

Advanced Neuromuscular Research and Care for Your Patients



When your child needs a hospital, everything matters.sm

Leading Specialists, Coordinated Care

Groundbreaking. Multidisciplinary. Comprehensive. These words don't just describe our world-class Neuromuscular Disorders program. They are fundamental to our work and the commitment we make to the families who come to us from across the country.

At Nationwide Children's Hospital, our Neuromuscular Disorders program not only offers leading specialists from key disciplines, we also coordinate patient care across each specialty. Patients benefit from coordinated care from specialists, all with expertise in pediatric neuromuscular disorders.

Coordinated care with pediatric specialists:

- Cardiology
- Developmental medicine
- Endocrinology
- Gastroenterology
- Hospice and palliative care
- State-of-the-art diagnostic services:
- Electromyogram (EMG) and nerve conduction testing

- Nephrology
- Neurology
- Nutrition
- Orthopedics
- Physical and occupational therapy
- Physical medicine and rehabilitation
- Pulmonary medicine
- Social services
- Genetic counseling and genetic testing Nerve biopsy
- Muscle biopsy
 Neuromuscular laboratory service



Neuromuscular Disorders Clinic

Adult and pediatric patients from across the country come to Nationwide Children's for the specialized care in our Neuromuscular Disorders Clinic. Our team of providers works closely with patient and family to diagnose, treat and manage quality of life. This clinic focuses on an array of neuromuscular disorders, including:

- Charcot-Marie-Tooth and other hereditary neuropathies
- Chronic inflammatory demyelinating polyneuropathy
- Guillain-Barre syndrome

- Myasthenia gravis
- Lambert-Eaton syndrome
- Congenital myasthenic syndromes
- Polymyositis and dermatomyositis
- Inclusion body myositis

- Congenital myopathies
- Friedreich's ataxia
- Mitochondrial and metabolic myopathies
- Periodic paralysis

Muscular Dystrophy Clinic

The Muscular Dystrophy Clinic at Nationwide Children's is nationally recognized for leading research and clinical care for patients with any of the muscular dystrophy types. Supported in part by the Muscular Dystrophy Association, we provide the highest level of comprehensive care to patients from across the country.

Our multidisciplinary team is the foundation of our clinic. The combined expertise of specialists in neuromuscular disorders, cardiology, pulmonary medicine, and other disciplines coordinates patient care in a single clinic location. Specialized therapists in the clinic manage activities of daily living, and assist patients with techniques to adapt to progressive symptoms. While some testing and referrals to additional specialists may require additional scheduling, we provide all essential services in the Muscular Dystrophy Clinic, limiting the need for multiple appointments. Conditions treated include:

- Duchenne muscular dystrophy
- Becker muscular dystrophy
- Congenital muscular dystrophy
- Facioscapulohumeral muscular dystrophy
- Limb-girdle muscular dystrophy
- Myotonic muscular dystrophy
- Emery-Dreifuss muscular dystrophy
- Distal muscular dystrophy
- Oculopharyngeal muscular dystrophy

Spinal Muscular Atrophy Clinic

Our Spinal Muscular Atrophy (SMA) Clinic is a joint program between Nationwide Children's Hospital and The Ohio State University Medical Center, providing comprehensive, multidisciplinary care for patients from across the United States.

One of the key components of the SMA Clinic is patient advocacy. We assist patients and families in coping with the chronic effects of SMA and help them identify available services. The instruction and educational materials we provide are geared specifically to the unique needs of SMA patients and families.

As a leading national program, we provide clinical teaching for medical students, residents, graduate students and postdoctoral fellows, and we also provide educational sessions for pediatricians and specialists in our primary service area and nationally.

The SMA Clinic is fully affiliated with the International Coordinating Committee (ICC) for SMA that links the multiple SMA lay and professional organizations into a unified and cohesive unit to pool information and projects related to SMA.

Leading Neuromuscular Research

Dr. Jerry Mendell, a distinguished muscular dystrophy researcher, leads the Neuromuscular Disorders clinical program and is the Director of 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 1990's and the first to study viral mediated gene therapy for muscular dystrophy in humans. He has made fundamental contributions in clinical research and in the molecular genetics of neuromuscular disease. In 2006, he and his team began the first-ever gene therapy trials for boys with Duchenne muscular dystrophy.

The Muscular Dystrophy Association named Nationwide Children's to its Clinical Research Network in 2008. In 2010, Nationwide Children's received a \$7 million National Institutes of Health 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, and ongoing studies include further research in the laboratory as well as clinical trials to assess efficacy in patients. The neuromuscular translational research team includes Dr. Kevin

Flanigan and Dr. Zarife Sahenk, investigators at the Center for Gene Therapy.

The SMA Clinic is directed by Dr. John Kissel of the Division of Neuromuscular Medicine at the Ohio State University College of Medicine. In collaboration with the Center for Gene Therapy in The Research Institute at Nationwide Children's and OSU, the clinic is actively involved in clinical trials for children and adults with SMA. Our Clinical Research Unit is the only center in the world that has carried out clinical trials in all three major forms of the disease. We have a number of ongoing trials, and many more in planning stages.

We participate in the Families of SMA-sponsored "Project Cure SMA," an international consortium of medical centers performing clinical trials in SMA. We are also part of a select group of 25 national clinical sites that make up Network of Excellence in Neuroscience Clinical Trials (NeuroNEXT). Created by the NIH, NeuroNEXT fosters cooperation among leading clinical sites, allowing them to efficiently design and implement clinical trials encompassing the entire spectrum of neurologic diseases.

Neuromuscular Clinical Trials

The following is a list of neuromuscular clinical trials, active as of July 1, 2013. For the most recent listings, and to learn about enrolling in a study, visit **NationwideChildrens.org/Neuromuscular-Clinical-Trials**. You may also call one of our study coordinators at (614) 722-2203.

- Follistatin (FS344) Gene Transfer to Patients with Becker Muscular Dystrophy and Sporadic Inclusion Body Myositis
- The United Dystrophinopathy Project: Genotype/Phenotype Correlation and Natural History in the Dystrophinopathies
- A Prospective Natural History Study of Progression of Subjects With Duchenne Muscular Dystrophy
- Phase 3 Study of Ataluren in Patients With Nonsense Mutation Duchenne Muscular Dystrophy
- Safety, Tolerability, and Pharmacokinetics of Single and Multiple Doses of HT-100 in Duchenne Muscular Dystrophy
- Clinical Outcome Study for Dysferlinopathy
- Early Treatment of Cardiomyopathy in Duchenne Muscular Dystrophy
- Spinal Muscular Atrophy (SMA) Biomarkers Study in the Immediate Postnatal Period of Development
- Finding the Optimum Regimen for Duchenne Muscular Dystrophy (FOR-DMD)

Ongoing clinical trials can also be found at www.ClinicalTrials.gov.



Referrals and Consultations

Online: NationwideChildrens.org

Fax: (614) 722-4000

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

Physician Direct Connect Line for 24-hour urgent physician consultations:

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





Ranked among the best children's hospitals in the U.S. in all 10 specialties.

Neurosciences Center 700 Children's Drive Columbus, Ohio 43205 NationwideChildrens.org/Neurology



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