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Abeona Therapeutics and Nationwide Children's Hospital receive prestigious Global Genes "Champions of Hope" award for Patient Advocacy

CLEVELAND, Oct. 9, 2013 /PRNewswire-USNewswire/ -- New biotechnology startup Abeona Therapeutics and partner Nationwide Children's Hospital (Columbus, OH) announced today that they received the Global Gene's Champion of Hope award for Collaborations in Advocacy for their work in developing therapies for children with Sanfilippo Syndrome, a rare genetic disorder.

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

Sanfilippo Syndrome is a deadly genetic disease that results 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 2. There is no cure and there are currently no approved treatments for Sanfilippo Syndrome. As such, children afflicted with Sanfilippo Syndrome die in the second decade of life.

"This award recognizes nearly two decades of Sanfilippo research by Nationwide Children's Hospital researcher Dr. Haiyan Fu, and sets the stage for a breakthrough therapy that may make significant impact on the quality of life of children with this rare disease," states Tim Miller, Ph.D, Abeona's President/CEO.

"My lab has focused on developing gene therapy approaches for efficient gene delivery to the central nervous system for the treatment of mucopolysaccharidosis IIIB, or Sanfilippo Syndrome type B," explains Dr. Fu, who is an investigator in the Center for Gene Therapy at Nationwide Children's.

"In collaboration with Dr. Douglas McCarty in our center, we have successfully used the trans-blood-brain-barrier properties of the AAV9 viral vector to deliver a therapeutic gene to the central nervous system in a preclinical model. We have recently expanded our efforts and developed a similar treatment for Sanfilippo Syndrome type A with success. I believe that these approaches offer the best hope for effective treatment of these, and other, neuropathic diseases."

Cleveland-based Abeona is a spinoff of Nationwide Children's Hospital, which is licensing the intellectual property for treating Sanfilippo Syndromes A and B. Preclinical studies have demonstrated significant benefits in treating Sanfilippo Syndrome, and Abeona is raising funds in October 2013 for two Phase I/II clinical trials anticipated to begin in 2014. The company will also file for orphan drug status before clinical trials begin.

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 and provide a unifying voice between patient advocate groups, researchers, clinicians and investors. Abeona 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. Through this joint effort, new therapies are moving into clinical trials set to begin in 2014.

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 of 160 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.

SOURCE Abeona Therapeutics