

Gene Therapy Investigated by Nemours Children's Health Researchers Approved by FDA as The First and Only Treatment for Inherited Hearing Loss

Participants who received the gene therapy in the CHORD clinical trial gained improved hearing, setting stage for accelerated approval

JACKSONVILLE and ORLANDO, Florida (September 9, 2026) — Research conducted by Nemours Children's Health played a pivotal role in the recent U.S. Food and Drug Administration (FDA) approval of Otarmeni™ (lunsotogene parvec-chwa), a gene therapy for a rare form of genetic hearing loss.

Otarmeni is an adeno-associated virus vector-based gene therapy delivered via infusion into the inner ear. The FDA approved Otarmeni based on the results of the CHORD clinical trial, which showed that 80% of participants achieved or surpassed a pre-determined goal of improved hearing. With longer follow-up, 42% achieved hearing levels that allowed them to detect whispers. The most common adverse reactions (≥5%) in the safety population of CHORD associated with Otarmeni include otitis media, vomiting, nausea, dizziness, procedural pain, gait disturbance, and nystagmus.

Nemours Children's Health locations in Jacksonville and Orlando were two of only nine sites in the United States to participate in the CHORD trial. Researchers administered the investigational therapy to two patients born with a type of severe-to-profound hearing loss associated with biallelic variants in the OTOF gene. These variants affect the otoferlin protein, which is essential for transmitting auditory signals between the inner ear and the brain. This type of hearing loss affects about 50 newborns per year in the United States.

In July 2024, Evie Landry, MD, Pediatric Neuro-Otologist, Nemours Children's Health, Jacksonville, administered the gene therapy to a 4-year-old boy at Wolfson Children's Hospital, one of 12 children studied and reported in [The New England Journal of Medicine](#). Within a few weeks, his mother reported that household noises that he once couldn't hear were now easily recognized. At 24 weeks, the patient showed significant improvement, and subsequent tests have indicated that his hearing has improved to levels where he can hear whispers.

In March 2026, Cedric Pritchett, MD, MPH, Pediatric Otologist and Chief of Pediatric Otolaryngology at Nemours Children's Hospital, Florida, administered the gene therapy to an 18-month-old girl. The patient tolerated the procedure well and has begun responding to sounds in her environment. Patients typically show measurable improvement at about 24 weeks after the procedure, the Nemours surgeon-scientists noted.

Dr. Landry said the development and approval of Otarmeni signals a new era for the use of gene therapy to treat severe-to-profound hearing loss.

"Otoferlin is just one of about 300 genes that are known to be associated with hearing loss," Dr. Landry explained. "We hope that this trial provides the scientific foundation for a new paradigm in the molecular diagnosis and treatment of hearing loss. If successful for other genes, this will pave the way for a myriad of genetic conditions, potentially changing the lives of children with hearing loss around the world."

"The approval of Otarmeni is a generational advance in the treatment of children with this type of deafness," Dr. Pritchett said. "We believe that we have achieved something special by participating in the CHORD trial and bringing this experimental therapy to our young patients."

"Clinical trials are the driver of scientific progress, ensuring safety and efficacy of new therapies for patients young and old," noted Matthew M. Davis, MD, MAPP, Executive Vice-President, Enterprise Physician-in-Chief and Chief Scientific Officer, Nemours Children's Health. "The clinical and academic preeminence of our teams at Nemours Children's Health allowed us to play a role in this groundbreaking trial and enable hearing for these young patients."

Otarmeni was developed by Regeneron Pharmaceuticals, Inc. The drug was granted accelerated approval by the FDA and will continue to be assessed in the confirmatory portion of the clinical trial.

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About Nemours Children's Health

Nemours Children's Health is one of the nation's largest multistate pediatric health systems, which includes two free-standing children's hospitals and a network of 80 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



*Evie Landry, MD, Pediatric Neuro-Otologist,
Nemours Children's Health, Jacksonville*

addressing children's needs well beyond medicine. In producing the highly acclaimed, award-winning pediatric medicine podcast Well Beyond Medicine, Nemours underscores that commitment by featuring the people, programs and partnerships addressing Whole Child Health. Nemours Children's also powers the world's most-visited website for information on the health of children and teens, [Nemours KidsHealth.org](https://nemourskidshealth.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. For more information, visit [Nemours.org](https://nemours.org).

About Wolfson Children's Hospital of Jacksonville

Nonprofit [Wolfson Children's Hospital](https://wolfsonchildrenshospital.com) in Jacksonville, Florida, is a part of [Baptist Health](https://baptisthealth.com), Northeast Florida's most comprehensive health system serving every stage of life which was named by Newsweek as one of "America's Greatest Workplaces in Health Care." Wolfson Children's is the only children's hospital in Northeast Florida and serves as the pediatric referral center for North Florida, South Georgia and beyond. Staffed 24/7 by pediatric nurses and other healthcare professionals specially trained to work with children, the 281-bed, patient- and family-centered hospital features the latest pediatric medical technology in a welcoming, child-friendly environment. At Wolfson Children's, nationally recognized pediatric specialists representing nearly every medical and surgical specialty work with pediatricians to provide care for children of all ages with congenital heart conditions, cancer, neurological disorders, diabetes and endocrinology disorders, orthopedic conditions, behavioral health conditions, traumatic injuries, and more. Wolfson Children's collaborates with pediatric organizations, including [Nemours Children's Health, Jacksonville](https://nemourschildrenshealth.com), [Emergency Resources Group](https://erjacksonville.com), [Mayo Clinic Florida](https://mayoclinic.org) and the [University of Florida College of Medicine — Jacksonville](https://ufl.edu/college-of-medicine-jacksonville). A Magnet™-designated hospital since 2010, Wolfson Children's Hospital has been named among the U.S. News & World Report 50 Best Children's Hospitals year after year, and in 2025, was one of only 15 children's hospitals in the country selected as a Leapfrog Top Children's Hospital, a recognition of patient quality and safety. For additional information, please visit wolfsonchildrens.com. Wolfson Children's Hospital relies on the generosity of members of our community. To support Wolfson Children's Hospital, please visit wolfsonchildrens.com/give.

Media Contact:

Leah Goodwyne

Leah.goodwyne@nemours.org

904.676.2229

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