Muscular Dystrophy Association (MDA) Congressional Comment
21st Century Cures Initiative

The Muscular Dystrophy Association (MDA) is the leading nonprofit health agency dedicated to saving and improving the lives of individuals affected by more than 40 neuromuscular diseases, including the muscular dystrophies, spinal muscular atrophy (SMA), amyotrophic lateral sclerosis (ALS), Friedreich’s ataxia (FA), Charcot Marie Tooth disease (CMT), myasthenia gravis (MG), mitochondrial myopathies, and more. The diseases MDA fights are all rare disorders of the muscles or parts of the nervous system that control the muscles. They are progressive and cause the muscles to weaken and atrophy; in some disorders this can happen very rapidly. Individuals with these disorders can lose basic functions that many people take for granted, like walking, standing, speaking, swallowing or breathing. Some muscle diseases appear at birth, while others emerge during early childhood, young adulthood or even late middle age. Most are genetic in origin, and most limit life span and quality of life. There are few treatments, and no cures.

MDA is working tirelessly to change this picture by funding worldwide research to develop treatments and cures; by providing comprehensive health care services and support to MDA families nationwide; and by rallying communities to fight back through advocacy, fundraising and local engagement.

Currently serving more than 100,000 Americans representing every state of our nation, MDA supports a national MDA clinic network comprised of 200 hospital-affiliated multi-disciplinary clinical care centers that provide comprehensive, world-class care to people with neuromuscular diseases. In addition to optimization of health outcomes, many institutions that host MDA clinics serve as hubs for basic and translational research, while many MDA clinics themselves conduct clinical research and trials and participate in national clinical trial networks.

Since its inception more than 60 years ago, MDA has provided an unparalleled level of private funding to basic, clinical and translational research; young investigator grants; neuromuscular and research fellowships; a clinical research network infrastructure for ALS, Duchenne muscular dystrophy (DMD), and myotonic muscular dystrophy (DM); and the development and implementation of the U.S. Neuromuscular Disease Registry. While our investment – thanks to the generosity of our donors within the American public – has been substantial, we have not done this alone. The neuromuscular community is a well-organized, collaborative community that has benefited from the contributions of many private organizations, world-renowned academic leaders and federal partners. Furthermore, in recent years, the neuromuscular therapeutic pipeline has attracted biotech and pharmaceutical partners and drug development momentum has hastened.

Despite more than 6 decades of investments, collaborations and research progress, people living with neuromuscular diseases still are in urgent need of comprehensive treatments and cures. Infants diagnosed with spinal muscular atrophy type I (SMA I) typically die before age two. Adults diagnosed with amyotrophic lateral sclerosis (ALS) typically die three to five years from the time of symptom onset. Those with Duchenne muscular dystrophy (DMD) have an average life expectancy in the mid 20’s. And, like so many of the other neuromuscular diseases in MDA’s program, symptoms from these diseases – such as impaired ability to walk, lift one’s arms and head, turn over in bed at night, breathe
independently, maintain cardiac function – have a profound impact on an individual’s quality of life and that of their family.

While our therapeutic pipeline continues to move more promising therapies into late phase II and III clinical trials, families anxiously await the ability to access critical treatments, with few answers. **We want nothing more than for safe and effective therapies to be delivered to patient families at the earliest moment possible.** We applaud the House of Representatives and the United States Congress for devoting attention and resources to ensuring this pathway exists and to expediting cures and treatments.

**Questions posed by Congress**

*What programs or policies have you utilized to support and foster research, such as patient registries, public-private partnerships and venture philanthropy?*

**MDA’s U.S. Neuromuscular Disease Registry**

MDA has implemented the U.S. Neuromuscular Disease Registry which records types of medical care provided, disease progression and health-related and quality of life outcomes of patients seen in MDA clinics.

