

Pediatric Neurology: Neuromuscular Disorders

At Nationwide Children's, we're not only actively managing the treatments and outcomes of patients diagnosed with neuromuscular disorders, we're also one of only a few institutions in the country actively working on a cure.

Our Neuromuscular Disorders Clinic uses a multidisciplinary team to diagnose and treat disorders affecting muscles in children, such as muscular dystrophy, myositis and congenital myopathy, neuropathies and myasthenic disorders, among others. But our program is more than a focused clinic. Both Nationwide Children's Hospital and The Research Institute at Nationwide Children's are home to leading clinicians and scientists who have dedicated their lives to treating and preventing pediatric neuromuscular disorders. Our full range of clinical services includes:

- Electrodiagnostic testing such as EMG testing
- Medical and rehabilitation supplies
- Muscle biopsy
- Nerve biopsy
- Neuromuscular laboratory service
- Physical therapy
- Respiratory therapy

Renowned Leadership

Jerry Mendell, MD, a distinguished muscular dystrophy researcher, leads the Neuromuscular Disorders clinical program, and the Center for Gene Therapy at The Research Institute. Dr. Mendell was among the first to test muscle cell transplantation for Duchenne muscular dystrophy in the early 1990s and the first person to study viral mediated gene therapy for muscular dystrophy in humans. Dr. Mendell has made fundamental contributions in clinical research and in the molecular genetics of neuromuscular disease. In 2006, Dr. Mendell and his team began the first-ever gene therapy trials for boys with Duchenne muscular dystrophy.

Within the past five years, Dr. Mendell and other researchers in the Center for Gene Therapy and the Center for Vaccine and Immunity have been awarded multi-million-dollar grants from the NIH and published key articles in high-impact journals such as *The New England Journal of Medicine*, *Cell*, *Proceedings of the National Academy of Sciences* and *Nature*.

[more...]



The Wellstone Center at Nationwide Children's

The Muscular Dystrophy Association named Nationwide Children's to its Clinical Research Network in 2008. In 2010, Nationwide Children's received a \$7 million NIH grant along with designation as a Paul D. Wellstone Muscular Dystrophy Cooperative Research Center. The Wellstone Center explores and refines therapeutic strategies that have shown promise in animal models of muscular dystrophy. Studies include further research in the laboratory as well as clinical trials to assess efficacy in patients. These efforts include replacing defective genes responsible for muscular dystrophy with healthy ones. Other trials use substitute or surrogate genes to restore muscle function. A third approach attempts to repair gene function using specially designed medications. All of the studies aim to slow the progression of muscular dystrophy.

Current Clinical Studies

Investigators with the Center for Gene Therapy at Nationwide Children's are currently conducting numerous clinical research studies in neuromuscular disorders, including muscular dystrophy. Current studies include:

- Implications of Dystrophin-Specific T Cells for DMD Gene Correction
- Clinical Outcomes Validation in Non-Ambulatory Patients with DMD
- Clinical Outcomes for Infants/Young Children Under Age 3 with DMD
- Clinical Outcomes Studies in BMD and Sporadic Inclusion Body Myositis
- Safety and Tolerability of GSK2402968 for Exon Skipping (Exon 51) in Non-Ambulant Subjects with DMD
- MDA Clinical Network: Natural History Study of Heart Disease in DMD and BMD
- United Dystrophinopathy Project: Genotype/Phenotype Correlation and Natural History in the Dystrophinopathies

Learn more about our current clinical studies and other research and clinical information at NationwideChildrens.org/Neurosciences.

Scheduling, Referrals and Consultations

The Neuromuscular Disorders Clinic accepts referrals from primary care physicians and pediatric specialists from across the United States and internationally.

Online: NationwideChildrens.org/Neurosciences

Fax: (614) 722-4000

Phone: (614) 722-6200 or 1-877-722-6220

Physician Direct Connect Line for urgent physician consultations:

(614) 355-0221 or 1-877-355-0221



Ranked by *U.S. News & World Report* among the best children's hospitals in the country in pediatric neurology and neurosurgery.

