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## A Promise of Progress

**I**n this time of unprecedented progress in neuromuscular disease research and care, it's imperative that we continue to build on our successes and keep the momentum going strong. MDA is changing the course of neuromuscular disease and improving quality of life for individuals and their families across the country — that's what keeps us going and fuels our efforts to push progress not only to its limits, but past them.

On the research front, MDA is actively uncovering the genetic clues of neuromuscular disease and developing ways to counter them by supporting clinical research networks, planning enhancements and expansion of our national neuromuscular disease registry, forging relationships with other organizations, and developing partnerships with pharmaceutical companies and other key industry stakeholders.

In addition, soon we will announce the award of 13 new research grants — any of which could lead to the next treatment or cure. (See page 6.)

On the care front, MDA's efforts to advance supportive treatments, refine the multidisciplinary care model and standardize best practices are helping individuals with neuromuscular disease live longer and enjoy better quality of life.

In March, we hosted the 2018 MDA Clinical Conference, bringing together more than 700 of the world's top experts in neuromuscular disease to learn about the latest treatments and care techniques. Attendees had valuable opportunities to network and share ideas, which are sure to lead to innovative solutions and previously unimagined approaches.

Of course, as we work to enhance research and care, we also are increasing and improving upon our pioneering community programs designed to empower individuals and families with services and provide needed resources and support to help them thrive and maintain independence. We're also looking at new ways to engage volunteers, which will strengthen our community and lend extra support to these programs. (See page 24.)

With so many possibilities within reach — for new discoveries, cutting-edge medical and scientific breakthroughs, innovative technologies and life-changing solutions — we're more committed than ever to fulfilling our promise of progress and transforming lives.



Lynn O'Connor Vos speaking at the 2018 MDA Clinical Conference

“We hosted the 2018 MDA Clinical Conference, bringing together more than 700 of the world's top experts in neuromuscular disease.”

– Lynn O'Connor Vos

Sincerely,

Lynn O'Connor Vos  
President and CEO  
Muscular Dystrophy Association

spring 2018

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## COMING SOON

## GET TO KNOW THE NEW MDA NATIONAL AMBASSADORS



*Faith Fortenberry and Justin Moy*

This year, MDA has selected two individuals to serve and represent families affected by muscular dystrophy and related neuromuscular diseases as National Ambassadors: 6-year-old Faith Fortenberry of Waco, Texas, and 17-year-old Justin Moy of Concord, Mass. Look for the next issue of *Quest* coming in Summer 2018 for exclusive interviews with Faith and Justin.



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## The Faces of Research

Every year, MDA awards research grants to the world's best scientists investigating promising theories and therapies that may accelerate treatments and cures for families living with muscle-debilitating diseases. On **strongly.mda.org**, you'll learn about some of the people who are doing that work, as well as the individuals who give their work meaning. Here are some excerpts from the Strongly blog.



Having previously taken and benefited from Emflaza, **Jag** is now part of a clinical trial for anti-myostatin. Since beginning the trial, there has been no decline in Jag's condition, and he has shown some improvement in

standing and endurance.

Although just 8 years old, Jag has traveled to Rome to see the Colosseum, admired the pyramids in Egypt, taken in the culture and sites in Nepal, and recently received Kai, his new service dog.



**Auinash Kalsotra**, assistant professor and Beckman Fellow at the University of Illinois at Urbana-Champaign, was awarded an MDA research grant to shed light on how defects develop in the heart in type 1 myotonic dystrophy (DM1).

*"This knowledge will undoubtedly lead to the development of new treatment modalities for this debilitating disease."*



**Angela Lek**, a postdoctoral research fellow at Boston Children's Hospital in Massachusetts, was awarded an MDA development grant to use cutting-edge techniques and a novel approach to search for drug targets in facioscapulohumeral muscular dystrophy (FSHD).

*"The main reason I entered the field of muscular dystrophy research is because my husband (LGMD researcher Monkol Lek) was diagnosed with a form of this disease."*



**Andrew Lieberman**, the Gerald Abrams Collegiate Professor of Pathology at University of Michigan Medical School in Ann Arbor, was awarded an MDA research grant to test a modified antisense oligonucleotide (ASO) therapy to treat spinal-bulbar muscular atrophy (SBMA).

*"The disease causes progressive muscle weakness only in men, and no therapies are currently available. My lab will be working to complete preclinical studies in a mouse model to establish the safety and efficacy of a new type of therapy."*

### MDA in the Lab

MDA funds cutting-edge research aimed at finding treatments and cures for neuromuscular diseases. Turn to page 6 to learn about our latest grants, or visit our Grants at a Glance page at [mda.org/gaag](http://mda.org/gaag).

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Tracking research updates and breakthroughs that help accelerate treatments and cures across MDA diseases

## MDA Research in Action

# MDA Makes First Grants Announcement of 2018

**New projects will impact a range of neuromuscular diseases**



In April, MDA is announcing the award of 13 new research grants with a total funding commitment of \$2.6 million.

The new grants are supporting research, development and research infrastructure projects around the world. They cover a wide range of diseases in MDA's program and are targeted to impact the greater neuromuscular disease landscape as we continue our efforts to develop treatments and cures. Highlights include:

- In Florida, scientists are working to determine the effects of abnormally expanded DNA in ALS and myotonic dystrophy. The results of these studies could point to new biomarkers for

these diseases and open up new possibilities for therapeutic intervention.

- In Alabama, researchers are continuing preclinical development of a compound that has been shown in studies to block inflammation and extend life span in animal models of Duchenne muscular dystrophy.
- In Pennsylvania, scientists are testing whether rescuing mitochondrial or synaptic

abnormalities in mouse models of Friedreich's ataxia can reverse dysfunction in the brain and lead to improvement of symptoms such as speech deficits and fatigue.

- In California, researchers are searching for pathways that could serve as potential drug targets in a process called autophagy – the fundamental metabolic process by which damaged cellular organelles and misfolded protein aggregates are degraded before they can lead to cell dysfunction. Results could point the way to therapeutics in inclusion-body myopathy, Pompe disease, centronuclear myopathies, vacuolar aggregate myopathies and other diseases in MDA's program.
- In Massachusetts, geneticists are creating a neuromuscular disease-specific program in order to pursue genetic diagnoses for individuals who go through the MDA limb-girdle muscular dystrophy testing program and do not receive a definitive diagnosis, as well as those under the age of 13 years who have an undiagnosed neuromuscular disease.
- In Australia, researchers are using a cutting-edge technique called CRISPR/Cas9 to optimize zebrafish models of Filamin C and BAG3 myofibrillar myopathies, and screen for drugs that could result in therapeutic benefit by increasing the level of functional protein or removing non-functioning proteins in these diseases.

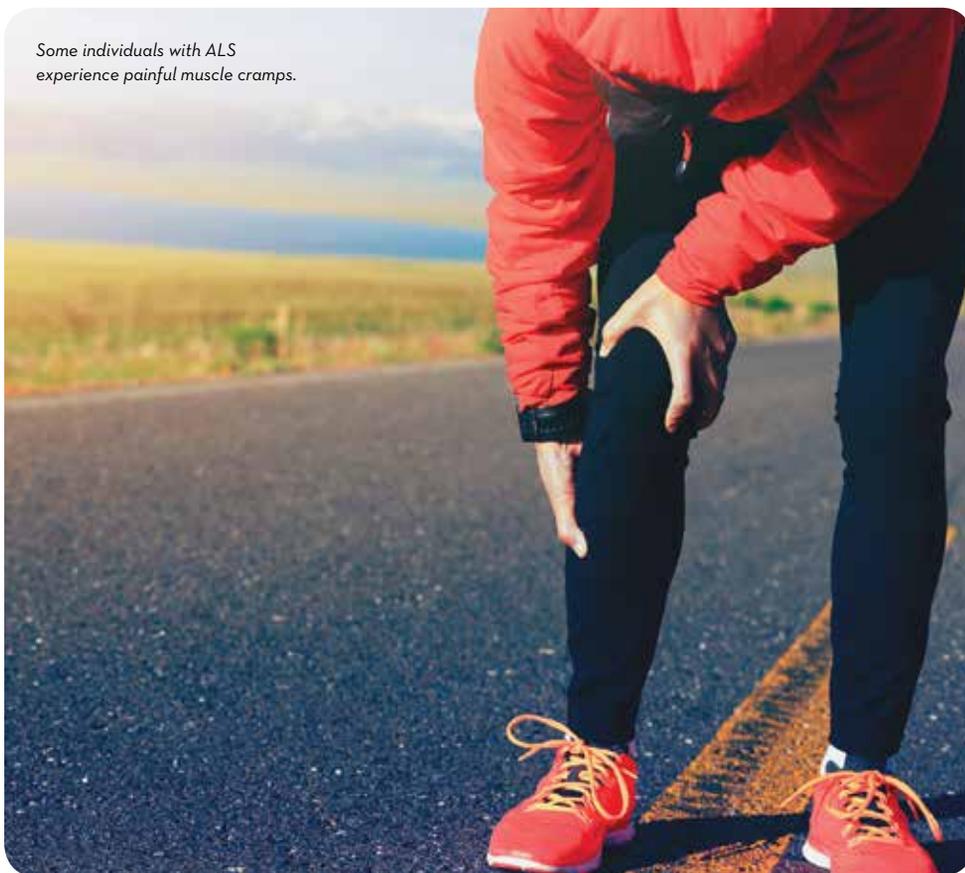
MDA currently is funding about 160 research projects worldwide.



