



MDA[®]
Services
for the Individual,
Family and Community

Dear Friends,



When our son Bryson was 5, we received news that no parent wants to hear — our boy is affected by a neuromuscular disease, Duchenne muscular dystrophy (DMD) to be exact. While we were shocked and heartbroken at the time, we quickly realized that our family could — and would — rise to the challenge.

One of the first steps we took was to call the Muscular Dystrophy Association. It didn't take long to realize this was a wise decision. It felt like our family grew exponentially overnight! With the support of the entire MDA community behind us, the challenges before us seemed less daunting and our fears about the future were replaced with optimism and hope.

Our new extended family includes MDA staff, doctors, nurses, clinicians, researchers, sponsors and other individuals and families who, like us, live daily with neuromuscular disease. We've realized we're not facing this journey alone — and you're not alone either.

In this booklet, you'll find information about MDA services that can make a critical difference in day-to-day life with neuromuscular disease. MDA provides medical care, up-to-date information about more than 40 forms of neuromuscular disease, online and face-to-face support, help locating and repairing durable medical equipment, summer camp for kids (Bryson's favorite!) and — above all — a world-class research program that someday will put an end to these diseases for good.

This booklet also will give you an idea of the many ways to get involved with MDA's mission. Volunteering with MDA has helped our family fight back against muscle disease — from raising funds for research, to lending our voices to advocacy campaigns, to sharing our experiences with others in the MDA community, to simply helping to spread more understanding about neuromuscular diseases.

It's now been several years since Bryson received his diagnosis, and our lives today are busy and fulfilling. Yes, muscle disease affects Bryson's life — but it does not limit it. Although there are challenges, there also is joy. Through it all, MDA has been there with practical help and real hope for treatments and cures. We wish you and your family the very best in your journey with neuromuscular disease, and hope that you, too, will turn to your local MDA community along the way for strength, help and hope.

With love and understanding,

Phil and Claire Foster
Parents of Bryson Foster
MDA National Goodwill Ambassador

The Muscular Dystrophy Association Is Here to Help



“We probably would not have survived the last four years without the help of the Muscular Dystrophy Association. When we were introduced to them, our lives changed. We were able to network as parents and meet other parents going through similar experiences. The staff at our local MDA office has been wonderful. They’ve just accepted us as part of their family.”

Robin Pennell, Virginia
Parent of a child affected by
limb-girdle muscular dystrophy

MDA is the gateway to information, resources and specialized health care for individuals and families coping with muscle disease. At 120 local offices around the country, MDA’s compassionate and knowledgeable staff provides support and guidance.

MDA’s comprehensive website (mda.org) is another valuable source of information about neuromuscular disease, research news and MDA programs.

This booklet provides basic information about MDA services and programs. For information specific to you, contact your local MDA office.

Turn to MDA with questions about:

- Specialized health care and referrals
- Research advances in your disease
- Durable medical equipment needs

- Explaining your disease to family members and others
- Connecting with others in your area with a similar diagnosis
- Informational brochures and publications
- Support groups (in person, on the phone and online)
- Support and resources for caregivers
- Free flu shots
- Presentations to your child’s school
- Referrals to community-based resources
- Advocacy
- How you can join the fight against neuromuscular disease
- ... and more.

MDA is here to help. If your local MDA office can’t address a specific need, they’ll help you find other resources. Don’t hesitate to contact MDA for assistance and answers.

MDA Is Hope

What is MDA?

In 1950, a group of adults with neuromuscular diseases and parents of children with these disorders joined forces to form the Muscular Dystrophy Association. Recognized by the American Medical Association “for significant and lasting contributions to the health and welfare of humanity,” MDA raises funds to defeat more than 40 forms of neuromuscular disease through programs of worldwide research, health care and support services, professional and public health education, and advocacy. The Association provides services in the United States and Puerto Rico through 120 local offices.

MDA’s programs are funded almost entirely by the voluntary contributions of concerned individuals and cooperating organizations.