The goals of the registry are to 1) gain a better understanding of the course of illness for specific neuromuscular diseases, 2) collect data about genotype-phenotype correlations to allow for better prediction of disease progression based on genetic information, 3) collect longitudinal patient data that will enable benchmarking of best clinical practices 4) use registry data as a platform to develop and implement a clinical quality improvement program for MDA clinics across the country 5) provide outcome-related information about MDA clinics for families seeking medical care and 6) establish a database of individuals eligible for clinical trials in neuromuscular diseases and expedite research by easing the burden of clinical trial recruitment.

MDA’s Registry Advisory Board includes membership from a variety of sectors including academia and the patient community, as well as advisors from the National Institutes of Health and the Centers for Disease Control and Prevention. We are also working closely with pharmaceutical industry partners on ways to utilize the registry to inform clinical trial design and implementation and accelerate therapeutic pipelines. (For additional information, see [http://www.neurology.org/content/82/10_Supplement/P7.008](http://www.neurology.org/content/82/10_Supplement/P7.008) for Muscular Dystrophy Association U.S. Neuromuscular Disease Registry – Preliminary Findings.)

**Public-Private Partnerships**

MDA has many examples of partnerships between our funded programs and federally-funded programs yielding great progress.

An important example is the NIH Centers of Excellence Program (Wellstone Centers). First established by the Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Act (MD-CARE Act) in 2001, then reauthorized in 2008 and under consideration for amendment currently, they have yielded great advances in understanding the specific causes of the various forms of muscular dystrophy.
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dystrophies, the mechanisms of these diseases, identification of therapeutic targets, and now even clinical trial development. These Wellstone Muscular Dystrophy Research Centers serve as a model to follow for best incentivizing therapy development. Wellstone Centers each share core facilities and have unique research specialties about which they communicate and share data frequently and transparently; serve as training and career development grounds for scientists and clinicians; and foster industry collaborations. Further, this network approach to research yields a layered funding approach where government funding serves as the foundation, supplemented by nonprofit and patient advocacy research support and dollars, and further supplemented by private biotechnology and pharmaceutical research investments.

- Supported by four- to five-year NIH awards of $1 million direct cost per year (~$1.5 million total cost)
- Total of six centers
- Funding from National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), National Institute of Child Health and Human Development (NICHD), National Institute of Neurological Disorders and Stroke (NINDS) and National Heart, Lung and Blood Institute (NHLBI)

When the Wellstone Center program was first initiated in 2005, MDA matched the funding provided by NIH to each Wellstone Center ($1.5 million) to ensure that the centers would have the traction needed to launch. Wellstone Centers have contributed to the understanding of significant scientific concepts and technological advances. The Wellstone Centers Program supports high quality translational and clinical research advancing understanding and therapy development for Duchenne muscular dystrophy (DMD), myotonic dystrophy (DM), fascioscapulohumeral muscular dystrophy (FSHD), limb-girdle muscular dystrophy (LGMD), and congenital muscular dystrophy (CMD). Furthermore, several of the Wellstone Centers’ current clinical trials in muscular dystrophies were made possible by discoveries that originated within Wellstone Centers.

MDA has utilized this collaborative research network approach and has partnered in establishing, through direct funding of nearly $1 million annually, 15 elite MDA medical clinics (among its 200 hospital-affiliated clinics) to speed and support research focused on ALS (amyotrophic lateral sclerosis), Duchenne muscular dystrophy (DMD), and myotonic muscular dystrophy (DM).

