A Message from Rodney Howell, MD, chairman, MDA Board of Directors, and Lynn O’Connor Vos, president and CEO, Muscular Dystrophy Association

We are living through unprecedented times in neuromuscular disease. Our work, across all of MDA, is paving a new path forward in terms of understanding neuromuscular disease and enabling life-changing innovations in research, treatment, and care.

In 2018 many of our efforts came to fruition with critical impact. Spinal muscular atrophy (SMA) was added to the Recommended Uniform Screening Panel (RUSP) for newborns. In 2018 we also launched the neuromuscular Observational Research Data Hub in 25 MDA Care Centers with plans to expand to 50 in 2019. And in December, the U.S. Food and Drug Administration accepted an application for the first-ever gene therapy treatment for a neuromuscular disease under Priority Review status.

We began 2018 by dialoging with key members of our community — neurologists and families — to gain deeper insight into their specific needs and respective experiences in working on and living with neuromuscular disease. Learning from this important research led to two landmark reports — “Understanding Neuromuscular Disease Care” and “ONEVoice.”

The Healthcare Partnerships Team was established in 2018 with the charter to build synergistic partnerships in the healthcare industry, starting with pharmaceutical and biotech companies and then expanding over time to other healthcare verticals including durable medical equipment (DME), retail and specialty pharmacy, diagnostics, and technology.

MDA has deepened its roots in the science of neuromuscular health and disease and re-dedicated our commitment to the support of clinical research initiatives. Since its inception, MDA has invested more than $1 billion in neuromuscular disease research to uncover new treatments and cures. We had 224 grants active during 2018 representing a funding commitment of more than $58M. We launched new educational seminars and programming for both healthcare professionals and our community.

We connected one-on-one with MDA individuals and families while responding to more than 20,000 inquiries received by our National Resource Center. And we made our annual Summer Camp program, “the best week of the year” for many of our kids and families, even better by offering families choices about where kids could attend.

In 2019, MDA has already seen some big accomplishments. In April we held our first ever combined Clinical & Scientific Conference, themed “Progress in Motion,” in Orlando Fla. Clinicians, scientists, policymakers, nonprofit, and industry leaders convened for a dynamic and informative five days. With more than 1,250 attendees, 23 sessions, 136 presentations, and 300 posters, this was MDA's largest conference ever. Also in attendance were 30 exhibitors, five companies on Technology Row, and 13 nonprofit partners in our Patient Advocacy Pavilion. The major themes of the conference were genetic medicine, clinical trials, regulatory science, emerging use of technology, and newborn screening. We were honored to have Dr. Janet Woodcock, director of the Center for Drug Evaluation and Research (CDER) at the Food and Drug Administration (FDA), provide the opening keynote address.

In May, the American Society for Gene and Cell Therapy (ASGCT) awarded MDA the Sonia Skarlatos Public Service Award for 2019. The 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.

We are so grateful and inspired by our Care Center teams, sponsors, volunteers, and donors for their unwavering support of our important mission. Thank you!
Not long ago, supportive care and mobility aids were all that could be offered to patients with NMDs. 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-supported research projects in 2018.

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

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.

More than $1 billion invested to date in accelerating the development of therapies and cures.
We are therefore bringing new resources and skills 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

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.

**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.

**Innovations in Science**

Support for MDA’s research enables us to fund teams working toward breakthrough therapies, which may have a life-changing impact on patients.

**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.
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.

In 2018, the Healthcare Partnerships Team was created to develop relationships with leading pharmaceutical and biotech companies in the NMD space. In addition, the team established and now manages an agency-caliber healthcare communication and educational platform. This includes offering accredited continuing medical education (CME) programs as well as community educational initiatives.

The team also manages MDA’s annual conference, which in 2018 brought together more than 500 medical and scientific neuromuscular experts. The conference focused on providing opportunities for physicians and allied health professionals to learn about new approaches and techniques for clinical management, hear about the latest information regarding clinical trial results, and engage in dialogue and networking among peers.

The team is responsible for publishing Quest, MDA’s quarterly publication, which has nearly 800,000 touch points annually.

The Healthcare Partnerships Team exists today to support the tremendous growth being seen in drug development and the critical educational needs of our community in response to new therapies and treatments being brought forward through R&D.
MDA has long appreciated that gene therapy, by targeting the very root cause of the disease, has tremendous therapeutic promise in a wide array of neuromuscular diseases.

**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.

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, clinicians, and members of industry.

**Industry recognition**

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

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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 US 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.
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 SMA is the leading genetic cause of death in infants in the US, 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 can play a key role in confirmatory diagnosis, 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 committed to understanding the natural history of neuromuscular disease. When babies are diagnosed early in life, it allows the opportunity to learn more about how the disorders manifest and to obtain insights into how early intervention affects disease course.
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.

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.

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:

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 US. The MDA Advocacy Conference brings together 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 healthcare 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.
Moving forward

Recent changes in care paradigms for neuromuscular diseases have greatly intensified the clinical, informational, 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
Meet our Partners

We believe great impact cannot be made alone. Together, with our generous partners, we’re raising life-changing funds and awareness for families with muscular dystrophy and related diseases. Since 1950, MDA has been proud to team up with caring corporations, organizations, and brands that generate tens of millions of dollars each year through creative campaigns, sales promotions, and year-round special events.

Go to https://www.mda.org/get-involved/meet-our-partners to learn more.

Partners in Progress

We are grateful to the following partners whose efforts and generosity each helped generate more than $3 million in 2018.

