A New Era in Neuromuscular Disease

The Muscular Dystrophy Association (MDA) is committed to transforming the lives of people with muscular dystrophy, ALS, and related neuromuscular diseases through innovations in science and innovations in care.
Not long ago, supportive care and mobility aids were all medical science could offer. But the treatment and care landscapes have begun to change dramatically for many people living with NMDs. Recent breakthroughs in genetic medicine, along with other discoveries, are accelerating the development of new therapies, including some that not just address symptoms but also — for the first time — directly modify the underlying disorders.

Support enables MDA to fund leading research teams working toward breakthrough therapies that can have a life-changing impact on patients.

MDA has invested more than $1 billion of donor contributions to the search for cures, including 224 research projects in 2018 alone.

MDA-funded breakthroughs include drugs for Duchenne muscular dystrophy (DMD), periodic paralysis, Pompe disease and spinal muscular atrophy (SMA).

And it’s not just about new therapies. Thanks to research and advocacy efforts of MDA and its partners, new diagnostics and screening policies enable earlier detection and treatment. The goal: prolonging muscle function and lives.

At an explosive pace, advances in care, communication, and collaboration are now upending long-held assumptions about people with NMDs; never have so many possibilities existed for a community that once faced progressive disability and premature death. While most NMDs still remain extremely challenging to live with and treat, today more people than ever before can look forward to lives with potential unimaginable just a few years ago.

Yet a tremendous amount of work remains to be done.

More than $1 billion invested to date in accelerating the development of therapies and cures.

Our Mission: Transformation Through Innovation

As the largest and longest-established organization for people with NMDs, MDA works tirelessly on the NMD community’s behalf. We’ve done so for nearly seven decades. Now, with growing scientific knowledge and the expansion of improved treatment options come new ways for us to transform the lives of people with muscular dystrophy, ALS, and related neuromuscular disease through innovations in science and innovations in care.
Innovations in Science

The transformed MDA has deepened its roots in the science of neuromuscular health and disease. We have re-dedicated our commitment to the support of clinical research initiatives.

Research
MDA is the largest source of funding for neuromuscular disease (NMD) research outside the federal government.

Therapies
Research supported by MDA is directly linked to life-changing therapies across multiple neuromuscular diseases. Importantly, MDA’s network model works across the full spectrum of NMDs to cross-pollinate ideas and cross-link disease-specific developments to potential applications that help the entire NMD community.

As knowledge about NMDs has vastly expanded, so has the complexity of diagnosis and treatment. Science has uncovered scores of specific genetic dysfunctions underlying NMD subtypes.

We are therefore bringing new resources and skills, along with a pan-NMD scope, to rapidly transfer this exponentially growing body of knowledge from laboratory to clinic, where it can change lives as never before.

Parts of MDA’s new role include:

- Working with investigators on new, improved clinical trial designs and real-world evidence
- Helping scientific partners view, track, and interpret unbiased and up-to-date data to make fully informed decisions about future innovations
- Convening clinical stakeholders to change standards of care as new approaches become available

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ALS = amyotrophic lateral sclerosis; DMD/BMD = Duchenne muscular dystrophy/Becker muscular dystrophy; SMA = spinal muscular atrophy; LGMD = limb-girdle muscular dystrophies; FSHD = facioscapulohumeral muscular dystrophy; DM = myotonic dystrophy; CMD = congenital muscular dystrophy; CMT = Charcot-Marie-Tooth disease.
Innovations in Science

Big data is a key part of MDA’s evolution.

MDA launched the MOVR (neuromuscular observational research) Data Hub as a transformative platform, combining MDA’s Care Center Network with a state-of-the-art information-management system.

As the largest centralized data hub for multiple neuromuscular diseases, MOVR aggregates clinical, genetic, and patient-reported data across broad communities of healthcare providers, researchers, and industry partners that will lead to rapid developments in patient care, treatments, and cures.

MOVR will be an unparalleled one-stop resource, an asset to researchers probing for answers that could unlock new NMD science, and a powerful force for improved coordination of care for individuals.

An Unprecedented Era in Therapy Development for Neuromuscular Diseases

Neuromuscular diseases (NMDs), an exceptionally diverse group of rare disorders, have recently become a focus of gene and antisense oligonucleotide therapies. The early results have been so astounding that it is now opportune to rethink research and clinical programs in NMDs. We urgently need greater numbers of patients to participate in clinical trials and better ways to track their clinical and genetic data to maximize the benefits of these therapies, ensure broad access, and support the fast and economic development of new drugs.

Indeed, that several experimental gene therapies are in advanced clinical trials in our diseases is the result of decades of effort from a large community of partners—including families, donors, researchers, and clinicians. MDA will continue to work together with these partners to keep the momentum going toward more treatments and cures.

Genetic Medicine

MDA has contributed greatly to the field of muscle disease and toward landmark research advances, including the identification of the first human disease-causing gene, as well as the first human trial of gene transfer for a muscle disease.

For several decades, MDA-supported researchers have discovered the gene-causing mutations for many other neuromuscular disorders, developed and refined gene delivery tools and methods, and established protocols for safe and effective gene therapy clinical trials. This was achieved by robust and rigorously-reviewed MDA grant programs aimed at driving the field of gene therapy forward, as well as MDA taking a leadership role in continually raising awareness and convening key experts to address challenges.

Industry Recognition

In 2019, MDA was granted the Sonia Skarlatos Public Service Award from the American Society for Gene & Cell Therapy (ASGCT).*

* This award recognizes a person or group that has consistently fostered and enhanced the field of gene and cell therapy through governmental agencies, public policy groups, public education, or non-governmental charitable organizations.
Innovations in Care

MDA programs and services enrich the lives of people with neuromuscular disease.