**MVP – Muscular Dystrophy Venture Philanthropy (MVP)**

In the early 2000’s, MDA launched its translational research program, now known as MVP, exclusively focused on funding the discovery and commercialization of treatments and cures for neuromuscular disease. Dedicated to building on our organizational strengths and the expertise of partners within the field, MVP makes selective investments in companies with promising paths to market; conducts professional diligence in evaluating investment opportunities; builds on MDA’s investment since inception of more than $1 billion in science, manpower and infrastructure; and leverages investments at strategic points in the drug development process. Strengths of our program have included FDA consultation for clinical trial endpoints; a responsive physician network through MDA clinics; international scale registries with common core datasets; efficient clinical trial networks established with experienced clinicians for multicenter trials using standardized, validated endpoints; current natural history data; and access to world-class expertise in all disease areas. The program has helped move the field forward and has generated a number of successes where MDA’s early investments were...
leveraged into follow-on funding from a number of sources including industry and the NIH TRND program. One of our earliest successes through our translational research program funding mechanism includes:

- In 2005, MDA invested $1.5 million in a phase I and phase IIa study of Ataluren (formerly PTC 124) for Duchenne muscular dystrophy (CF Foundation also invested in this drug).
- In 2008, Genzyme and PTC Therapeutics signed a co-development deal worth up to $400 million to move the drug forward for four indications, including DMD and Cystic Fibrosis.
- Ataluren is now in phase III testing for DMD.

**Hosting Conferences and Focused Symposia**

MDA is committed to enhancing the communication of new research findings and information relating to the delivery of effective medical care across the various neuromuscular diseases. To achieve this goal, MDA hosts an annual conference series, with scientific and clinical conferences held in alternate years.

- MDA’s most recent Clinical Conference was held March 16-19, 2014. The Clinical Conference focuses on bringing together health care professionals who are dedicated to diagnosing and caring for patients with neuromuscular diseases. Clinic teams share critical updates on the diagnosis and medical, rehabilitative and psychosocial management of neuromuscular disorders, with the goal of promoting an effective team approach to providing optimal patient care across the 200 hospital-affiliated clinical care centers funded by MDA.

- MDA’s most recent Scientific Conference was held April 21-24, 2013. The sold-out event attracted more than 500 participants from around the world, focused on therapeutic strategies for neuromuscular diseases, with sessions highlighting different aspects of therapy development. Some 70 platform presentations and 200 poster presentations were included. The conference agenda focused on neuromuscular research strategies and cross-cutting themes — rather than individual diseases — including novel drug targets, biomarkers, therapeutic modalities and new animal models.

In 2012, MDA initiated a series of focused symposia, where a small group of experts discuss specific issues in neuromuscular disease research. These symposia have included:

- MDA/AFM joint evening symposium on gene therapy at the ASGCT annual meeting;
- Glial cells in amyotrophic lateral sclerosis;
- Translating academic research into drugs; and
- Newborn screening for Duchenne muscular dystrophy.
How can Congress incentivize, coordinate and accelerate basic research for diseases we know relatively little about?

The clinical and research community is in great need of robust financial support of clinical centers of excellence, cooperative clinical trial networks, basic science in muscle disease and removal of hurdles in the implementation of therapeutic approaches. Optimizing NIH funding of neuromuscular and motor neuron disease research and renewal of the Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments Act (MD-CARE Act) are essential components of providing resources to expedite lifesaving therapeutic development. Furthermore, as more and more therapeutics are anticipated to begin to move into human clinical trials, there is an urgency for clinical sites to become clinical trial-ready which requires additional funding resources, specialized personnel, and coordination within and among national trial sites.

MDA’s clinical and research community has voiced their need for support to go to the most productive research groups and to develop oversight bodies with subspecialist evaluation to determine funding of projects (i.e. neuromuscular specialists evaluate research proposals). Others have suggested providing tax incentives and grants to companies who devote significant resources to neuromuscular and rare disease therapeutic development.

Furthermore, researchers have expressed concern regarding the incredible cost to conduct clinical trials, with little incentive or reward to the developers in disease populations that are small. Clinical trial participation continues to be expensive (with participation being cost-prohibitive to some families) and time-consuming to both participate in and implement.

Significant resources are necessary to increase our understanding of clinical trial design and to incentivize pursuing this in rare disease, including funding travel for trial participants, expanding the number of clinical-trial ready sites and increasing the number of people trained to administer clinical research.