Read more about MDA grants at [mda.org/gaag](https://mda.org/gaag).

## ALS (amyotrophic lateral sclerosis)

*Some individuals with ALS experience painful muscle cramps.*



# Trial to Test Muscle Cramps Drug Seeks Participants

## Investigators aim to assess safety and efficacy of FLX-787

Researchers are looking for people with motor neuron diseases (MND), such as ALS, to participate in a phase 2 clinical trial to evaluate the safety and effectiveness of the investigational drug FLX-787.

FLX-787, under development by Flex Pharma, is taken in tablet form and is designed to reduce muscle cramps in adults with MND.

Trial length is approximately three months, during which participants will visit with study investigators five times. For the duration of the study, participants will record muscle cramp frequency in an e-diary.

To be eligible to participate, individuals must be at least 18 years old with a documented diagnosis of MND, such as ALS; have a slow vital capacity of at least 50 percent predicted value; be able to walk unassisted or using an assistive device; be able to communicate verbally without difficulty; and meet additional criteria.

The trial is taking place at 15 study sites across the United States, and support for travel costs may be available.



To learn more about this trial, visit [leapcuremnd.studies.leapcure.com](https://leapcuremnd.studies.leapcure.com).

**ALS (amyotrophic lateral sclerosis)**



*A second-generation drug called CK-2127107 may be better tolerated and more effective than tirasemtiv.*

## Disappointing Results in VITALITY-ALS Trial

### Tirasemtiv program is discontinued

Cytokinetics reported negative results from its international phase 3 VITALITY-ALS trial to test the investigational drug tirasemtiv in people with ALS.

The goals of the study were to assess the effects of tirasemtiv versus a placebo on respiratory function and muscle strength. Investigators assessed slow vital capacity (SVC), a measure of breathing function, as well as other measures of respiratory function and muscle strength.

The trial missed its primary endpoint of change from baseline in SVC after 24 weeks of treatment with tirasemtiv as compared to placebo. Based on these results, Cytokinetics has announced it will suspend the development of tirasemtiv.

Although the negative results in the phase 3 ALS trial are disappointing, Cytokinetics reported that it has a second-generation drug called CK-2127107, which is thought to work through the same biological mechanism as tirasemtiv and may be better tolerated and more effective than tirasemtiv. (See Participants Sought for FORTITUDE ALS Trial.)

CK-2127107 currently is in phase 2 trials for ALS and spinal muscular atrophy (SMA).

 **If you participated in VITALITY-ALS, or the extension study VIGOR-ALS, and you have questions, be sure to speak with your physician.**

## Participants Sought for FORTITUDE ALS Trial

### Trial will assess safety and efficacy of CK-2127107

Researchers are looking for people with ALS to participate in FORTITUDE, a phase 2 clinical trial, sponsored by Cytokinetics, to evaluate the safety, tolerability and effectiveness of the investigational drug CK-2127107.

CK-2127107 is a fast skeletal muscle troponin activator, designed to increase the ability of muscle to contract by sensitizing it to calcium. The drug aims to increase muscle function and decrease muscle fatigue in ALS. In five completed phase 1 trials conducted in healthy volunteers, CK-2127107 proved safe and demonstrated the ability to increase muscle force. While this approach does not fix the underlying molecular problem in ALS, it could slow the progressive weakness in muscles associated with breathing and voluntary movement in ALS.

The outcome measures used in this study include slow vital capacity, muscle strength tests, ALS functional rating scale-revised (ALSF<sub>RS</sub>-R), voice recording, fine motor skills assessment, ALS Assessment Questionnaire (ALSAQ-5) and health outcome measures.

In order to be eligible to participate, individuals must be at least 18 years old with a diagnosis of familial or sporadic ALS, be able to swallow tablets and meet additional criteria. Support for costs associated with travel to a trial site may be available.



*Trial length will be approximately 18 weeks, which includes screening, treatment and a safety follow-up visit.*

 **To learn more about this trial, visit [clinicaltrials.gov](https://clinicaltrials.gov) and enter NCT03160898 in the "Other Terms" search box.**

## Duchenne muscular dystrophy (DMD)

# Ezutromid Shows Potential to Treat DMD

### Results hint muscle damage may be reduced

Summit Therapeutics plc announced encouraging interim results from its phase 2 PhaseOut DMD clinical trial that suggest the experimental treatment ezutromid (formerly SMT C1100) potentially may be effective as a treatment for DMD.

Ezutromid is designed to increase and maintain the production of utrophin, a muscle protein that has been shown in animal studies to partially substitute for dystrophin in muscle fibers. Dystrophin is the muscle protein that is missing or deficient in DMD.

The results showed that treatment with ezutromid was associated with reduced levels of a protein called developmental myosin, which is a biomarker that signals the presence of muscle damage. The combined data suggest that treatment with ezutromid may increase production of the utrophin protein and, in turn, protect muscle from damage. Additional clinical studies will be



*Trial results suggest that treatment with ezutromid may help restore and maintain the healthy function of muscles.*

needed to confirm these results and to determine whether the approach potentially could be a treatment for DMD.

MDA has supported the development of utrophin-increasing strategies for many years, including the early development and testing of ezutromid, which potentially could be of benefit in DMD regardless of the underlying genetic mutation that causes the disease.

The PhaseOut DMD trial is ongoing, with topline results expected to be reported later in 2018.



For more information about the PhaseOut trial, visit [clinicaltrials.gov](https://clinicaltrials.gov) and enter NCT02858362 in the "Other Terms" search box.

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**Facioscapulohumeral muscular dystrophy (FSHD)**

# ACE-083 Shows Promise in FSHD

**Participants sought for part 2 of a trial to test ACE-083 in humans**

Acceleron is developing ACE-083 to reduce muscle atrophy and weakness in the arms and legs for individuals with diseases such as FSHD and Charcot-Marie-Tooth disease (CMT).



Acceleron Pharma announced encouraging preliminary results from its phase 2 clinical trial to test the experimental drug ACE-083 in FSHD. Results from the completed part 1 of the 2-part trial showed that ACE-083 was safe and well tolerated by participants and that it potentially may be effective as a treatment for FSHD.

Delivered by intramuscular injection, ACE-083 is based on the naturally occurring protein follistatin, and is designed to enhance the body's own

promoters of muscle growth specifically in the muscles into which the drug is administered.

Preliminary results from the trial demonstrated that treatment with ACE-083 was associated with increases in total muscle volume by more than 12 percent in the two specific muscles – one along the shin, and the other in the biceps – that were evaluated.

Acceleron says the data support its decision to move forward with the next part of the trial, which it expects to begin during the second

quarter of 2018 at trial sites in the United States and Canada.

To participate, volunteers must be at least 18 years old; have a genetically confirmed diagnosis of FSHD1 or FSHD2, or a first-degree relative with genetically confirmed FSHD1 or FSH2; and meet additional criteria.

 **For more information about participating in this trial, including complete eligibility criteria, visit [clinicaltrials.gov](http://clinicaltrials.gov) and enter NCT02927080 in the “Other Terms” search box.**

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## Lambert-Eaton myasthenic syndrome (LEMS)

# Encouraging Results for Firdapse in LEMS Trial

**Data support the submission of a New Drug Application, planned for 2018**

Catalyst Pharmaceuticals has reported encouraging results from its second phase 3 clinical trial to test the investigational drug amifampridine phosphate (brand name Firdapse) in people with LEMS. The results support the company's plans to submit a New Drug Application to the U.S. Food and Drug Administration (FDA) in 2018.

Investigators found that Firdapse was safe and well-tolerated by trial participants. In addition, those who were treated with Firdapse saw stable

or improved scores on several assessment scales and strength tests conducted at different points throughout the trial, while those who were treated with placebo experienced a worsening of scores and symptoms.

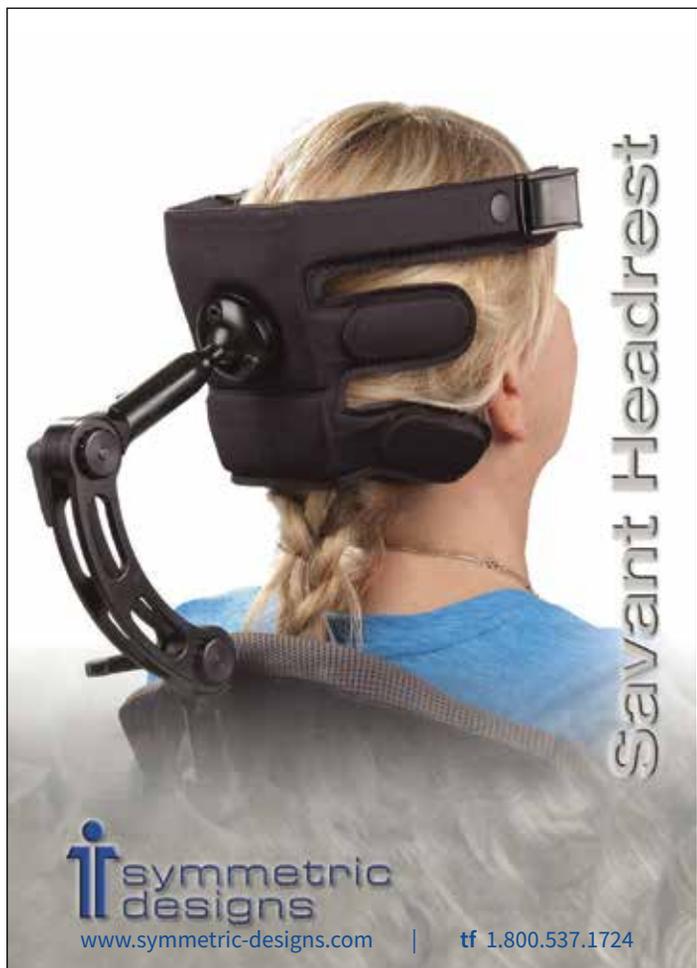
LEMS is an autoimmune disease – a disease in which the immune system attacks the body's own tissues. The attack occurs at the connection between nerve and muscle (the neuromuscular junction) and interferes with the ability of nerve cells to send signals to muscle cells.