These programs include:

Research

MDA is among the foremost funders in the world of research into neuromuscular diseases. MDA-funded scientists are at the cutting edge of research into cell-based therapies and testing of potential treatments for the more than 40 diseases under its umbrella. For the most current research news, go to mda.org.

Clinics (for more, see page 4)

At facilities in MDA’s nationwide network of clinics, individuals receive diagnostic and follow-up care from expert teams of muscle disease specialists.

Support (for more, see page 8)

You are not alone. MDA is here every step of the way, providing resources and





"When our son was first diagnosed, we didn't know anything about muscular dystrophy. Within 24 hours, we had contacted the Muscular Dystrophy Association and that was a fantastic move to make. We learned throughout that there was hope."

Doriann Myers, Nevada
Parent of a child affected by
Duchenne muscular dystrophy

guidance, assistance locating and repairing needed equipment, support groups, summer camps for youngsters, online "e-communities" and much more.

Information (for more, see page 9)

Knowledge is power. MDA conducts educational programs for individuals with neuromuscular diseases, the general public and medical professionals, including publishing a wide variety of print and audiovisual materials, and maintaining a comprehensive website at mda.org. MDA sponsors scientific symposia and other professional meetings to increase knowledge of neuromuscular disease among medical professionals.

Advocacy (for more, see page 10)

MDA's advocacy efforts are committed to making life better for people with neuromuscular diseases by providing representation in matters of public policy and research advancement, nationally and internationally; and by facilitating active involvement in these areas by the people it serves.

Accessing MDA services

Initial diagnosis

The first step to access MDA services is attending an MDA clinic to confirm your diagnosis of one of the neuromuscular diseases covered by MDA (see page 32 for a complete list).

If the disease diagnosed is not covered by MDA, referrals will be made to an appropriate health or community agency.

Assistance with medical services

MDA will assist with payment for select services through MDA clinics or local medical equipment providers. To maximize MDA resources, payment is made after private and public insurance and/or other resources have paid. Please be sure to contact the MDA office in your area before ordering services, if MDA payment is desired.

MDA Is Help

The MDA team

MDA District offices are your headquarters for access to specialized health care at MDA clinics, as well as for local support groups, summer camp, resource referral and more. Staff at your local MDA office can provide information, support and guidance.

In fact, MDA should be your first phone call when you have nonmedical questions about living with a neuromuscular disease. Medical questions should be directed to the MDA clinic team, and MDA staff can provide that contact information if needed.

In most areas, your primary contact at MDA will be the health care service coordinator (HCSC), who can connect you with all appropriate MDA services and provide

information about federal, state and local resources that may prove helpful.

The MDA clinic

MDA maintains a network of 200 specialized clinics across the United States and in Puerto Rico. Most MDA clinics are located in teaching hospitals, and many MDA clinic directors are university medical school professors as well as practicing physicians. MDA clinics are at the forefront of research and treatment methods; some clinics also serve as sites for clinical trials of the latest experimental therapies.

Local MDA staff can direct you to the closest MDA clinic, or you can find a local MDA clinic online at mda.org/locate, or by calling (800) 572-1717.



"The MDA clinics have been a very positive part of our lives, and they've really, truly become a part of our family. I really can't imagine what it would be like for our son and for us if there weren't an MDA clinic."

Sean Masters, Arizona
Parent of a child affected by
spinal muscular atrophy

MDA has more than 40 MDA/ALS centers across the country. MDA distinguishes some clinics as MDA/ALS centers because of the medical team's particular expertise with the disease and the research taking place there.

MDA has organized 15 of its elite clinics into networks to support and speed clinical trials of promising research. Clinics in the Duchenne Muscular Dystrophy Clinical Research Network are: University of California, Davis, in Sacramento, Calif.; Nemours Children's Hospital in Orlando, Fla.; Washington University in St. Louis; Nationwide Children's Hospital in Columbus, Ohio; and Children's Medical Center in Dallas.

Clinics in the ALS Clinical Research Network are: California Pacific Medical Center in San Francisco; Columbia University in New York; Emory University in Atlanta; Massachusetts General in Boston; and Methodist Hospital in Houston.

