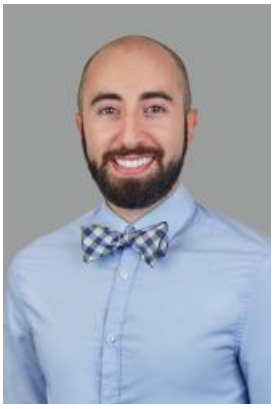
Nemours

Nemours Children's Hospital, Florida Now Offering Groundbreaking New Gene Therapy for Rare Muscular Disease

Nemours Children's treats first patient in Florida with new, FDA approved gene therapy ELEVIDYS.



Producer & Editor Note: Patient, Doctor Interviews, Broll and Photos Available By Request or Download Here

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ORLANDO, Fla. (Sept 25, 2023) – Nemours Children's Hospital, Florida has been named an official treatment site for Sarepta Therapeutics' gene therapy for the treatment of Duchenne Muscular Dystrophy (DMD). In addition, coinciding with "National Muscular Dystrophy Awareness Month," on September 13, Dr. Omer Abdul Hamid, a pediatric neurologist at Nemours Children's Hospital, Florida treated the very **first** patient in the entire state of Florida. Nemours Children's is also an approved and certified pediatric muscular dystrophy program by the Muscular Dystrophy Association.

DMD is a rare type of muscular dystrophy that is caused by a genetic variant in the dystrophin gene that is responsible for keeping muscle cells intact. Duchenne primarily affects males, occurring in approximately 1 in 3,500 to 5,000 male births worldwide; 9,000 to 12,000 people in the U.S. and 250,000 to 300,000 people globally. Until now, patients have only had treatments to manage their symptoms.

Symptom onset occurs in childhood, beginning with muscle weakness, and as the disease progresses, it can interfere with the ability to walk and eventually breathe resulting in a lower life expectancy. The disease primarily affects boys, but in rare cases, can affect girls, according to the Muscular Dystrophy Association.

The new, one-time treatment, ELEVIDYS, was recently granted approval by the U.S. Food and Drug Administration (FDA) through the Accelerated Pathway Program and is the first gene therapy available for DMD. Nemours' experts believe this drug may help to address the genetic causes responsible for DMD and hope it will lead to halting disease progression, as well as managing the symptoms it causes.

"There is no cure for DMD and we have very few treatments that address the underlying cause," said Omer Abdul Hamid, MD, Pediatric Neurologist, Nemours Children's Hospital, Florida. "This innovative, new gene therapy will treat the cause of the disease and is another tool for us as providers to help treat these patients; we are happy to be able to offer it to our patients who are candidates. It is also giving a lot of hope to the DMD community including doctors, researchers, patients, families and advocates that have all been searching for answers for a long time."

The gene therapy is for the treatment of ambulatory pediatric Duchenne patients aged 4 through 5 years with a confirmed gene mutation. Nemours Children's is an approved and certified pediatric muscular dystrophy program by the Muscular Dystrophy Association.

About Nemours Children's Health
Nemours Children's Health is one of the nation's largest multistate pediatric health systems, including two free-standing children's hospitals and a network of more than 70 primary and specialty care practices. Nemours Children's seeks to transform the health of children by adopting a holistic health model that utilizes innovative, safe, and high-quality care, while also caring for the health of the whole child beyond medicine. Nemours Children's also powers the world's most-visited website for information on the health of children and teens, KidsHealth.org.

The Nemours Foundation, established through the legacy and philanthropy of Alfred I. duPont, provides pediatric clinical care, research, education, advocacy, and prevention programs to the children, families and communities it serves.

About ELEVIDYS

ELEVIDYS is a trademark of Sarepta Therapeutics, Inc. registered in the U.S. Patent and Trademark Office and may be registered in various other jurisdictions.

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https://nemours.mediaroom.com/FirstPatientinFloridaReceivesGroundbreakingTherapy