Continuing a 65-year tradition of giving strength to the MDA community, 100,000 dedicated firefighters hit the streets or storefronts in 2018 with boots in hand asking pedestrians, motorists, customers, and other passersby to donate to MDA raising more than $22 million. The partnership between the IAFF and MDA began in 1954 when the organization committed by proclamation to support MDA until a cure is found, and the organization’s unwavering commitment to MDA has remained strong to this day. As MDA’s largest national partner, they have raised more than $650 million for MDA to date.

As MDA’s largest corporate partner, CITGO Petroleum Corporation has raised more than $240 million for MDA through a wide variety of fundraising events, including golf tournaments, sporting events, in-store retail programs, and social events. CITGO also organizes special events in Houston; Lemont, Ill.; Corpus Christi, Texas; and Lake Charles, La., where the company has major operations. CITGO marketers and retailers throughout the country participate annually in the MDA Shamrock Program and other events.

Harley-Davidson Motor Company has put its commitment to freedom and independence into action by supporting MDA since 1980. During its more than 38-year partnership with MDA, the Harley-Davidson family of dealers, customers, employees, suppliers, and H.O.G. chapters raised more than $103 million to help save and improve the lives of MDA families.
It takes a village to achieve a mission as important as ours. When lives are at stake, every voice, every dollar, and every individual matters.

MDA is proud to be a grassroots organization with a national reach. Although we’ve been leading the fight against muscular dystrophy and related diseases for almost 70 years, we haven’t done it alone.

In decades past, people held backyard carnivals, participated in bike-a-thons, and donated to the telethon. Today, that incredible spirit of creativity, generosity, and unparalleled passion is as vibrant as ever in a new era of giving and innovation.

Nearly 1 million touchpoints for readers of Quest Magazine & MDA’s Strongly blog
MDA Fundraising Events

MDA Muscle Walk
MDA Muscle Walk is more than a fundraising walk — it’s a life-changing event that strengthens families and communities. In 2018, about 29,000 people participated in 140 MDA Muscle Walks, raising $6.1 million. Since 2011, MDA Muscle Walk has raised more than $43 million to help bring strength to life for the families we serve.

To learn more, and to sign up and participate, go to mdamusclewalk.org.

MDA Team Momentum
Team Momentum is MDA’s endurance training program that empowers individuals of all athletic abilities to train for a half or full marathon while supporting families with muscular dystrophy. In 2018, MDA Team Momentum — and its more than 725 participants — raised more than $1.2 million to help families. Since 2014, MDA Team Momentum has had 3,591 participants and raised a combined $5.29 million.

To learn more, and join a race event, go to mdateam.org.

MDA Shamrocks and other pinups
Each year MDA’s iconic St. Patrick’s Day fundraiser is supported by more than 20,000 retail locations across the country — including supermarkets, convenience stores, restaurants, and other community-minded businesses. The public also joined in our first Green Day for MDA, which was held in conjunction with the Shamrocks campaign. In 2018, MDA Shamrocks, including all pinups, raised more than $14 million, and for more than 35 years, partners, employees, and donors have helped raise more than $310 million.

To learn more, go to mda.org/shamrocks.

Your Way for MDA
Your Way for MDA is an online platform that gives MDA supporters the opportunity to decide how they’ll raise money for kids and adults in their community. In 2018, thanks to the public’s creative ideas, Your Way for MDA raised $110,919. Since the program began in 2015, MDA supporters have raised more than $500,000.

To learn more, and contribute to Your Way, go to mda.org/get-involved/ fundraise-your-way

MDA Distinguished Events
More than 36,000 people participated in 330 galas, golf tournaments and Topgolf tournaments across the country in 2018. More than $22 million was raised through these signature events to help advance MDA’s mission.

To learn more, go to mda.org/get-involved/paticipate-in-an-event
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FOR THE YEAR ENDED DECEMBER 31, 2018 (IN THOUSANDS)

Assets
Cash, cash equivalents, and investments $72,285
Receivables and other assets $10,735
Fixed assets, net $645
Total assets $83,665

Liabilities
Accounts payable and accrued expenses $5,758
Research and training grants payable $10,469
Line of credit $7,500
Pension and postretirement plan obligations $49,991
Total liabilities $73,718

Net Assets
Without donor restrictions $5,364
With donor restrictions $4,583
Total net assets $9,947

Total liabilities and net assets $83,665

Revenue
Special events, net $90,926
Contributions $12,043
Other Revenue $3,499
Total revenue $106,468

Expenses
Patient and community services $46,664
Research $18,763
Professional public health education $12,220
Fundraising $15,348
General and administrative $13,714
Total expenses $106,709

Financial Report

CONTRIBUTIONS 11%
SPECIAL EVENTS, NET 86%
OTHER REVENUE 3%
GENERAL AND ADMINISTRATIVE 13%
FUNDRAISING 14%
PATIENT AND COMMUNITY SERVICES 44%
RESEARCH 18%

2018 ANNUAL REPORT
ABOUT THE MUSCULAR DYSTROPHY ASSOCIATION

MDA is committed to transforming the lives of people affected by muscular dystrophy, ALS, and related neuromuscular diseases. We do this through innovations in science and innovations in care. As the largest source of funding for neuromuscular disease research outside of the federal government, MDA has committed more than $1 billion since our inception to accelerate the discovery of therapies and cures. Research we have supported is directly linked to approved, life-changing therapies across multiple neuromuscular diseases. We support the largest network of multidisciplinary clinics providing world-class care at more than 150 of the nation’s top medical institutions, and each year thousands of children and young adults learn vital life skills and gain independence at MDA Summer Camp and through recreational programs.

For more information, visit mda.org.