Clinics in the Myotonic Dystrophy Clinical Research Network are: University of Rochester (N.Y.); University of Florida in Gainesville; Ohio State University in Columbus; Stanford University in Stanford, Calif.; and Kansas University Medical Center in Kansas City.

The clinic team

MDA clinics utilize a multidisciplinary team approach, meaning individuals can see knowledgeable health care specialists from a variety of disciplines, all at one location.

Specialists can include:

- neurologists
- cardiologists
- physiatrists
- orthopedists
- psychologists
- pulmonologists
- social workers

- physical and occupational therapists
- nurse case managers
- speech/language pathologists
- dietitians
- genetic counselors

The MDA health care service coordinator (HCSC) is a central figure at clinic visits. He or she is usually present on clinic days to answer questions, distribute MDA educational materials, coordinate any MDA services you may require and assist with community resource referrals.

The diagnostic process

The first step in medical care is determining the nature of the disease. The MDA clinic team can perform diagnostic examinations and recommend pertinent laboratory tests.

Some of these tests may be extensive, yet in most instances they can be done on an outpatient basis. Information about genetic-based diagnostic testing is available at the MDA clinic.

Following clinical examination and analysis of the laboratory tests, many neuromuscular diseases can be quickly and accurately identified. Some neuromuscular diseases, however, can be more difficult to diagnose. In these cases, the physician will make what is called a "differential diagnosis," listing two or more diseases that have similar symptoms.

A definitive diagnosis may require waiting until the disease has progressed to a stage that is unique to that disease.

With the majority of neuromuscular diseases, the first noticeable symptom is usually a persistent weakness in one or more muscles.

Muscles can become weak for many reasons. The first question the physician will seek to answer in trying to establish a diagnosis is whether muscle function



“When I was diagnosed with muscular dystrophy, we didn’t know where to turn. But the Muscular Dystrophy Association was really there for me and my family. They walked us through what the disease was, what we could expect, how we should work together as a team to tackle this illness and make sure that it didn’t impair me from achieving my dreams.”

Vance Taylor, Maryland
Limb-girdle muscular dystrophy

is abnormal because there is a disease of muscle itself, or whether muscle function is abnormal because of a disorder that has developed in other tissue (e.g., nerve). The following two pages describe the most common diagnostic and laboratory procedures used to determine why muscle function is abnormal and arrive at a definitive diagnosis.

Diagnostic procedures

Neuromuscular diseases can present in a variety of ways and at different ages. Prior to receiving a confirmed diagnosis, you’ll likely undergo an array of testing to ensure that as much information as possible is obtained and other diseases are ruled out. Many treatment options are dependent on obtaining a specific diagnosis.

Among the various types of diagnostic tools your medical team may use are:

Clinical examination

The clinical examination of someone suspected of having a neuromuscular disease focuses on muscle appearance and strength. Muscles are examined with special attention given to those of the arms, legs, shoulders and hips. A few neuromuscular diseases affect facial muscles, and these too are examined.

Determining which muscles are not weak is as important as determining which muscles are weak. Each neuromuscular disease typically shows a specific pattern of muscle involvement. A final diagnosis is based in large part on the pattern of muscle involvement detected during the clinical examination.

Family history

Many neuromuscular diseases are genetic in origin. Knowledge of other affected family members can help to establish and/or confirm a diagnosis. However, it’s possible to have a genetic disease even without a family history of that disease.

Nerve conduction studies and electromyogram

These two tests often are performed at the same evaluation. The nerve conduction studies measure the ability of nerves to conduct impulses to muscle; this is an important test when evaluating for disorders such as Charcot-Marie-Tooth disease. An electromyogram (EMG) measures electrical activity of muscle. Muscle of someone with a neuromuscular disease may have an electrical activity characteristically different from that of normal muscle.

Serum enzyme tests

Serum enzyme tests measure the amount of muscle proteins present in the blood. When muscle tissue is healthy, these enzymes, particularly creatine kinase (CK), remain in muscle and the amount present in blood is relatively low.