Firdapse is a potassium channel inhibitor designed to prolong signals released from nerves and allow greater stimulation of muscles. The drug is under clinical investigation as a symptomatic therapy to treat LEMS, spinal muscular atrophy (SMA) and some forms of myasthenia gravis (MG). It also is under investigation as a treatment for adults and children with congenital myasthenic syndrome (CMS).

Catalyst Pharmaceuticals has an Expanded Access

Program (EAP) for Firdapse for individuals with LEMS or some types of CMS. The program is an open label pre-approval safety study, in which people who meet the inclusion and exclusion criteria can receive Firdapse, at no cost, when their physician determines it may help improve the person's condition.

 **For questions about the Expanded Access Program for Firdapse, call 844-347-3277.**



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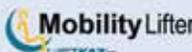
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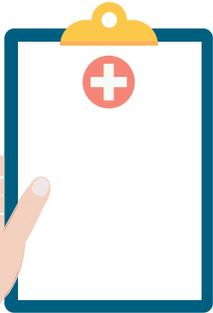
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## Myasthenia gravis (MG)

# Enrollment Is Open for MG Trial

Investigators assess the safety and drug effects of RA101495



*Trial length is approximately three months, during which participants will visit with study investigators in person seven times. At in-person visits, treatment effects will be measured across two MG-specific assessment scales, along with other clinical tests.*

Researchers are looking for people with MG to participate in a phase 2 clinical trial, sponsored by Ra Pharmaceuticals, to test the experimental drug RA101495 in people with generalized MG who have tested positive for acetylcholine receptor (AChR) autoantibodies.

Administered subcutaneously (an injection under the skin), RA101495 is designed to prevent the body's attack on the space across which nerve fibers transmit signals to muscle fibers, called the neuromuscular junction (NMJ).

RA101495 acts by targeting and blocking a part of the immune system called the complement system, which is responsible

for helping antibodies clear damaged cells and potentially toxic microbes that could cause infections. In patients with AChR autoantibody positive generalized MG, the body's own immune system turns on itself to produce antibodies against the AChR (a receptor located on muscle cells at the NMJ), activating the complement system.

Goals of the trial are to assess the safety of RA101495 and determine whether the treatment is able to reduce muscle weakness in people with generalized MG.

To be eligible to participate, individuals must have a diagnosis of generalized MG, test positive for AChR autoantibodies and meet additional criteria.

The trial is taking place at 21 trial sites across the United States, and support for travel costs may be available.



To learn more about this trial, visit [clinicaltrials.gov](https://clinicaltrials.gov) and enter NCT03315130 in the "Other Terms" search box.

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**Spinal muscular atrophy (SMA)**

# STRONG SMA Gene Therapy Study

**Participants sought to test safety and dosing of gene replacement therapy**

Researchers are looking for individuals with type 2 SMA to participate in the phase 1 STRONG clinical trial, sponsored by AveXis, to test safety, dosing and proof of concept for efficacy for its SMA gene replacement therapy AVXS-101.

In this study, the therapy will be injected once near the lower end of the spinal cord in a procedure called an intrathecal injection. AVXS-101 is designed to deliver a new gene that can produce the Survival Motor Neuron (SMN) protein. SMN is critical to the function of the nerves that control muscles and is missing or deficient in children with SMA. Increased production of the protein may lead to improvements in muscle strength and function.

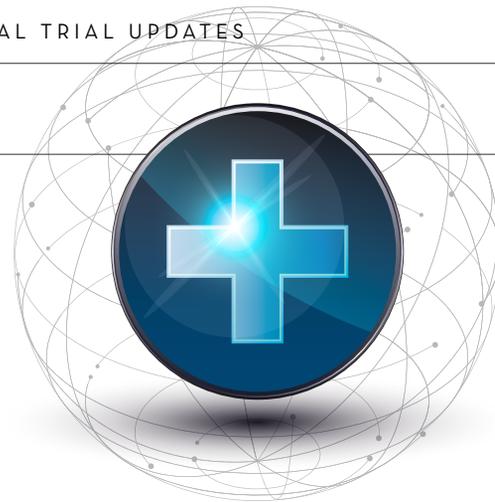
All participants in the open-label STRONG study will receive treatment with the experimental therapy. Study-related visits,

tests and treatments will be provided to participants at no cost. Participation is expected to last 12 months.

To be eligible to participate, individuals must be under the age of 5 years, have type 2 SMA, have three copies of the SMN2 gene, be able to sit unassisted for 10 or more seconds, be unable to stand or walk, and meet additional criteria.

Travel assistance may be available for families who don't live near one of the research centers.

 **To learn more about this trial, go to [clinicaltrials.gov](http://clinicaltrials.gov) and enter NCT03381729 in the "Other Terms" search box, or visit [studysmanow.com](http://studysmanow.com) and email the trial coordinator at the site nearest you.**



*Increased production of SMN protein may lead to improvements in muscle strength and function.*

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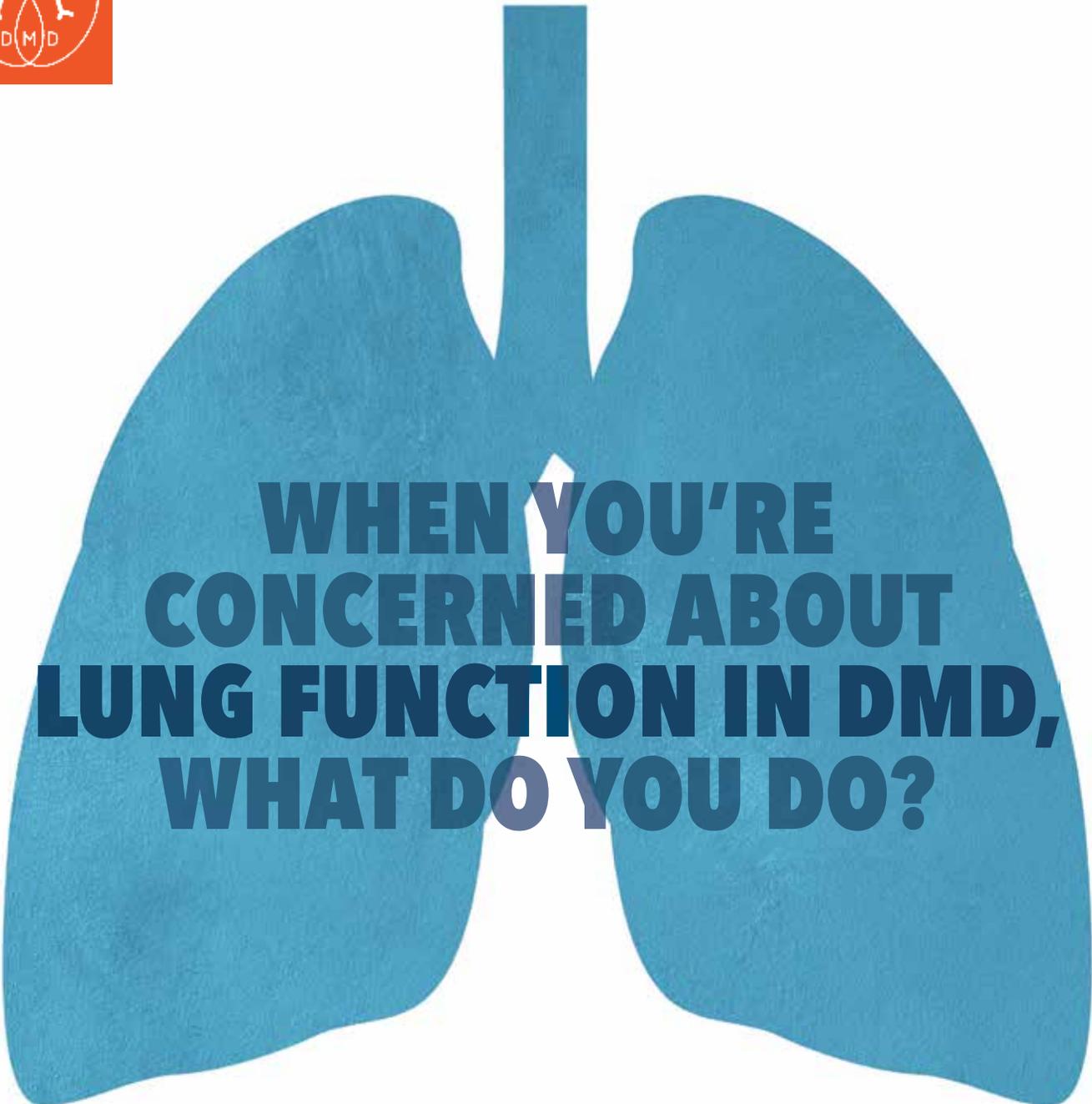
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and to sign up for news updates.

