# PROGRAM PROGRAM

The Neuromuscular Program at Children's Hospital of Philadelphia is led by experts in the diagnosis, treatment and support of children living with neuromuscular diseases (NMDs) and their families. Each year, we care for more than 500 patients from around the world who have a wide range of NMDs. Our program is recognized as a leader in the field by Cure SMA, the Muscular Dystrophy Association, the Charcot-Marie-Tooth Association Centers of Excellence and the National Institutes of Health's Inherited Neuropathies Consortium.

Our Neuromuscular Program has the expertise to provide state-of-the-art diagnostics and a high level of patient-centric clinical care. The program is at the forefront of research and development of new therapeutic strategies for pediatric NMDs, including the investigation of disease-causing genes and participation in clinical trials.

In addition to the program's twice weekly general clinics, multiple subspecialty clinics are offered to address disease-specific needs and to implement individualized care plans for the following diagnoses.

Brachial Plexus Injury: This program team includes experts in pediatric neurology, occupational therapy, orthopaedic surgery, neurosurgery, plastic surgery and radiology. Personalized treatment strategies are offered for infants and children with brachial plexus and peripheral nerve injuries that help them regain use of affected limbs.

Charcot-Marie-Tooth (CMT) Disease: This clinic offers individuals with CMT and other inherited neuropathies diagnosis and evaluation by a specialized team, including neurologists, physical therapists, occupational therapists, genetic counselors, social workers, orthopaedics and surgery. This clinic is part of the Charcot-Marie-Tooth Association Centers of Excellence and the National Institutes of Health's Inherited Neuropathies Consortium. It offers patients the opportunity to participate in a natural history study.

MUSCLE-MD: This clinic is for individuals with myopathies and includes evaluation and treatment by a neurologist, cardiologist and pulmonologist. Individuals seen in this clinic often alternate visits between this clinic and the general NMD clinic.

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Myasthenia Gravis (MG): Individuals with MG are evaluated and treated by neurologists with expertise in this area, as well as specialists in neuro-ophthalmology and general surgery, who perform minimally invasive thymectomies when indicated.

Spinal Muscular Atrophy (SMA): CHOP is a leader in care for individuals with SMA, and is one of the largest dosing centers for nusinersen (Spinraza®) and onasemnogene abeparvovec (gene therapy). This multidisciplinary program includes additional specialists in orthopaedics, endocrinology, pulmonary, and physical and occupational therapy, with a focus on maximizing function through supportive care and addressing new challenges in the course of a dramatically altered disease.

Undiagnosed NMDs: Neurologists, including Carsten G. Bönnemann, MD, an expert from the National Institutes of Health (NIH), evaluate children who present with complex symptoms/phenotypes suggestive of NMDs, but who remain undiagnosed despite years of extensive work-ups. Families have the opportunity to participate in the NIH's Undiagnosed Diseases Program and have access to new genomic technologies to facilitate diagnosis and disease discovery.

# **PARTNER WITH US**

To refer a patient or request a second opinion: 267-426-6298 CHOPUSA@email.chop.edu

### **LEARN MORE**

chop.edu/neuromuscular-program

### **CONDITIONS TREATED**

We treat the following conditions:

- · muscular dystrophy
- congenital myopathies
- inflammatory myopathies
- · metabolic myopathies
- myotonic syndromes and muscle channel opathies, such as periodic paralysis
- spinal muscular atrophy and other motor neuron disorders
- Charcot-Marie-Tooth disease and other peripheral and acquired neuropathies, including Guillain-Barré syndrome and chronic inflammatory demyelinating polyneuropathy
- myasthenia gravis and congenital myasthenic syndromes
- · brachial plexus injuries

## OUR TEAM. YOUR PARTNERS.

Individuals with NMDs are best served by interdisciplinary care that addresses all of their physical, social and emotional needs. Every child's plan of care is coordinated to include time spent with team members from specialty areas, including: neurology, cardiology, endocrinology, genetics, orthopaedics, physical and occupational therapy, pulmonology, surgery, child life, nutrition, nursing, and social work. Our team is compassionate and strives to provide the most current, comprehensive, patient-centric and specialized care possible.