Many, but not all, neuromuscular diseases that cause muscle damage lead to a significant increase in the muscle CK and other enzyme levels found in the blood. Thus, serum enzyme tests can be important aids in the diagnosis of neuromuscular diseases.

The value of these tests is often greatest at the early stages of the disease. At the earliest disease stage, muscle mass is relatively great and changes in serum enzyme levels may occur even before symptoms such as weakness become apparent. In some neuromuscular diseases, muscle enzyme levels fluctuate with disease activity.

At later disease stages, however, muscle mass may be so reduced that serum enzyme levels may even appear normal.

Genetic tests

A small amount of blood can be used to extract DNA from blood cells. This is extremely valuable for diagnosing genetic mutations which can cause specific neuromuscular diseases.



An MDA clinic physician may recommend that you consult with a physical, occupational, respiratory and/or speech therapist to assist you in managing symptoms of your neuromuscular disease and to maintain independence.

Muscle biopsy

In this procedure, muscle tissue is surgically removed for microscopic and/or biochemical analysis. For some neuromuscular diseases, a final diagnosis depends on the analysis of a muscle biopsy. The amount of muscle removed is roughly equivalent in size to the tip of a little finger. In some conditions, such as the inflammatory myopathies and central core disease, the muscle tissue has a characteristic appearance under the microscope. Muscle tissue can be analyzed for abnormalities in a number of proteins within muscle cells.

Managing neuromuscular disease

Upon diagnosis, a number of services may be recommended by the MDA clinic team as measures to medically manage neuromuscular disorders.

Periodic re-evaluations

Follow-up visits are provided at MDA clinics. Ongoing medical management can include therapies and measures for controlling symptoms, and medical interventions to assist individuals in maintaining the highest possible quality of life.

Therapy

An MDA clinic physician may recommend that you consult with a physical, occupational, respiratory and/or speech therapist to assist you in managing symptoms of your neuromuscular disease and to maintain independence.

Physical therapy: MDA will assist with payment for one consultation annually to (a) evaluate the need for physical therapy and (b) instruct family members and others on how to administer prescribed exercises. Physical therapy can keep still-healthy muscles functioning, help you maintain independence and prevent the onset of painful muscle contractures.

Occupational therapy: MDA will assist with payment for one consultation annually as prescribed. Occupational therapy enables people to make maximum use of their physical capabilities through the use of strategies and techniques, specially designed implements and daily living aids in the home and work environments.

Respiratory therapy: MDA will assist with payment for one consultation annually for instruction in the use of prescribed respiratory therapy equipment designed to augment or increase vital lung capacity.

Speech therapy: When prescribed by an MDA clinic physician, MDA will assist with payment for one consultation annually to evaluate the muscles responsible for speech and swallowing. Through such an evaluation, a speech-language pathologist can determine if exercise, use of an augmentative communication device and/or modifications to meals are appropriate.

Social services

Social services are a vital aspect of the MDA clinic program. MDA local offices and clinics may provide additional resources for families seeking to identify alternate sources of payment for medical services and equipment needs.

Social workers also may assist with connecting you to other community resources and providing emotional support for you and your family.

Genetic counseling

Genetic counselors can assist families by:

- obtaining and interpreting a genetic (DNA) or other appropriate test;
- obtaining a diagnosis for a genetic condition;
- determining whether parents of an affected child are carriers of the disorder;
- assessing the risk of passing on the disease to future children;

- determining whether other family members may be at risk; and
- exploring resources for payment of genetic testing.

Flu inoculations

Influenza is particularly hazardous to people whose respiratory muscles have been weakened by neuromuscular disease. Therefore, MDA assists with flu inoculations when medically recommended.



“There’s a multitude of support groups that MDA provides. We’re able to sit down in a private setting and share our experiences with other families. By doing so we realize that we’re not alone and we actually realize that if we can start sharing information with others and helping others, that we can also help ourselves.”

