FOR IMMEDIATE RELEASE

Newly Approved Drug Offers Hope for Local Family

KENDALL PARK, New Jersey (March 14, 2017) – For the Fantel family and many like them, the U.S. Food and Drug Administration approval of Spinraza™ (Nusinersen) for the treatment of Spinal Muscular Atrophy (SMA) offers fresh hope for living with a devastating diagnosis.

Ray Fantel, age 8, was diagnosed with SMA Type 1 when he was 5 months old. SMA is an incurable, terminal disease and the most frequent genetic cause of death in infants. The average life expectancy of a child with SMA is 2 years. "Thinking back to that day still brings tears to my eyes," said Ray's mother Marcy Fantel of the diagnosis, "My husband Matt and I decided we would do everything we could to help Ray on this journey."

SMA is a degenerative neuromuscular disease; Ray cannot sit up without assistance and has never crawled or walked. SMA affects all the muscles of the body, but most concerning is its impact on muscles that help patients breathe and clear congestion. Ray has needed medical equipment to support his breathing since he was 6 months old. Last year, he had back surgery to install hardware along his spine to help him breathe. Because even a simple cold can be life-threatening to a child with SMA, Ray stays home during flu season (mid-October through March) to protect him from respiratory illness, causing him to miss school and other activities.

The FDA approval of Spinraza™ opens a new path on Ray's journey. The drug became the first approved treatment for SMA on December 23, 2016. Infants receiving Spinraza™ injections in clinical studies were less likely to need ventilator support and could meet motor milestones previously thought impossible for children with SMA.

Dr. Emanuel Lerner, Ray's pediatrician since birth, also cares for a young girl with SMA who was part of the clinical trial for Spinraza™. He reports that, due to the drug, she has maintained more function for her age than other patients with SMA. She was recently admitted to the hospital for an infection that would normally result in prolonged hospitalization due to her SMA. However, she was discharged much sooner than expected when it became clear that she was not deteriorating as she normally would have. "I hope as this drug becomes available to more children with SMA, the unrelenting progression can be slowed for many more," Dr Lerner said.

"Spinraza™ is not a miracle drug, but it brings hope," said Mrs. Fantel. The Fantel family especially hopes that the drug will help strengthen Ray's respiratory muscles and stabilize his breathing. Ray's own hopes are higher: he wants to stand and walk.

Now that Ray's primary insurance has approved the injections, he will start receiving treatment in April. This spring is a time of hope for Ray, the Fantel family, and the entire SMA community. "We do not know what is possible," said Mrs. Fantel, "and we will not let Ray be discouraged from his dreams."

Contact:
Marcy Fantel
marcyfantel@hotmail.com
(732) 821-1279
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