INTHENews

A New Paradigm for Neuromuscular Disease

Until several years ago, the neuromuscular disease category had few to no approved therapies. But today, decades of research and commitment to neuromuscular disease are catapulting us into a space with never-before-conceived treatments.

This paradigm shift for spinal muscular atrophy (SMA) started with the U.S. Food and Drug Administration (FDA) approval of Spinraza (nusinersen) in December 2016. MDA funded foundational work in SMA and invested in the early-stage development of Spinraza, the first FDA-approved disease-modifying drug for SMA. Spinraza has been shown to change the course of the disease for some patients, increasing survival, motor milestone achievement and motor function.

MDA's Strongly blog (**strongly.mda.org**) contains stories about some of the patients whose lives have been changed by Spinraza — including an MDA National Ambassador, Faith Fortenberry, who went from being unable to lift her arms to lifting a drinking glass, taking the top off a marker and brushing her teeth.

Now fast forward to May 2019, when only the second FDA-approved gene therapy in the history of the United States was approved to treat SMA, placing the field of neuromuscular disease at the forefront of genetic medicine.

The approval of Zolgensma (onasemnogene abeparvovac-xioi) for the treatment of pediatric patients younger than 2 with SMA is another true breakthrough for this disease, which is the number one genetic cause of death in infants. When SMA is detected in a newborn and Zolgensma is administered, the course of the disease can be changed drastically.

"There are few genetic disorders so dramatic in their rapid killing of infants, and from what I have seen, the effects of gene therapy in SMA type 1 is dramatic beyond belief," MDA Chairman of the Board R. Rodney Howell, M.D., said on the day Zolgensma was approved. "It is a wonderful day to celebrate."

The availability of Zolgensma represents a major step in advancing the advent of safe and effective gene therapies and could propel the development of additional gene therapy approaches to treat a range of rare neuromuscular diseases. It is truly a new era in neuromuscular medicine.

Read more about the approval of Zolgensma and other research news at **strongly.mda.org**.