Jeff Vittek, California
Parent of a child affected by
myotonic muscular dystrophy

MDA support services

Support services help those with neuromuscular diseases, their families and caregivers address the challenges that accompany these conditions.

Local MDA offices offer regularly scheduled support group sessions that provide a chance to meet and share with others facing similar challenges.

Support services also are provided online at mda.org, and through *myMuscleTeam*, which enables families to keep friends and loved ones updated and assists in coordinating caregiving tasks. (For more on this program, see page 11.)

Equipment assistance

Equipment program

MDA assists individuals with obtaining medical equipment through its national equipment program and referrals to community resources.

MDA’s national equipment program is available to anyone affected by the diseases in MDA’s program for whom medical equipment has been recommended through the MDA clinic.

To the extent feasible and when available, MDA provides refurbished wheelchairs in good condition and other durable medical equipment when medically prescribed due to an individual’s neuromuscular disease. This includes wheelchairs, hospital beds, walkers and canes, communication devices and similar items.

Through its local field offices, MDA gratefully accepts donations of medical equipment for distribution through its equipment program. MDA is able to make minor repairs to gently used equipment donated to the Association for this purpose.

Many federal, state and local resources exist to assist individuals with equipment purchases. Local MDA offices maintain an extensive database of resources available in your area. You can contact your local MDA office at (800) 572-1717 to be connected with knowledgeable staff who will help you navigate the process of obtaining prescribed equipment.

Equipment repairs

MDA provides help with the cost of repairs to all types of medical equipment required due to an individual’s neuromuscular disease. To request such assistance, it’s important to contact your local MDA office before repairs are completed. The amount allowable toward repairs is established annually by the Association.

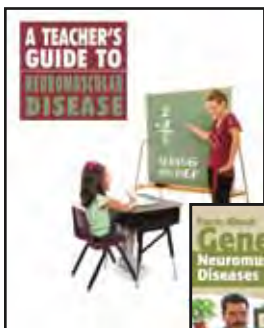
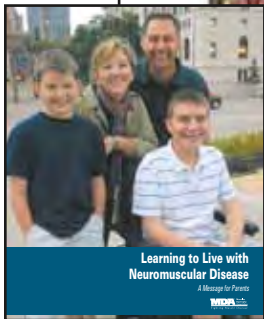
Community resources

Services not provided by MDA are often available from other organized community agencies. MDA assists in securing help from these community resources.



“One of the things that we’re really keeping up on is the status of research. That’s a big deal for us and there’s always updates on research that come through either Quest magazine or the MDA website.”

Bill Stehling, Arizona
Parent of a child affected by
Duchenne muscular dystrophy



MDA Is Information

MDA is an excellent source of current, accurate and practical information about all the diseases in its program, and about living with a neuromuscular disease. Information is available in print, video and online.

MDA website (mda.org)

This site contains thousands of pages of in-depth information, including:

- Information about each disease in MDA’s program, explaining the underlying biology, symptoms, interventions, inheritance patterns, current research and other relevant information
- News updates on research advances and health care issues
- Searchable back issues of MDA’s print and online magazines (Quest and the MDA/ALS Newsmagazine)
- Printable versions of all MDA publications
- State-specific resource guide and links to nationwide resources
- Advocacy information about pending legislation relevant to the MDA community
- Information about clinical trials and MDA research grants
- Information about MDA services, programs, resources and spokespeople
- Contact information for all MDA clinics and local offices
- Tools to help you get involved in the fight against neuromuscular disease
- Library of expert-hosted webinars on topics of importance to the MDA community
- Information for young adults living with neuromuscular disease through MDA’s Transitions Center (see page 11)

MDA/ALS Newsmagazine (alsn.mda.org)

This award-winning online publication features research news and analysis, blogs about life with ALS and monthly feature sto-

ries. It also provides practical information for individuals and families living with ALS.

Quest magazine (quest.mda.org)

This award-winning quarterly publication is free to those MDA serves. Each issue contains articles about research, health and living with muscle disease, including parenting, caregiving, independent living, helpful products and strategies, legislation and profiles of interesting people. Also, the online magazine site offers everything in the print magazine, up-to-the-minute research news and more.

