Gene Therapy From Nationwide Children's Hospital Is First To Receive FDA Approval For Duchenne Muscular Dystrophy

Nationwide Children's Hospital

In children with Duchenne muscular dystrophy (DMD), a gene mutation causes progressive weakness and damage to muscles—including muscles responsible for heart function and breathing—that lead to death in early adulthood. Now children with DMD have a new treatment option, thanks to a gene therapy developed at Nationwide Children's Hospital that is the first DMD treatment to target the genetic cause of the disease in addition to the symptoms.

ELEVIDYS, a gene therapy that was invented at the Center for Gene Therapy at Nationwide Children's Hospital, was approved by the FDA in June 2023 to treat ambulatory children with DMD who are 4 and 5 years old. This achievement has involved multiple collaborators within Nationwide Children's and a major industry partner, Sarepta Therapeutics, Inc.

DMD is an X-linked condition—meaning the gene mutation occurs on the X chromosome— typically affecting boys. Left untreated, most boys require wheelchair assistance by the time they are teenagers and lose their lifelong battle with DMD before turning 40 due to impaired cardiac and pulmonary function.

ELEVIDYS was developed by Dr. Jerry Mendell, recently retired neurologist and principal investigator at Nationwide Children's and Louise Rodino-Klapac, PhD, formerly head of the gene therapy research laboratory at Nationwide Children's and currently the executive VP, CSO and head of R&D at Sarepta Therapeutics. The gene therapy technology specifically addresses the dystrophin protein, which strengthens muscle fibers and protects them from injury in healthy people and is impaired in children with DMD.

Using a shortened version of the dystrophin gene and an adeno-associated virus vector, Mendell and Rodino-Klapac developed a way to deliver the building blocks of that key protein to the muscle cells of a person with DMD and translate those components into a functional version of the dystrophin protein. Studies have shown increased levels of healthy dystrophin in children with DMD 12 weeks after an intravenous injection of ELEVIDYS.

Studies also suggest the treatment helps children with DMD maintain their ability to walk, climb stairs and rise from sitting to standing. Time to rise from sitting to standing is a particularly important measure, because needing longer than 5 seconds to complete that task is predictive of losing the ability to walk. In a recent study, children treated with ELEVIDYS were 91% less likely to have a time to rise of 5 seconds or longer after one year than children who received a placebo injection.

The translational capabilities of Nationwide Children's, such as the Office of Research Regulatory Affairs which manages filings with the FDA for Investigational New Drug Applications, and the Clinical Research Services that manage clinical trials at Nationwide Children's, helped the institution attract a commercial partner. The Office of Technology Commercialization initially entered into an option agreement with Sarepta Therapeutics in late 2016, then licensed the technology in 2018 following promising positive Phase I clinical trial results. This licensing deal helped to move the therapy into clinical trials and further development quickly and efficiently.

Sarepta Therapeutics filed a Biologics License Application with the FDA in the fall of 2022. On June 22, 2023, the FDA announced accelerated approval for the therapy. To date, Sarepta Therapeutics has treated more than 140 individuals in the clinical development program, and eligible patients are now being treated in a commercial setting.

Learn more at FDA Approved Gene Therapy - Duchenne Muscular Dystrophy.

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