# The Great Accessible Outdoors

*With a little planning, outdoor adventures abound, no matter your mobility level*

BY CHRIS ANSELMO



Jessica Albanese at Everglades National Park in Florida

**J**essica Albanese, 20, is passionate about the outdoors. The Delaware native, who lives with limb-girdle muscular dystrophy (LGMD), has navigated several of our country's natural wonders in her power wheelchair.

"Traveling is a passion of mine," Albanese says. "I've hiked in the Great Smoky Mountains, I've seen the wild ponies at Assateague Island, and I've visited the alligators at Everglades National Park."

As the weather gets warmer, the great outdoors will beckon millions of Americans out of their winter slumber. While the natural world can be difficult to navigate for individuals with neuromuscular diseases, with the right preparation and knowledge, there are countless Live Unlimited moments waiting to be had.

## GET OUTSIDE

Outdoor activity comes in many different forms, whether it's visiting a local park, observing wildlife or camping in the woods. It also offers health benefits beyond helping you stay active.

According to a 2015 study, participants who went on a 90-minute walk in a natural setting experienced a greater mental health boost than participants who walked in an urban setting. Plus, being outdoors is a great way to get some sun, a vital source of mood-boosting vitamin D.

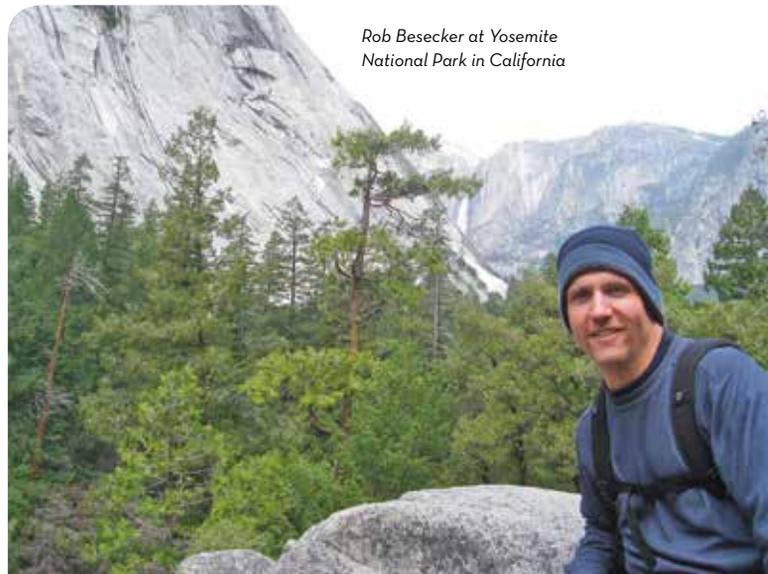
Rob Besecker, 43, who lives with myotonic muscular dystrophy, has found the outdoors to be pivotal in helping him



Cory Lee at Golden Gate National Recreation Area in San Francisco

manage his condition. "I love the fresh air and scenery that often accompanies being outside," he says. "Sometimes I feel too confined in my home, and it's a way to get out and enjoy what nature and the environment have to offer."

Besecker has been to many national parks and even trekked to Mt. Everest base camp ([evereststrong.com](http://evereststrong.com)). But you don't have to



Rob Besecker at Yosemite National Park in California

### More Ways to Play

Read more about outdoor activities, including how to find an accessible playground, in an online extended version of this article at [mda.org/quest](http://mda.org/quest).



visit faraway places to find adventure, says Cory Lee, who writes about his travels at [curbfreewithcorylee.com](http://curbfreewithcorylee.com). The 27-year old, who has spinal muscular atrophy (SMA) and uses a power wheelchair, is a firm believer that great outdoor experiences can happen right outside your door. “I would start local,” Lee says. “You don’t have to travel far away or hop on a plane to have awesome experiences. Go to a local park once a week, then try somewhere new every few weeks until you feel comfortable.”

## PLAN YOUR ADVENTURE

If you are visiting an outdoor destination for the first time, there will undoubtedly be some planning required. Shane Burcaw, 26, who lives with SMA, has traveled all over the country. As a power wheelchair user, he has learned not to make assumptions about a location’s accessibility. “I don’t always trust the word ‘accessible’ since it means so many different things to different people,” he says.

Seasoned adventurers recommend several steps to

find and prepare for exciting natural destinations:

- 1. Research, research, research.** To find outdoor locations near you, visit your city or state parks and recreation website. Travel blogs and articles are other great resources to generate ideas.
- 2. Seek advice from forums and Facebook groups.** If you are part of an online community for your condition, ask around for outdoor recommendations. Be sure to ask specific questions that pertain to your needs. If you are looking to get off the beaten path, “The Accessible Travel Club is my go-to Facebook group,” says Lee.

- 3. Call ahead.** Websites and travel guides may say a location is accessible, but they often don’t provide details. Call the location to learn if it has the accessibility features you need. Also ask about current conditions (i.e., are the accessible trails open and cleared?).

- 4. Look at Google Street View.** This is a great tool to get a preview of an outdoor

location. Google offers views inside some parks, with pictures that are relatively detailed. “This is an especially useful tool for planning my excursions,” says Burcaw.

- 5. Send a scout.** If you know someone who lives near your destination, ask them to visit the location and take pictures of specific aspects, such as trail surfaces, gradients and any other accessibility-related concerns.

## CONNECT WITH NATURE

For Lee, going outdoors is not just a way to connect with nature, but it has been a great way to connect with people.

“The thing that I enjoy the most about the outdoors is interacting with new people,” he says. “Whenever I do an outdoor activity, I tend to meet others, and we have a great time together. If you put yourself out there, it’s pretty easy to make friends.” **Q**

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*Chris Anselmo is a Connecticut-based freelance writer living with Miyoshi myopathy. He chronicles his journey with the disease at [sidewalksandstairwells.com](http://sidewalksandstairwells.com).*

## ENJOYING NATIONAL PARKS

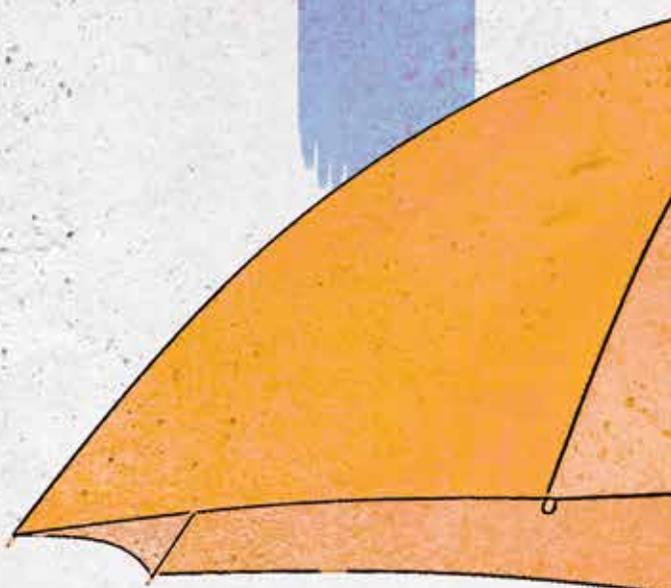
Of all our country’s natural wonders, our national parks are among the most famous and breathtaking. The National Park Service, which oversees 417 natural and historic areas, offers free admittance to any U.S. citizen or resident with a permanent disability through its Access Pass.

Jessica Albanese, an avid traveler, frequently uses her Access Pass. “Some national parks, like Acadia National Park in Maine, have a pretty high entrance fee, so it helps take some financial strain off of me to really enjoy the outdoors,” she says.

Shane Burcaw used his Access Pass to visit Yellowstone National Park last year and raves about his experience. “Yellowstone National Park impressed me with how many areas were wheelchair accessible,” he says. “The wooden and paved pathways take you right into the most beautiful areas.”

**To learn more about the National Park Service Access Pass, visit [nps.gov/planyourvisit/passes.htm](http://nps.gov/planyourvisit/passes.htm).**





THE

POWER

OF AN

UMBRELLA



*Spreading resources and support across dozens of neuromuscular disorders gives MDA big advantages*

BY AMY MADSEN

The logo for the Muscular Dystrophy Association (MDA) is displayed in white on the orange canopy of a large umbrella. The letters 'MDA' are stylized, with a heart shape integrated into the letter 'D'. A registered trademark symbol (®) is located to the upper right of the 'A'.

MDA<sup>®</sup>

A simple line-art icon of a DNA double helix, positioned to the left of the main text block.

Compared to organizations dedicated to raising awareness for single diseases, the Muscular Dystrophy Association stands out. As an “umbrella organization,” MDA covers not one, but dozens of neuromuscular disorders, funding research to discover underlying causes and develop treatments, providing care and resources, and advocating for policies to benefit the individuals and families who face the everyday challenges these diseases present.

A simple line-art icon of a medical professional, such as a doctor or nurse, wearing a white coat and a stethoscope, positioned to the right of the main text block.

Working across multiple diseases opens up unique opportunities for MDA to speed progress. For example:

- **MDA research dollars** go further, as the takeaways from MDA-funded research projects into one disease are often leveraged to inform and advance discovery and drug development in many other diseases.
- **MDA Clinical and Scientific Conferences** bring the world's top neuromuscular clinicians, researchers and industry partners together to network; share the latest news; discuss challenges, opportunities and potential solutions; and pinpoint possible areas to collaborate and share data and resources.
- **Clinicians and other medical professionals** at MDA Care Centers can offer solutions for dealing with symptoms in one disease based on what they've found to work on similar symptoms in other diseases.