How can we work together to better translate advances in science into safe and effective new therapies for patients?

Developing strategic partnerships, increasing research funding streams, and raising awareness of clinical trial participation are all necessary to translate advances in science into therapies for patients.

Currently, there are basic barriers to obtaining an accurate diagnosis for neuromuscular diseases, as the vast majority of testing is processed by commercial laboratories at a high price to insurers and families. Insurance companies frequently deny authorization for genetic testing for neuromuscular diseases in which there are no treatments and it will not significantly alter the course of medical intervention; however, knowing the specific mutation of a patient is necessary to conduct clinical trials. A family then faces the decision of whether to pay hundreds of dollars, or even thousands in some cases, out-of-pocket to obtain an accurate diagnosis or live with a more “general” diagnosis which would preclude
them from participating in clinical trials. The barriers to therapeutic development exist from the moment of diagnosis, to great detriment to the clinical, scientific and patient communities.

Furthermore, this genetic testing often can be performed prenatally or at birth, allowing any potential treatments to begin as soon as possible (even an experimental treatment, with parental consent), before the disease has done irreversible damage to muscles. Early diagnosis also could inform parents’ future reproductive choices, and eliminate the long and frustrating “diagnostic odyssey” (search for a diagnosis) that many families undergo. Families can spend years seeking a correct diagnosis for their or their child’s symptoms. Yet, newborn screening has only been currently approved for one neuromuscular disease in a select few states – the fatal childhood onset Pompe disease, for which enzyme replacement therapies are effective. Having newborn screening in place for diseases such as Duchenne muscular dystrophy and spinal muscular atrophy could allow clinical trial participation of infants prior to experiencing irreversible muscle damage that comes with age and disease progression.

As therapy development in rare diseases such as neuromuscular disease continue, genetic testing will become increasingly important. Innovative therapies may increasingly rely upon hitting the appropriate drug target, which may be a very specific segment of a particular gene. Personalized medicine relies upon matching the appropriate therapy to the appropriate patient. Failing to do so results in increased cost for inappropriately targeted treatment, missed opportunities to evaluate the therapy, and potentially precluding a patient from participating in a clinical trial that would be appropriate for that specific patient and his or her mechanism of disease. With limited “shots on goal” in a small, rare disease population, we cannot slow down the drug development pipeline and must ensure we are maximizing success in enrolling clinical trials and bringing treatments to patients.

**How do you coordinate your research and outreach with other patients?**

For most of the 40 diseases MDA fights, there exists at least one disease-specific patient advocacy organization or family foundation. For some of the diseases, there are dozens of disease-specific organizations. MDA strives to maintain open communication with each organization to reduce duplication and foster collaboration whenever possible. There exist some examples where these partnerships have yielded great outcomes such as the collaborative effort around the MD-CARE Act during the past decade and the facilitation of the FDA ALS subpart 15 Hearing.

Equally as important have been collaborations and the exchange of best practices with organizations outside of the neuromuscular space. MDA serves in both leadership and active participatory roles in dozens of patient coalitions through groups where cross-over between communities allows for great exchange. These groups include the patient coalitions associated with the various institutes of the National Institutes of Health (NIAMS Coalition, Friends of NICHD, ORDR CPAG, etc), the Centers for Disease Control, the Food and Drug Administration, as well as organizations that exist to convene groups (BIO, Genetic Alliance, Faster Cures, NORD).

MDA has worked closely with the American Academy of Neurology and the CDC to develop and test standards of care for several of the neuromuscular diseases. The MDA U.S. Neuromuscular Disease Registry was, in part, developed to learn more about adherence to standards of care and how those interventions impact health outcomes.
The Muscular Dystrophy Coordinating Committee (MDCC) also provides an excellent forum to coordinate research and exchange ideas. Originally established by the MD-CARE Act, the MDCC consists of inter-federal agency representatives as well as muscular dystrophy patient advocacy organizations, including MDA. The MDCC has worked to eliminate duplication and maximize opportunities for collaboration with government and private partners. The MDCC developed the MD Action Plan in 2005 and is in the process of updating that Action plan.