The cornerstone of MDA’s Innovations in Care programming is MDA’s National Care Center Network, a system of multidisciplinary medical clinics across the U.S. at more than 150 top medical institutions. These centers conduct 70K+ medical visits annually for individuals living with muscular dystrophy, ALS, and related neuromuscular diseases. Each of our MDA Care Centers offers individuals and families best-in-class, comprehensive care from an integrated multidisciplinary team of healthcare specialists conveniently sited at one location. In a single day, patients can see multiple healthcare providers who work together to ensure coordinated individual care for every patient to best fit their specific needs. Highly trained MDA Care Specialists serve as an important part of the care team, helping families navigate the health system, answering questions, distributing MDA educational materials, coordinating MDA services, and assisting with community resource referrals.

MDA Care Centers also serve as regional and local hubs of NMD research activity for clinical trials and natural history studies. As major gateways to the MOVR Data Hub—both as expert accumulators of NMD knowledge and as sites where this knowledge can be tapped firsthand—the centers perform a vital function in advancing care.
Innovations in Care

MDA has taken a leadership position in promoting newborn screening.

Now that we understand better the crucial role genetics plays in NMDs, access to early screening, diagnosis, and treatment is vitally needed. For example, we know that spinal muscular atrophy (SMA) is the leading genetic cause of death in infants in the United States, and that early identification and intervention are key to treating the disorder.

Tactics to support newborn screening include peer-reviewed journal articles, convening coalition partners to promote federal engagement around congressional and regulatory actions, engagement with state policymakers to encourage implementation, the establishment of an advocacy grant program, and partnering with researchers to help them complete studies of new testing technology.

The International Association of Fire Fighters and MDA are also collaborating on a campaign to encourage states to expand their newborn screening programs by adding tests for specific neuromuscular disorders so that all newborns with these conditions can have the best possible chance at receiving the care and support services they need as early as possible.

Once babies with these conditions are identified via state newborn screening programs, MDA Care Centers at more than 150 top institutions across the U.S. can play a key role in confirmatory diagnoses, treatment, and long-term follow up and care.

In many cases, the follow-up care may be lifelong and, in some situations, the clinical symptoms may not manifest until later in life.

We are also committed to understanding of the natural history of neuromuscular disease. When babies are diagnosed early in life, it allows the opportunity to learn more about how the disorder manifests and to obtain insights into how early intervention affects the disease course.
Innovations in Care

MDA is changing with a clear mission, an expanded vision, and a renewed focus to meet the needs of a community undergoing a remarkable transition — and whose prospects for transformation have never been better.

**Camps**

Throughout our evolution, many of MDA’s most admired efforts and activities have stayed constant. Our MDA Summer Camps remain places where, for a week, kids and teenagers with NMD can gain independence and have fun as they learn vital life skills, like building confidence and learning self-advocacy. Each camp is staffed with dedicated health professionals and trained camp volunteers who meet the medical and physical needs of each camper — all at no cost to families.

**Collaboration**

Key events such as MDA Muscle Walk, MDA Team Momentum, and the MDA Shamrocks Program continue to unite our community and bring awareness to our mission. As we expand our collaborative efforts with new partners, we continue to cherish and nourish our long-standing links to groups and individuals who have helped make us who we are today, including 50

**Advocacy Amplified via Collective Impact**

As the group whose constituency spans a broad range of NMDs, MDA helps focus and unify efforts at the legislative and regulatory levels, advocating for changes that can address needs across these diseases.

MDA engages more than 9,000 advocates, reaching all 50 states across the U.S. The MDA National Advocacy Conference brings together over 100,000 individuals and families living with muscular dystrophy, ALS and related neuromuscular diseases to carry a unified message to Congress, and beyond. Additionally, MDA keeps advocates informed about key initiatives and provides channels for advocates to take action through a monthly newsletter distributed to 40,000+ individuals.

MDA is a leading voice in the patient advocacy community in our work with policymakers to ensure that all individuals living with neuromuscular disease have access to the health care they need through comprehensive health coverage.

We advocate for policies that maximize opportunities for independence. We also maintain an Accessible Air Travel Resource Center because the ability to travel by air impacts many aspects of life, including options for employment, education, and whether you can get back and forth from a clinical trial or a specialist’s office that is far from home.

MDA: A New Era in Neuromuscular Disease
Medical and Community Education

We offer a broad and expanding array of resources and events expertly developed to respond to the rapidly changing treatment landscape. Our resources for providing relevant medical education to professionals are unparalleled and our services and initiatives reflect our leadership in this area. We provide both accredited continuing medical education (CME) and non-CME programs.

As the most comprehensive neuromuscular disease meeting in the U.S., our annual MDA Clinical & Scientific Conference provides a unique opportunity to learn from, be inspired by, and share ideas with experts from academia, government, and industry.

At the community level, we established MDA Engage, a flagship educational event series that brings local high-impact educational programs to the NMD community. Each of the Engage programs incorporates multiple modules of interest, from therapy development roundtables to disease management to genetic testing, designed specifically for community audiences. Each event also includes a social element for families and participants with the aim of strengthening the community and helping attendees make personal connections.

Since 1950, MDA has served as the only national patient advocacy group supporting more than 43 neuromuscular diseases and the communities affected by these conditions.

Moving forward

Recent changes in care paradigms for neuromuscular diseases have greatly intensified the clinical, information, and advocacy needs of the communities we serve. MDA is changing with a clear mission, an expanded vision, and a renewed focus to meet the needs of a community undergoing a remarkable transition — and whose prospects for transformation have never been better. Join us in our revitalized efforts to create a new, better world for people with NMDs.

For more information, visit www.mda.org