- **The vast body of knowledge** and wealth of experience gained from more than \$1 billion in research investments over the last six decades fuels MDA's efforts to inform and influence policymakers on matters of importance to the neuromuscular disease community.

For more, read what some of the world's leading neuromuscular disease experts have to say about the power behind MDA's approach:

> **LORI WALLRATH, Ph.D.**, *professor and vice chair in the department of biochemistry at the University of Iowa in Iowa City, currently is receiving MDA support for her work to screen for drug targets aimed at restoring muscle function in rare types of muscular dystrophy.*

"An umbrella organization that covers dozens of neuromuscular diseases, such as MDA, brings together scientists and physicians from different areas to share ideas and discoveries, especially through events such as annual conferences. This cross-fertilization of ideas and practices is key to making progress at the lab bench and in the clinic.

"MDA brings an arsenal of tools and resources to the battle against neuromuscular diseases. MDA devotes substantial resources to advocacy, research and patient/family support. Their partnership with local and national organizations heightens public awareness and provides support for community activities such as the MDA Lock-Ups, Muscle Walks and Summer Camp experiences for children and teens.

"The benefit of an organization supporting multiple diseases is immediately apparent at the annual MDA conferences. When speakers who work on different diseases are grouped together in a session, underlying common features of the diseases are brought to light. Approaches used to treat a particular muscular



*"An umbrella organization that covers dozens of neuromuscular diseases, such as MDA, brings together scientists and physicians from different areas to share ideas and discoveries."*  
— Lori Wallrath, Ph.D.



Lori Wallrath, Ph.D.

dystrophy may quickly develop into a testable hypothesis for the treatment of a seemingly unrelated muscle disease.”

> **HEATHER DURHAM, Ph.D.**, professor in the department of neurology and neurosurgery at Montreal Neurological Institute and Hospital, McGill University, Québec, Canada, has received funding support from MDA almost continuously since 1998. Durham’s current MDA research grant is supporting her work to evaluate strategies that could prolong the function of motor neurons, the nerve cells that lose function and die in ALS.

“Part of making progress in the development of treatments for a neuromuscular disease is to understand the properties of the cells that predispose them to damage — for example, why motor neurons, glia or muscle cells fail when other cells manage to survive. MDA historically has invested in research into such basic functions of the cells in the neuromuscular system, with results that can be applied to increase understanding of multiple different disorders.

“Even more important is the cross-fertilization that occurs when scientists and clinicians focusing on specific diseases are brought together. We learn from one another and see commonalities in pathogenic mechanisms and treatment options.

“In terms of MDA Care Centers and neuromuscular clinics, there is tremendous efficiency in bringing together patients and providing the multidisciplinary care that makes such a

Heather Durham, Ph.D.



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## Grants at a Glance

MDA supports promising research projects across the full spectrum of neuromuscular diseases. Learn about the current MDA grants at [mda.org/gaag](http://mda.org/gaag).

difference in their lives. Particularly in areas with smaller populations, clinicians will see patients across the spectrum of neuromuscular diseases, and their practice needs to encompass all the services for multidisciplinary care.

“The Scientific and Clinical Conferences held by MDA are excellent and unique opportunities for continuing education across the spectrum of neuromuscular disorders and for scientists and clinicians to interact. I find these particularly enjoyable and beneficial as, along with other interactions, they lead to the sharing of tools and studies that otherwise might not be done.

“In fact, while speaking about my work in ALS at an MDA Scientific Conference, I became aware of complementary work by a colleague focusing on Friedreich’s ataxia. This led to new experiments, a collaboration with a pharmaceutical company and success with a major funding source.”

Susan T. Iannaccone, M.D.



> **ROBERT H. BALOH, M.D., Ph.D.**, *associate professor of neurology and director of neuromuscular medicine at Cedars-Sinai Medical Center in Los Angeles, received a development grant from MDA, which supported his early efforts to become an independent investigator with his own lab. Currently, with funding support from MDA, he is studying the molecular mechanism of type 2A Charcot-Marie-Tooth disease (CMT) due to mutations in the mitofusin 2 gene. His areas of clinical specialty include ALS, CMT, inclusion-body myositis (IBM), myasthenia gravis (MG), musculoskeletal disorders and more.*



Robert H. Baloh, M.D., Ph.D.

“While neuromuscular diseases each have their own unique aspects, they share many features in common, both clinically and pathophysiologically. That’s why neurologists receive subspecialty training in neuromuscular medicine, so that they have a broad knowledge of different diseases to make them a good diagnostician and physician.

“Likewise, in basic research there are many overlapping mechanisms in different diseases across the spectrum of neuromuscular disorders, so there is tremendous benefit in both taking care of patients and supporting research across a variety of neuromuscular disorders.

“Patients and families typically don’t come to the physician knowing what problem they have; they just know they need help, and often that they have some type of neuromuscular condition. It is important that they know they can go to an MDA Care Center and receive support regardless of their specific neuromuscular diagnosis.

“MDA has committed to fighting the entire range of neuromuscular diseases for children and adults. There is so much to be learned by coming together as a community of researchers and clinicians taking care of these individuals. MDA supports patients, families, and junior and senior researchers in a way no other organization has done for neuromuscular diseases.”

> **SUSAN T. IANNACCONI, M.D.**, *is a professor of pediatrics and neurology & neurotherapeutics at the University of Texas Southwestern Medical Center in Dallas. She also serves as co-director of the MDA Pediatric Care Center at Children’s Medical Center and principal investigator for the MDA-sponsored DMD Clinical Research Network. She received several MDA grants from 1988 through 2007 to support her work in natural history and treatment studies for spinal muscular atrophy (SMA).*



*“I also believe MDA’s clout as a large umbrella organization is instrumental in successfully lobbying for research funding and patient care.”*  
– Susan T. Iannaccone, M.D.

“I think the largest contribution by MDA has been to advance neuromuscular medicine as a science and a specialty. Focusing on detailed clinical aspects and physical diagnoses of patients was crucial for developing understanding of pathophysiology and, finally, strategies for treatment. Parallel to this was establishing neuromuscular disease training programs and curriculum for neurology training.

“I also believe MDA’s clout as a large umbrella organization is instrumental in successfully lobbying for research funding and patient care. The passage and renewal of the MD Care Act may be the single most important development in the history of muscular dystrophy research.” 

---

*Amy Madsen is a science writer and marketing communications manager for MDA’s research program.*

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*MDA volunteers discover many ways to help others with their time and talents*

# CHAMPIONS



FOR A



BY BARBARA & JIM TWARDOWSKI, RN

Throughout the year, thousands of volunteers support MDA in a multitude of ways: providing clerical services in MDA offices, serving on committees, working behind the scenes at events, securing auction items from restaurants and retailers, and much more. Whatever tasks are required, volunteers generously give their time and talents, supporting MDA hometown offices, MDA Summer Camp sessions and countless MDA events across the country.

Payton Rule (right), who lives with CMT, has volunteered with the St. Louis MDA office.



Payton Rule, 19, volunteered in the St. Louis MDA office during her senior year of high school, helping with clerical work. Diagnosed with Charcot-Marie-Tooth disease (CMT) at age 5, Rule got to know MDA by attending MDA Summer Camp for seven years.

Known as “the best week of the year,” the accessible camps are free to children

who have one of the neuromuscular diseases MDA programs support. During week-long sessions, an army of counselors and medical personnel provide nearly 3,800 campers with round-the-clock care and attention.

“The MDA camp was an impactful experience for me,” she says. It gave her a chance to live without limits and make enduring friendships.

Because of this experience and the guidance MDA provided after Rule’s diagnosis, she and her family are determined to give back.

“I love the organization. They’ve helped me a lot — through the MDA Care Clinic and other resources,” Rule says. “Volunteering is a way for me to give back to an organization I really admire.”

In the fall, Rule will be attending Washington University in St. Louis. Her goal is to become a neurologist and treat people who have neuromuscular diseases.

## FINDING YOUR PASSION

There are many ways to support MDA, from spending a few hours each week helping out at an MDA office to spending a day at an event, such as a Muscle Walk, or a whole week at an MDA Summer Camp.

When Matt Coppin, 38, graduated from college and was looking for employment, a friend invited him to help out at the MDA Summer Camp in St. Louis. Coppin became a camp counselor and began a lifelong relationship with MDA. “Through camp, I fell in love with the cause,” says Coppin, who took a job as a fundraising coordinator for MDA.

In his role with MDA, Coppin frequently met with fire fighters — the International Association of Fire Fighters (IAFF) is MDA’s largest national partner with its signature Fill the Boot fundraising program. Through these interactions, Coppin discovered his passion.



## HELP YOURSELF BY HELPING OTHERS

Studies show volunteers benefit from giving of their time. Incorporating service into your life can make you feel more accomplished and satisfied. Researchers have coined the term “helper’s high” to describe the positive feelings you have when doing something good for others. Some studies even say volunteering can improve your health. Supporting a cause you care about — like MDA — is a great way to reap the benefits of volunteering. Learn how at [mda.org/get-involved/become-a-volunteer](http://mda.org/get-involved/become-a-volunteer).

