippo therapies enables us to complete preclinical development and sets the stage to enter clinical trials in 2014" explained Tim Miller, Ph.D, Abeona's President/CEO. "These investments highlight the quality of research conducted by our collaborators at Nationwide Children's Hospital and allow us to focus on the development of therapies with the potential to significantly impact children with these deadly diseases."

"There are no FDA-approved treatments for Sanfilippo Syndrome, and we're very pleased that Abeona Therapeutics is collaborating with Nationwide Children's Hospital to accelerate the entry of these therapies into clinical trials," said Kevin Flanigan, MD, Professor of Pediatrics at Nationwide Children's hospital and principal investigator of a Sanfilippo Natural History study underway at the institution.

"Abeona Therapeutics is a leader in developing non-invasive gene therapies for the treatment of children with Sanfilippo Syndrome. We're excited to help pave the way to clinical trials for these breakthrough therapies," commented Cara O'Neill, MD, Medical

Director of Team Sanfilippo whose three-year-old daughter suffers from the disease.

Cleveland-based Abeona Therapeutics is a start-up company created around intellectual property licensed from Nationwide Children's Hospital to develop treatments for Sanfilippo Syndrome Types A and B. Preclinical studies demonstrate the therapy's significant benefits in treating the disease, and the company will file for orphan drug status prior to its two Phase I/II clinical trials which are anticipated to begin in mid 2014

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, including The Children's Medical Research Foundation, Inc. (USA), Team Sanfilippo (USA), Fondation Sanfilippo (Switzerland), Stop Sanfilippo (Spain), Ben's Dream: The Sanfilippo Research Foundation (USA), and the Sanfilippo Children's Research Foundation (Canada). 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.

About Nationwide Children's Hospital

Ranked in all 10 specialties on *U.S. News & World Report's* 2013-14 "America's Best Children's Hospitals" list 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 currently ranked sixth among free-standing children's hospitals for National Institutes of Health-funding, supporting basic, clinical, translational and health services research. The Research Institute encompasses three research facilities totaling 525,000 square feet dedicated to research on the Nationwide Children's campus in Columbus, Ohio. More information is available at NationwideChildrens.org/Research.

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