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FOR IMMEDIATE RELEASE

Abeona Therapeutics Receives U.S. Orphan Drug Designations for treatment of Sanfilippo Syndromes A and B

Cleveland, OH, May 12, 2014 – Abeona Therapeutics, a start-up company created around intellectual property licensed from Nationwide Children's Hospital to develop treatments for Sanfilippo Syndrome Types A and B, has been granted Orphan Drug Designations for its lead investigational therapies by the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development. Following the successful close of seed financing in late 2013, Abeona is raising funds to advance its gene therapy-based clinical programs for both Sanfilippo Syndrome type A and B. Phase I/II clinical trials for both diseases are anticipated to begin in 2014.

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 as well as the inability to eat or walk, and rarely live past their second decade of life.

"It is so exciting to see this groundbreaking science take another critical step forward towards bringing this treatment to children", said Cara O'Neill MD, whose SavingEliza.com campaign has gathered international attention in the past month for this disease and their patient advocate group, Cure Sanfilippo Foundation (USA).

"The FDA's approval of our request for orphan drug designation is an important regulatory milestone for Abeona that supports our strategy for treating these devastating diseases" explained Tim Miller, Ph.D, Abeona's President/CEO. "The benefits include seven years of market exclusivity from product launch in the United States, tax credits for clinical research costs and waiver of Prescription Drug User Fee Act (PDUFA) filing fees."

"We are encouraged by the FDA's continued recognition of the need for new treatments for rare and orphan diseases like Sanfilippo Syndrome and the designation of these gene therapies as potential therapeutic options" 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.

"There are no FDA-approved treatments for this deadly disease, and we're thrilled this designation may help accelerate the path towards approval for these much needed therapies," commented Kathleen Buckley, President of Team Sanfilippo, whose fourteen-year old son also suffers from the disease.

About Orphan Drug Designation

Orphan drug designation is granted by the FDA Office of Orphan Products Development (OOPD) to novel drugs and biologics, which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders – defined as a disease or condition that affects fewer than 200,000 individuals in the U.S. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular disease with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that indication.

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.