Matt Coppin (left) began a lifelong relationship with MDA through MDA Summer Camp.



He left his desk job behind and has been a fire fighter and paramedic for 12 years now.

“There are few things in life you can point to and say, ‘That changed my life.’ Going to MDA Summer Camp that first year really did,” says Coppin.

This summer will be his 19th year as a summer camp counselor, and throughout the year, Coppin serves on the Summer Camp Planning Committee and is the MDA liaison for the Metro West Fire Protection District Local 2665.

## SERVING AT SPECIAL EVENTS

MDA events like Lock-Up, Muscle Walk, golf tournaments, and galas put the “fun” in fundraising. Many volunteers are needed to plan and execute the charitable gatherings held in communities across the nation.

Amber Gross, 24, first volunteered with MDA as a Summer Camp counselor in Apopka, Fla., where she had a great experience. “It was a no-brainer that I’d come back again to create more great memories — not only for me, but most importantly for the campers. It’s my favorite week of the year,” Gross says.

Gross, who is a graduate student working toward a Doctor of Physical Therapy

### Make a Difference

Some people volunteer in several areas while others choose just one. No matter your time, interest or skill set, you can support MDA. To become an MDA volunteer, go to [mda.org/get-involved/become-a-volunteer](http://mda.org/get-involved/become-a-volunteer) and fill out a sign-up form. The local MDA office will call you to discuss what they need and what might be a good fit. To volunteer for MDA Summer Camp, visit [mda.org/summer-camp/volunteer](http://mda.org/summer-camp/volunteer).

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Amber Gross (right) volunteered at the MDA Orlando Toast to Life Gala in 2015.

degree, soon found opportunities to get involved at MDA events throughout the year. She has participated in two Muscle Walks and two Orlando Toast to Life galas, where she assisted with

set-up, explained to attendees how the silent auction works, directed speakers onto the stage and carted auction items to winners' cars.

### GETTING STARTED

"We have opportunities for everyone, no matter what they're looking to do," says Alicia Dobosz, MDA's director of recreation and community programs, who helps match individuals with volunteering roles that fit their time, interests and skills.

In fact, before she joined MDA, Dobosz was a volunteer. While working as a personal care attendant during college, Dobosz heard her clients speak so highly of MDA that she decided to



*"Volunteers really are the driving force that allows MDA to do what we do."*

*– Alicia Dobosz, MDA director of recreation and community programs*

volunteer at an MDA Muscle Walk in Chicago. Later, she learned that the MDA Family Support and Clinical Care team had an open position in Chicago. "I was so drawn to the mission I saw in action at the Muscle Walk that I applied right away," says Dobosz, who worked on the team for three years before moving to her current role.

"Volunteers really are the driving force that allows MDA to do what we do," she says. "Without volunteers, we wouldn't be able to move our mission forward supporting the programs and services we offer. Volunteers allow us to serve the number of families that we do and make the events the great experience they are for so many people." 

*Barbara Twardowski has Charcot-Marie-Tooth disease (CMT) and uses a power wheelchair. Jim, her husband, is a registered nurse. The couple lives in Louisiana and writes about accessible travel, health and lifestyle.*

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## What do I need?

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- An email address



## What if I need help?

Caregivers and others can help you in person or even over the phone



## What kind of information is collected?

- Basic demographics (e.g., age, sex, height, weight)
- Military history
- Physical activity
- Family history



## Do I need to update my information?

YES! Every six months – you'll get an email reminder



## Will my information be private?

- YES! Only approved registry scientists can see it, NOT employers or insurers
- You CANNOT be looked up in the registry by name



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More information for research

A better understanding of ALS

The chance to help create a better future for persons with ALS

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# COMMUNICATION

BY KAREN HENRY



*Augmentative and alternative communication aids help individuals with diseases that impact speech stay connected*

Individuals with ALS and other neuromuscular diseases that affect the ability to speak may feel as if their connection to the world around them is slowly shrinking. Fortunately, there is a growing number of augmentative and alternative communication (AAC) aids that can help people communicate when speaking is limited or no longer possible.

## Care from Day One

We know early diagnosis, highly specialized care and access to promising clinical trials help ensure the best possible outcomes for individuals and families living with muscular dystrophy, ALS and related neuromuscular diseases. To find an MDA Care Center near you, go to [mda.org](http://mda.org) and type in your state or ZIP code in the "Find MDA in Your Community" box.

*AAC aids fall into two general categories:*

### 1. LOW-TECH OPTIONS

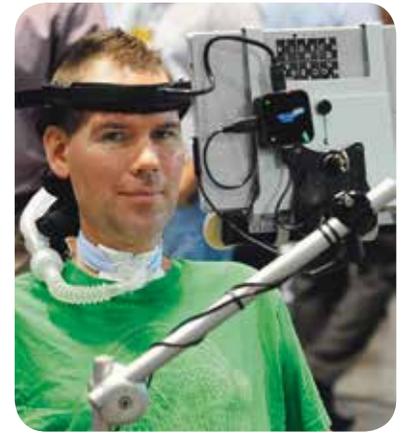
"For those who have arm and hand function, low-tech options could be as basic as using pen and paper as a primary method of communication," says Sarah Stranberg, MA, CCC-SLP, a speech-language pathologist at the MDA Care Center at Stanford Neuroscience Health Center. Other low-tech options include dry-erase boards; alphabet, word, message and picture boards; and voice amplifiers.

Picture boards can be particularly helpful for children and those whose neuromuscular disease impacts cognition. For those whose main problem with speech is volume, voice amplifiers can make a person's speech louder.

Low-tech AACs are widely available, affordable and easy to use.

### 2. HIGH-TECH OPTIONS

High-tech AAC options include commercially available products such as personal computers, tablets and speech-generating devices. In recent years, a variety of apps have entered the market that can turn a tablet or smartphone into an AAC device. Text-to-talk apps can be used to generate speech for those who are able to use their fingers to send text messages, and there are even a few mobile apps that use eye tracking technology.



Steve Gleason, a former NFL player who lives with ALS, uses an AAC device and has been active in promoting access to life-sustaining communication devices for individuals with ALS.

Most apps are free or relatively inexpensive and easy to use.

Eye-operated communication systems and voice banking currently represent the state of the art in the AAC field. Voice banking allows an individual to record his or her voice while it is functional and use it as a

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personal synthetic voice when needed. (Learn more about voice banking in the online exclusive article “Voice Banking Offers the Promise of a Natural Voice” at [mda.org/quest](http://mda.org/quest).)

Eye tracking technology allows an individual to operate a speech-generating device with eye movement. One of the main advantages of using eye tracking control with an AAC device is that it can progress with the user over time, which eliminates the need to learn to use a new piece of equipment as speech and muscle function become progressively limited.

### EMERGING TECHNOLOGY

A number of institutions are working on developing brain computer interfaces (BCIs), which allow people to use brainwaves to control computerized devices, including AAC devices. In addition to their potential for more intuitive control, BCIs hold great promise for individuals who are completely locked in, such as those in the advanced stages of ALS.

### TECH TRAINING

“There is always some level of training involved with speech-generating devices,” says Megan Case, MA, CCC-SLP, ATP, a speech-language pathologist at the MDA ALS Care Center at Ohio State University Wexner Medical Center. Training may happen at an MDA Care Center, in an individual’s home or at an outside clinic. The time needed for training varies from person to person.

Occupational therapists can assist in the evaluation process, help determine how an individual can use a

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## PAYING FOR HIGH-TECH COMMUNICATION AIDS

Speech-generating devices are considered durable medical equipment, so they must be prescribed by a physician and submitted to insurance for approval.

Once an individual and his or her speech therapy team has selected an AAC device, the speech therapist sends the recommendation and supporting documentation to the physician for review and sign off. The physician's prescription and documentation then go to the manufacturer of the AAC device, which is responsible for obtaining insurance approval.

Social workers can help identify funding resources for those who need financial support beyond what insurance will cover.

Erica Wright, LISW, a social worker at the MDA ALS Care Center at Ohio State University, says that she begins by checking to see if the individual is eligible for coverage in addition to his or her primary insurance, such as through Medicaid. She'll then look for funding sources outside of insurance.

For those who cannot afford a device right away, some states or local MDA offices may have loan programs that can provide devices on a temporary basis until funding is secured.

For help finding resources, contact the MDA Resource Center at **800-572-1717** or [resourcecenter@mdausa.org](mailto:resourcecenter@mdausa.org).

communication device if they cannot touch the screen, and teach them how to operate the device. After the initial training, regular follow-up appointments should be

scheduled to troubleshoot.

### CHOOSING AN AAC AID

"We don't always know if and when a person will need

a communication device, so the earlier on we can have that discussion, the better," Stranberg advises. "We want there to be something in place if and when they need it."

Selecting the most appropriate AAC aid begins with an intake evaluation at an MDA Care Center, which typically involves collaboration between speech, physical and occupational therapists. The MDA Care Center's multidisciplinary care team will gather information about the person's diagnosis, physical and mental status, current and future communication needs, and individual preferences.

"We want to select a communication aid that meets the individual's needs, not just the most popular device out there," Case says. "Some patients want nothing to do with high-tech AAC, so we explore what options will best fit their communication needs.

"Insurance only pays for high-tech equipment once every five years, so we want to make sure the client can use the equipment down the road," she adds. (See "Paying for High-Tech Communication Aids.")

