NATIONWIDE CHILDREN'S
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Sept. 20, 2013

A Special Statement for our Supporters and Advocates of SMA Research

Following the submission of an Investigational New Drug application, the FDA has given its approval to physician-scientists at Nationwide Children's Hospital to begin a Phase I clinical trial of a systemic AAV9-delivered human SMN gene.

The clinical trial is expected to begin in early 2014 and will be limited to Type I SMA patients, ages birth to 9 months.

Previous research from Nationwide Children's Principal Investigator Brian Kaspar, PhD, demonstrated the AAV9 viral vector crossing the blood brain barrier. Based on this research and additional preclinical studies, the SMN gene will be delivered by injection into the bloodstream as part of this Phase I trial. Neurologist Jerry Mendell, MD, director, Center for Gene Therapy at Nationwide Children's, will lead the study.

Generous funding for this trial and the FDA studies required for the investigational drug application has been provided by The Sophia's Cure Foundation.

An additional program also using the AAV9-delivered SMN gene will examine a different route of delivery by injecting the virus into the cerebrospinal fluid. This program will also move toward clinical testing thanks to substantial support from the Families of SMA and the National Institute of Neurological Disease and Stroke (NINDS).

Dr. Kaspar notes, "We are extremely excited to be advancing two delivery routes to SMA patients and will continue to tirelessly move these programs in a rapid and safe manner. We thank everyone for their support in our efforts to develop treatments for this disease."