**What is the role of government in your work, including any barriers to achieving your goals and advancing breakthroughs?**

For many decades, MDA led and funded treatment and cure-driven research efforts without formalized collaborations with federal partners. Since the passage of critical legislation such as the MD-CARE Act and the ALS Registry Act, funding into neuromuscular disease research academic and clinical entities throughout the nation has increased substantially. Our federal agency partners have begun to have the funding traction needed to move the needle for improving health outcomes for the hundreds of thousands of Americans who are living with these progressive and fatal diseases. Particularly as exemplified by the results of the MD-CARE Act, government has provided the focus and coordination, allowing organizations such as MDA to supplement and act as force multipliers in particular research areas. This exemplary model of public-private partnership has helped to draw biotech and pharmaceutical partners, and the neuromuscular landscape now contains opportunities that would have likely not existed without the involvement of the federal government’s funding and infrastructure resources. We must keep these collaborations and funding sources robust for our therapeutic pipelines to reach their full potential.

Some in the neuromuscular clinical community have expressed their concern that the current uncertain funding environment, characterized as “tenuous”, may provide the biggest barriers to advancing breakthroughs. One clinician describes it as follows:

“The funding environment in the U.S. makes it very difficult to take on a project that might not pan out in the next few years. I have several promising projects that are currently on the shelf; they are too risky to put resources into because other projects are more likely to provide the data I need for the next grant.”

-Submission to MDA Clinical and Research Community Survey on Congressional 21st Century Cures Initiative, June 2014

Others have commented that their local regulatory steps are “sluggish” and that resources are needed locally by clinical researchers to expedite human subjects review.
How should regulators evaluate benefit-risk? How do you work with regulators regarding benefit-risk? Can this process be improved?

In February of 2013, the Food and Drug Administration (FDA) sponsored a subpart 15 Hearing focused on ALS to assess how to accelerate therapy development in ALS and consider the benefit-risk equation in a disease where the risk of doing nothing outweighs the side effects of many trial therapies. MDA worked closely with the FDA to ensure that the Federal Register notice was widely disseminated to the patient, provider and research community; that a broad and representative demographic was represented in both docket submissions and day-of hearing testimony; and that the hearing itself was accessible to the targeted patient community.

Researchers and families have expressed the view that the severity and progression of the disease should enter into the discussion of the risk-benefit ratio. Any benefit may be worthwhile in these situations without causing overt harm due to the progressive nature of these diseases. The risk-benefit analysis is experienced differently for families who have a loved one with a rare and life-shortening disease. As one member of MDA’s clinical community states:

“Drug screening and safety are important, but it must be recognized that patients with uniformly fatal neuromuscular disease may be willing to endure side effects of an effective drug that may be unacceptable for a healthier patient population. I think that combining the safety and efficacy trials may help move promising drugs through the pipeline.”

-submission to MDA Patient Community Survey on Congressional 21st Century Cures Initiative, June 2014

In 2012, FDA initiated its Patient-Focused Drug Development (PFDD) program to obtain the patient perspective on certain diseases and their treatments. The FDA convened a series of very constructive public meetings to outline how it would proceed and to ensure that it had captured the patient voice for its PFDD meetings planned for Fiscal Years 2013-2015. During these meetings, MDA joined other leaders within the rare disease community as we worked through a number of benefit-risk issues that require special focus and attention, such as understanding the unique patient and parent perspective with serious or fatal pediatric onset diseases. These issues cut across specific diseases and raised important issues for regulators to consider. MDA supports such an approach for future FDA meetings, where categories or common issues are evaluated, rather than solely considering disease-specific issues. Focusing on common mechanisms of disease or recurrent issues not only ensures the best use of limited federal resources, it provides a foundation for FDA to draw upon in regulation and, more broadly, it ensures that all stakeholders benefit from one another’s experiences and expertise. For instance, it might be appropriate to focus on pediatric-onset neuromuscular diseases, or to consider categories of therapeutic options, such as exon-skipping which may be used in multiple diseases, or to consider the mental health effects of various forms of treatment on pediatric populations and their caregivers.
Are there success stories the committee can highlight and best practices we can leverage in other areas?