Stranberg recommends that people who use high-tech communication aids also have a low-tech option available. "You always want to have something you can pull out of your bag to communicate no matter where you are," she says. **Q**

*Karen Henry is a freelance writer and editor living with limb-girdle muscular dystrophy (LGMD) in the Denver area.*

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# The first and only treatment for children and adults with spinal muscular atrophy (SMA)<sup>1</sup>

## SPINRAZA may help some individuals with SMA achieve and maintain motor function

### INDICATION

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

### IMPORTANT SAFETY INFORMATION

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

**The most common side effects of SPINRAZA include** lower and upper respiratory infections, constipation, headache, back pain, and post-lumbar puncture syndrome.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

This information is not intended to replace discussions with your healthcare provider.

**For additional Important Safety Information, please see brief summary of full Prescribing Information on the next page.**



## IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

### USES

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

### WARNINGS

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

### COMMON SIDE EFFECTS

- **The most common side effects of SPINRAZA include** lower and upper respiratory infections, constipation, headache, back pain, and post-lumbar puncture syndrome (headache related to the intrathecal procedure).
- Serious side effects of complete or partial collapse of a lung or lobe of a lung have been reported.

**Talk to your healthcare provider about any side effect that bothers you or that does not go away.**

### OTHER INFORMATION

SPINRAZA is a medication that should be administered as an injection into the lower back (a procedure called intrathecal injection) by, or under the direction of, an experienced healthcare professional.

### QUESTIONS?

The risk information provided here is not comprehensive. To learn more, talk about SPINRAZA with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at [www.spinraza.com](http://www.spinraza.com) or 1-844-4SPINRAZA (1-844-477-4672).

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# access MDA

Your guide to the MDA community, from news briefs to inspiring profiles

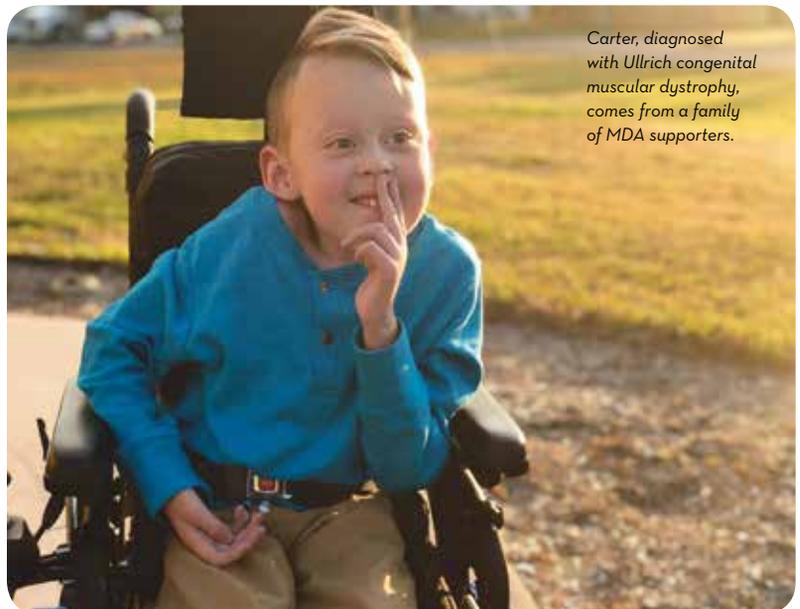
## Legacy of Support

After decades of raising money for MDA, a family learns its impact firsthand

Since she was a young girl, Tara Rhodes remembers spending Labor Day weekend raising money for MDA with her grandfather, Leroy Nottingham, a retired fire fighter in Fort Myers, Fla., and longtime MDA liaison for Fill the Boot. In 2015, their lifelong commitment to MDA became even more personal when Rhodes' 2-year-old son, Carter, was diagnosed with Ullrich congenital muscular dystrophy.

"We were basically at a loss; we didn't know what to do," Rhodes says. "I talked to my grandfather, and he said to call [MDA Divisional Director] Katie Bobin. She helped me get in touch with their healthcare coordinator and within a week of the diagnosis we were at the clinic."

As a longstanding MDA supporter, Nottingham knew that the organization would connect Carter to the best care for his condition, and he believes MDA offers the best hope of finding a cure. Carter, now 4, has been >



*Carter, diagnosed with Ullrich congenital muscular dystrophy, comes from a family of MDA supporters.*

Four-year-old Carter (right and below center) wants to be a fire fighter when he grows up.



seeing the same neurologist at his MDA Care Center since his first visit, and the center's multidisciplinary care team has been instrumental in getting him the care he needs.

Lately, Carter has replaced Rhodes as Nottingham's partner at Fill the Boots, MDA Lock-Ups and other MDA events. "He enjoys doing that with his great-grandfather," says Rhodes. The experience has been good for Carter. "He loves interacting with people, and he wants to be a fire fighter when he grows up," Rhodes says.

Rhodes and her family also attend the MDA Muscle Walk of Fort Myers, and Carter is looking forward to going to MDA Summer Camp when he is old enough. While Nottingham and Rhodes never expected to have a personal connection to MDA, they are now seeing how the organization they've supported for decades helps families like theirs.

 Learn how you can get involved with the MDA community in a way that's meaningful to you at [mda.org/get-involved](http://mda.org/get-involved).

## A Mission with Muscle

With its gala event, MDA Muscle Team Nashville mixes a fun evening with fundraising

Over the past three years, MDA Muscle Team Nashville has put on an annual sports-themed gala event, raising more than \$600,000 to benefit MDA's cause. The event brings together MDA families with sports figures, entertainers and business people from the Nashville community for a night

of food, entertainment and fundraising.

"It's a great evening," says Scott Hickman, a local attorney and Muscle Team Nashville chair. At each event, Hickman helps make sure everyone – from volunteers to sponsors and attendees – has everything they need to have a good time.

While it's a lot of work, Hickman does it all to support his 9-year-old nephew, Peyton, who has Duchenne muscular dystrophy (DMD) and attends the gala every year.

"For me, it's intensely personal," says Hickman, who has given a speech called the "Mission Moment" at each event. "Something I emphasize is that I treat this as if we're in a race, and we have to win the race to find the cure. We're helping kids go to MDA Summer Camp, helping them adapt and deal better with the effects of muscle disease, and that's helping them run faster. Then, the

The 2017 Muscle Team Nashville Gala was held at the Musicians Hall of Fame and Museum.



Christine Electra Williams, currently Miss Chattanooga, escorts Emily at the gala.

Shawn Parr (left), emcees the gala after years of being the voice of MDA's national telethon.



research is taking hold of the finish line and pulling it closer.”

Hickman's race analogy is especially meaningful to the crowd, which includes many athletes and sports fans. At past events, attendees have enjoyed hearing from Tim Shaw, a former Tennessee Titans linebacker who was diagnosed with ALS in 2014.

“His speech at the first event [in 2015] will be my lasting memory from the event no matter how long we do it,” says Hickman. “It was perfect. It was moving, it was funny. It was just masterful.”

And even though Hickman has been instrumental in the conception of the event and its smooth production, he is quick to point out that the contributions made by local MDA staff and the entertainers and celebrities – such as Shawn Parr, a nationally-syndicated on-air personality who emcees the event – are what really make it great. All of their contributions help bring awareness and funds to MDA.



Carlee is an MDA Muscle Team Nashville MVP.



**From hosting a gala to planning a bake sale, there are so many ways to fund-raise to help kid and adults living with neuromuscular disease. If you have an idea for a fundraiser, MDA will give you the tools to make it a success and cheer you on every step of the way. Learn more at [mda.org/yourway](http://mda.org/yourway).**

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# A Fresh Angle

**With his upcoming film, Bill Crossland brings a unique perspective on living with a disability**

Bill Crossland, a 29-year-old filmmaker from Bucks County, Pa., who has congenital muscular dystrophy, has been a movie lover for as long as he can remember. He traces his passion back to when he was 5 and he got his hands on a VHS copy of "Jurassic Park."

"Then I got a little camcorder, and not long after that I started making movies," says Crossland. "It just sort of escalated from there."

Crossland pursued his passion through high school, learning how to direct and edit while running the student news program, and he went on to study film at Temple University in Philadelphia. At Temple, Crossland got his first film set experience and made several short films. After graduating, Crossland started developing an idea for a romantic comedy feature film partially based on his own experiences as a young disabled man. He pursued the project on and off until eventually creating a short film based on his script.

"It was pure insanity from start to finish," he says of creating the short film. "I wore a lot of different hats – probably more than I should have, but luckily I had a team of people I trusted. I acted in it, directed it, wrote it and edited it. While I had some

experience directing, I hadn't really acted before, so that was a totally new experience."

Another totally new experience that came out of the short film, called "Catching Up," was receiving a national showcase for his film at the 2016 Sundance Film Festival. Crossland submitted his film to a few film festivals and was happy to show it locally, but getting accepted to Sundance was a shock.

"We didn't expect to end up there," he says. "So obviously we had to go. It was overwhelming, but the best part about it was the response we got from the crowds. I would introduce it and we would hang around



*Bill Crossland looks forward to premiering his film at the 2019 Sundance Film Festival.*

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afterward and people would say 'This is awesome, we need to see more of this.' The crowds really encouraged us to take it to the next level."

The response inspired Crossland to create a Kickstarter campaign to fund the development and casting of the feature film version of "Catching Up." Crossland hopes to have the film completed later this year, so it can premiere at the 2019 Sundance Film Festival.