Other MDA publications

Visit MDA’s disease pages (mda.org/disease) for a concise, informative overview of each of the more than 40 diseases in its program. Other publications include detailed guidebooks for caregivers, parents and teachers, and booklets about coping with medical and daily living challenges. All publications are free to those served by MDA, and can be downloaded online or obtained through your local MDA office.

Videos

MDA offers several professionally produced videos covering medical issues of interest to people with muscle diseases. These include:

- With Hope and Courage: Your Guide to Living with ALS (DVD)
- Breathe Easy: A Respiratory Guide for People Living with Neuromuscular Diseases (video)
- Breath of Life (video for medical professionals)

MDA Is Empowerment

Advocacy



“My ultimate happiness was found in the world of advocacy. I encourage anyone who is thinking about taking on a leadership role in advocacy. It is very rewarding. Receiving help is an art ... Through advocacy I can give back.”

Angela Wrigglesworth, Texas
spinal muscular atrophy

An important way to get involved with the MDA community is through the Association’s advocacy program. Based in Washington D.C., the advocacy team monitors and supports programs, legislation and health care policies relevant to people with neuromuscular diseases, such as medical care, insurance, accessibility, transportation, independent living, personal assistance services and research funding.

Sign up to be an MDA Advocate on the MDA website (mda.org/advocacy) and receive e-updates and up-to-the-minute news on policy issues impacting the MDA community.

MDA’s “Take 5” advocacy initiative encourages everyone to “take five minutes” to contact their elected officials using online tools that provide talking points about important legislation and contact information for elected officials.

MDA’s National Task Force on Public Awareness is a group of volunteers that advises the Association on issues of interest and importance to people with disabilities. The Task Force consists of adults who are leaders in their communities and who are affected by one of the neuromuscular diseases in MDA’s program.

Since MDA began, adults with neuromuscular diseases have been among its leaders as members of the Board of Directors and advisers. Under their guidance, MDA has educated the public about living with disabilities, and supported efforts to provide people with disabilities with equal rights and opportunities.

National legislation to which MDA lent leadership and support include:

- Prescription Drug User Fee Act (reauthorized; includes an accelerated approval pathway for drugs for rare diseases);
- Muscular Dystrophy Community, Research and Education (MD CARE) Amendments Act (passed into law);
- Spinal Muscular Atrophy Treatment Acceleration Act;
- Genetic Information Nondiscrimination Act (passed into law);
- Medicare Improvements to Patients and Providers Act (passed into law);
- Access to Complex Rehabilitation and Assistive Technology Act; and
- ALS Registry Act (passed into law).



“MDA camp is basically one of the most amazing places a child with muscular dystrophy can go. You can do anything from sports to crafts. We have a carnival, a dance. We ride horses. We have a petting zoo. We have a talent show. I mean, it’s just amazing.”

Caroline, MDA camper



Through the MDA Facebook community, we’ve met so many people who have been a great support. We can ask for advice, be encouraged and make friends with people who truly understand what our family is going through.

The Walters Family, Oklahoma
The family includes a son affected by Duchenne muscular dystrophy

MDA Is Community

Summer camp

MDA camp is a magical place offering a wide range of activities specially designed for youngsters ages 6 to 17 who have a neuromuscular disease. Camp offers an unmatched opportunity to develop lifelong friendships, share interests and build self-confidence. Activities are geared to the abilities of the campers and range from outdoor sports such as swimming, boating, baseball and horseback riding, to less physically demanding programs like arts and crafts, and talent shows. Volunteer counselors work one on one with campers around the clock, providing the care, close supervision and attention the campers need. Thousands of youngsters attend MDA camp each year at no cost to their families.

myMuscleTeam (mda.org/mymuscleteam)

MDA’s *myMuscleTeam* is a simple, free, powerful tool that helps MDA families maximize their most important resource — their “Muscle Team” of family and friends. Users create private, secure *myMuscleTeam* Web pages that enable them to post journal entries to keep friends and loved ones updated; and utilize a “care coordination calendar” where they can post requests for assistance and schedule volunteer help.