The Muscular Dystrophy Community Assistance, Research and Education Amendments (MD-CARE Act), originally enacted in 2001 and updated in 2008, represents a tremendous success in public-private partnership as incentivized by congressional leadership. In the last 14 years, this legislation has helped change the muscular dystrophy landscape by coordinating and focusing federal research on all nine forms of muscular dystrophy, studying epidemiologic data, and developing and disseminating patient care guidelines -- all of which have made a significant impact on the quality of life and life expectancy of children and adults diagnosed with muscular dystrophy.

NIH's research through the Senator Paul Wellstone Muscular Dystrophy Cooperative Research Centers, has impacted many of the scientific breakthroughs across the muscular dystrophies, and has led to the expansion and intensification of MD research, including the leveraging of significant non-federal sources of funding. Since 2001, there have been 67 clinical trials of drugs or therapies for muscular dystrophy and there are currently more than 40 human clinical trials underway. A number of the potential therapies now in clinical investigation can be traced to the basic research efforts sponsored by the Centers. The impact of the investment has extended beyond muscular dystrophies and the neuromuscular community, with discoveries within Wellstone Centers forming the basis of new conceptual models that have potential impacts on therapy development for the muscular dystrophies and beyond neuromuscular diseases. Specifically:

- In 2010, through combined Wellstone Center research funding and support from nonprofit patient advocacy groups, after years of not understanding the underlying cause of facioscapulohumeral muscular dystrophy (FSH), researchers showed that abnormal production of a specific protein was the major molecular cause of FSH. In 2012, researchers identified the first ever therapeutic target in FSH, and the research field exploded with all new routes of investigation and discovery and clinical trials. Commenting about this 2010 discovery, Dr. Francis Collins, Director of the NIH stated: “If we were thinking of a collection of the genome’s greatest hits, this would go on the list.” Kolata, Gina "Reanimated 'Junk' DNA Is Found to Cause Disease". The New York Times. August 29, 2010.
What is the financial burden of your disease? How would better treatments and cures help save money for your family and the federal government?

As part of our initiative to understand the economic burden of several neuromuscular diseases, in 2010 MDA commissioned The Lewin Group to estimate the economic impact on the United States of ALS, DMD, myotonic muscular dystrophy (DM) and spinal muscular atrophy (SMA). The study considered the direct, indirect and, ultimately, the total national economic burden associated with these diseases in 2010. It utilized commercial and Medicare claims data to estimate direct medical costs and a national survey of a random sampling of families living with each of the neuromuscular disorders to estimate indirect costs.

Using the moderate prevalence estimate, we estimated that the total economic cost of the diseases above was approximately $3.2 billion in 2010. We also estimated that the total national burden is in the range of $1.2 to $4.8 billion/annually (using the low and high prevalence estimates respectively).

When using the moderate prevalence estimates, among the diseases studied, ALS is associated with the highest annual national economic burden ($1.03 billion), followed by SMA ($957 million), DMD ($791 million) and MMD ($450 million). It should be noted that, due to low sample sizes of the populations, there is a large margin of error for the estimate for SMA. (Larkindale J, Yang W, Hogan P, Simon C, Zhang Y, Jain A, Habeeb-Louks E, Kennedy A, Cwik V. Cost of illness for neuromuscular diseases in the United States. Muscle & Nerve. 2014;49(3):431-438.)

In June of 2014, MDA modified the questions posed by Congress in the request for comment related to the 21st Century Cures Initiative and distributed two nationwide electronic surveys: one survey tailored to families registered with our organization and another tailored to clinicians and researchers within our neuromuscular community. We found that close to 70 percent of families with a neuromuscular disease experience financial problems as a result of their neuromuscular disease, and that this is consistent across diseases without respect to severity or life-threatening nature of the disease. These costs include not only co-pays, deductibles and other medical expenses, but also the expenses related to home modifications, the purchase of accessible transportation and in-home caregiving support.

The middle class is particularly hit with out-of-pocket costs as they often don’t qualify for income-related means-tested programs for health insurance and community-based support. The financial strain for families includes the loss of personal work productivity, family members leaving the workforce to care for the person with neuromuscular disease, the compounding financial strain of multiple family members being diagnosed with a genetic condition, and paying out-of-pocket to see neuromuscular specialists if the family’s insurance refuses to authorize the visit. One family we work with reports, “We actually declared bankruptcy and lost our house due to medical bills about 2 years ago.” We have heard from many families who barely make due, have lost their homes, or have had to declare bankruptcy as a result of their neuromuscular disease.

Many families surveyed also state that Congress must “consider other cost reductions that are recouped through decreased dependency on programs such as Medicaid, Social Security, and Medicare that would result from increased funding of therapy development.” -Submissions to MDA Patient Community Survey on Congressional 21st Century Cures Initiative, June 2014
How can Congress help?

The 21st Century Cures Initiative is precisely how Congress can help most. Developing and delivering cures is a highly complex endeavor that requires involvement and input from a variety of different interests. As exemplified through Congress’ unique ability and recent successes within our neuromuscular community to leverage resources and facilitate public-private collaborations among a variety of stakeholder groups, Congress’ role within the pipeline is critical. For instance, are there collaborations that could be furthered between the NIH and the FDA, to ensure that the FDA benefits from the vast knowledge and experience gained in the NIH? In our experience, the two agencies are working very well on behalf of patients, but are there further efforts that might help facilitate the transfer of research knowledge to the regulators?

Models such as the Muscular Dystrophy Coordinating Committee, funding approaches such as those seen within the Wellstone Centers, and continued commitment to biomedical funding and innovation are critical. But they are not enough.

For families living with neuromuscular diseases, there are few treatments and no cures.

And while Congress must continue to seek incentives to attract and keep biotech and pharmaceutical industry partners in our disease spaces, much congressional impact can be made further downstream such as increasing appropriations and funding to state public health laboratories and the federal agencies that support them so that we can apply life-saving drug therapies to all patient communities at the earliest moment possible (newborn screening) to achieve maximum treatment effect.

We must ensure that our processes maintain scientific rigor, while becoming more streamlined. Examples of such streamlining would include incentivizing universities to utilize centralized institutional review boards (IRBs) and looking to innovative clinical trial models such as those created by the National Institute of Health National Institute of Neurological Disorders and Stroke’s (NINDS) Neuro NEXT program.

In 2014, we as a nation have made great strides in therapeutic development and clinical innovation. And while we have begun to change the natural history of some of our neuromuscular diseases, we have not changed the outlook for people who are being diagnosed in physician’s offices throughout America every day. But we are so close. MDA is working tirelessly to change this picture by funding worldwide research to develop treatments and cures; by providing comprehensive health care services and support to MDA families nationwide; and by rallying communities to fight back through advocacy, fundraising and local engagement.

While our therapeutic pipeline continues to move more promising therapies into late phase II and III clinical trials, families anxiously await the ability to access critical treatments. We want nothing more than for safe and effective therapies to be delivered to patient families at the earliest moment possible. We applaud the House of Representatives and the United States Congress for devoting attention and resources to ensuring this pathway exists and to expediting cures and treatments. MDA is eager to partner with you to achieve this goal we’ve been working towards for more than six decades.