MDA Transitions Center (transitions.mda.org)

Focused on empowering young people with neuromuscular disease, the MDA Transitions Center provides comprehensive resources for independent living, personal attendant care, higher education, employment, transportation, recreation and health care. The interactive site features blogs, photo galleries, useful apps and Wiki projects.

MDA online communities

MDA hosts a variety of other online opportunities to meet and share with adults living with neuromuscular disease. In addition to Facebook (MDANational) and Twitter (@MDAnews), a slate of regularly scheduled chat sessions enable real-time interaction from the comfort of home. MDA chats (mda.org/services/finding-support/mda-chats) are organized by disease, roles (e.g., parents, caregivers) and themes (e.g., “positive thinking”).

Volunteer

Volunteers are needed for activities and events that raise awareness about neuromuscular diseases and raise funds for MDA’s research and services programs. Many MDA volunteers find that being part of a larger effort to defeat neuromuscular diseases fortifies and renews their spirits. Every local MDA office has a full calendar of events that depends on volunteers, and there also are opportunities for volunteers to help with administrative tasks — just ask!

MDA volunteer opportunities include:

- Golf tournaments
- MDA Muscle Walk
- Hop-a-Thon (disability awareness/fundraising program for young children)
- MDA Lock-Up (local business and community leaders are “arrested” and must raise their “bail”)
- Youth events (school clubs and organizations combine fun with fundraising)
- MDA summer camp
- Assisting in the local MDA office

To learn more, contact your local office or visit mda.org.

MDA's Purpose and Programs

The Muscular Dystrophy Association fights neuromuscular diseases through an unparalleled worldwide research effort. The following diseases are included in MDA's program:

Muscular Dystrophies

Myotonic dystrophy (*Steinert disease*)
Duchenne muscular dystrophy
Becker muscular dystrophy
Limb-girdle muscular dystrophy
Facioscapulohumeral muscular dystrophy
Congenital muscular dystrophy
Oculopharyngeal muscular dystrophy
Distal muscular dystrophy
Emery-Dreifuss muscular dystrophy

Motor Neuron Diseases

Amyotrophic lateral sclerosis (*ALS*)
Infantile progressive spinal muscular atrophy
(*Type 1, Werdnig-Hoffmann disease*)
Intermediate spinal muscular atrophy
(*Type 2*)
Juvenile spinal muscular atrophy
(*Type 3, Kugelberg-Welander disease*)
Adult spinal muscular atrophy (*Type 4*)
Spinal-bulbar muscular atrophy
(*Kennedy disease*)

Inflammatory Myopathies

Polymyositis
Dermatomyositis
Inclusion-body myositis

Diseases of Neuromuscular Junction

Myasthenia gravis
Lambert-Eaton (myasthenic) syndrome
Congenital myasthenic syndromes

Diseases of Peripheral Nerve

Charcot-Marie-Tooth disease
Friedreich's ataxia
Dejerine-Sottas disease

Metabolic Diseases of Muscle

Phosphorylase deficiency (*McArdle disease*)
Acid maltase deficiency (*Pompe disease*)
Phosphofructokinase deficiency
(*Tarui disease*)
Debrancher enzyme deficiency
(*Cori or Forbes disease*)
Mitochondrial myopathy
Carnitine deficiency
Carnitine palmityl transferase deficiency
Phosphoglycerate kinase deficiency
Phosphoglycerate mutase deficiency
Lactate dehydrogenase deficiency
Myoadenylate deaminase deficiency

Myopathies Due to Endocrine Abnormalities

Hyperthyroid myopathy
Hypothyroid myopathy

Other Myopathies

Myotonia congenita
Paramyotonia congenita
Central core disease
Nemaline myopathy
Myotubular myopathy
Periodic paralysis

MDA's website, mda.org, is constantly updated with the latest research news and information about the diseases in its program. Follow MDA on Facebook, Twitter, Google+ and YouTube.



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