Recently, Crossland has spoken at a few events for young adults with neuromuscular disease, covering topics that he wrote about in "Catching Up," such as transitioning into adulthood and the social aspects of having a disability.

"I think it'll be cool to put forth a new portrayal of a disabled character that's not a figure of pity or inspiration," says Crossland. "Realism is something I'm uniquely qualified to bring to the film, knowing that not every day [as a disabled person] is about your disability or finding a cure. It's life. This character has flaws, there are positive and negative things about him, and it's not one-note. That's what I'm most excited about, presenting a complex character, who happens to have a disability."



Bill Crossland (in wheelchair) on the set of his short film "Catching Up"

 Learn more about Bill Crossland's film, "Catching Up," and follow its progress on Facebook or on Twitter @CatchingUpMovie. You can also contact the filmmakers at [catchingupfilm@gmail.com](mailto:catchingupfilm@gmail.com).

An advertisement for the Zipie Voyage stroller. On the left, a woman in a black jacket is leaning over a black and red stroller, adjusting it for a child. The background is a blurred outdoor setting. On the right, the same stroller is shown from a three-quarter view against a solid green background. The text "LOOKS LIKE A STROLLER. LOOKS CAN BE DECEIVING." is written in white on the green background. The Zipie Voyage logo is also present.

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EMFLAZA is indicated for the treatment of Duchenne muscular dystrophy in patients 5 years of age and older.

Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

Please see Indication and Important Safety Information on the next page and accompanying brief summary.

  
**Emflaza**®  
(deflazacort)

6 mg | 18 mg | 30 mg | 36 mg tablets  
22.75 mg/mL oral suspension

## INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

### INDICATION

EMFLAZA® is indicated for the treatment of Duchenne muscular dystrophy in patients 5 years of age and older.

### IMPORTANT SAFETY INFORMATION

**Contraindication:** Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

**Do not stop taking EMFLAZA, or change the amount you are taking,** without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid “withdrawal syndrome”. Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal. A steroid “withdrawal syndrome,” seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.

- **Hyperglycemia:** Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.
- **Increased Risk of Infection:** Tell your healthcare provider if you have had recent or ongoing infections or if you have recently received a vaccine or are scheduled for a vaccination. Seek medical advice at once should you develop fever or other signs of infection, as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.
- **Alterations in Cardiovascular/Kidney Function:** EMFLAZA can cause an increase in blood pressure, salt and water retention, or a decrease in your potassium and calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.
- **Behavioral and Mood Disturbances:** There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.
- **Effects on Bones:** There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.
- **Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.
- **Ophthalmic Effects:** EMFLAZA may cause cataracts or glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.
- **Vaccination:** The administration of live or live attenuated vaccines is not recommended. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.
- **Serious Skin Rashes:** Seek medical attention at the first sign of a rash.
- **Drug Interactions:** Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medicines you are taking, including over-the-counter medicines (such as insulin, aspirin or other NSAIDs), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during the treatment.

**Common side effects that could occur with EMFLAZA include:** Facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds.

#### Please see the accompanying full Prescribing Information

For medical information, product complaints, or to report an adverse event, please call **1-866-562-4620** or email at **usmedinfo@ptcbio.com**.

You may also report adverse events directly to FDA at **1-800-FDA-1088** or **www.fda.gov/medwatch**.

# **EMFLAZA® (deflazacort) Consumer Brief Summary of the FDA-Approved Product Information Initial US Approval: 2017**

## **What is EMFLAZA?**

EMFLAZA® is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 5 years of age and older.

## **When should I not use EMFLAZA?**

- Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA

## **What should I tell my healthcare provider before taking EMFLAZA?**

It is important to tell your healthcare provider if you have had recent or ongoing infections, develop a fever, have recently received a vaccine or are scheduled for a vaccination, or experience any other side effects.

## **What warnings should I know about EMFLAZA?**

**Do not stop taking EMFLAZA, or change the amount you are taking,** without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid “withdrawal syndrome”. Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal. A steroid “withdrawal syndrome”, seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids.

For patients already taking corticosteroids during times of medical stress, the dosage may need to be increased.

**Cushing’s Syndrome:** Cushing’s syndrome occurs with prolonged exposure to exogenous corticosteroids, including EMFLAZA. Symptoms include high blood pressure, truncal obesity and thinning of the limbs, purple striae, facial rounding, facial plethora, muscle weakness, easy and frequent bruising with thin fragile skin, posterior neck fat deposition, osteopenia, acne, amenorrhea, hirsutism, and psychiatric abnormalities.

**Hyperglycemia:** Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.

**Increased Risk of Infection:** Medical advice should be sought immediately if you develop a fever or other signs of infection as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.

## **Alteration in Cardiovascular/Kidney Function:**

EMFLAZA can cause an increase in blood pressure and water retention or a decrease in your potassium or calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.

**Behavioral and Mood Disturbances:** There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.

**Effects on Bones:** There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.

**Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.

**Ophthalmic Effects:** EMFLAZA may cause cataracts or glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.

**Vaccination:** The administration of live or live attenuated vaccines is not recommended. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.

**Serious Skin Rashes:** Seek medical attention at the first sign of a rash.

## **What are the side effects that could occur with EMFLAZA?**

- facial puffiness or Cushingoid appearance
- weight increased
- increased appetite
- upper respiratory tract infection
- cough
- frequent daytime urination
- unwanted hair growth
- central obesity
- colds

## **What other medications might interact with EMFLAZA?**

Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medication you are taking, including over-the-counter medicines (such as insulin, aspirin, or other NSAIDs), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during treatment. Do not take EMFLAZA suspension with grapefruit juice.

**The information presented is not comprehensive. Talk to your healthcare provider for more information or see [www.EMFLAZA.com](http://www.EMFLAZA.com) for the full FDA-approved product information.**

For medical information, product complaints, or to report an adverse event, please call **1-866-562-4620** or email at [usmedinfo@ptcbio.com](mailto:usmedinfo@ptcbio.com).

You may also report adverse events directly to FDA at **1-800-FDA-1088** or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

# Seeking an End to ALS

Community-minded coffee chain Dutch Bros raises millions to support MDA



For one day each year, Dutch Bros Coffee locations donate all proceeds to MDA.

On May 5, 2017, Dutch Bros Coffee, a coffee chain with more than 290 locations across seven states, raised over \$1 million for MDA. Every year, the chain holds a one-day promotion called “Drink One for Dane,” where it donates all of its proceeds from that day to MDA, in honor of Dutch Bros co-founder Dane Boersma, who was diagnosed with ALS in 2005 and passed away from the disease four years later.

Dutch Bros was founded by Dane and his brother, Travis, the current CEO, more than 25 years ago as a single coffee cart in the brothers’ hometown of Grants Pass, Ore. Since then, Dutch Bros has expanded across the Western United States, building a network of coffee shops and, more importantly, a tight-knit community.

“We have that family feel,” says Travis. “Our common quest is this vision of a compelling future and that resonates with the communities that we’re in.”

In fact, Dutch Bros franchise owners and operators are entirely made up of employees who worked at the coffee shops, most of whom started >



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out preparing and serving coffee as “broistas.” So when the chain created the “Drink One for Dane” promotion in 2006, which usually takes place in the first week of May to coincide with ALS Awareness Month, it was personal, not just for Travis, but for the whole company.

“We’re a people business, and company-wide we rally together,” says Travis. “Many [of our franchise owners and employees] knew Dane and were impacted by this. Without the people we have nothing; it starts with our customers and then to our broistas and franchise owners, and that’s what has driven it. The money is the thing that we hope can serve as a resource for people who are determined and driven to go kill and squash this thing.”

Since 2006, the “Drink One for Dane” promotion has brought in more than \$3.5 million for MDA. And while the promotion honors Travis’ brother and co-founder by name, he says that Dane would want the event to be celebrated because of how it reinforces the ethos of Dutch Bros: community.

“Dane was a warrior,” he says. “He was the most selfless man I’ve ever met and he is the reason that our culture is what it is today and that our business is so alive. I think, if anything, he would want [the promotion] to be about others.”

 **For more information about Dutch Bros and the “Drink One for Dane” promotion, which will be held in May, visit [dutchbros.com](http://dutchbros.com).**

## Swell on Wheels

Harley-Davidson Director of Government Affairs Ed Moreland (left) trades in his leather jacket for a symbolic white lab



coat, a special award to recognize Harley-Davidson’s fundraising efforts for MDA’s research program. The Harley-Davidson family has raised more than \$103 million over 38 years to advance treatments and cures for neuromuscular diseases. MDA President and CEO Lynn O’Connor Vos (center) and MDA Chairman of the Board R. Rodney Howell, M.D., (right) presented the award.

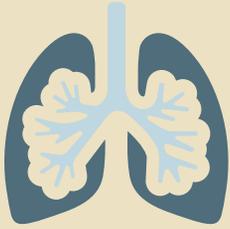
## MDA Shamrocks: Symbols of Strength, Independence and Life

Thank you to our top 2018 MDA Shamrocks national partners! These dedicated corporate partners and their caring employees and customers, as well as many others across the country, help raise funds for neuromuscular disease research, care and support through the nation’s largest St. Patrick’s Day fundraiser.



Muscular Dystrophy Association  
[mda.org/shamrocks](http://mda.org/shamrocks)





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## The therapy

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"Endorsed by Rob Roozeboom (pictured right), member MDA National Task Force on Public Awareness"

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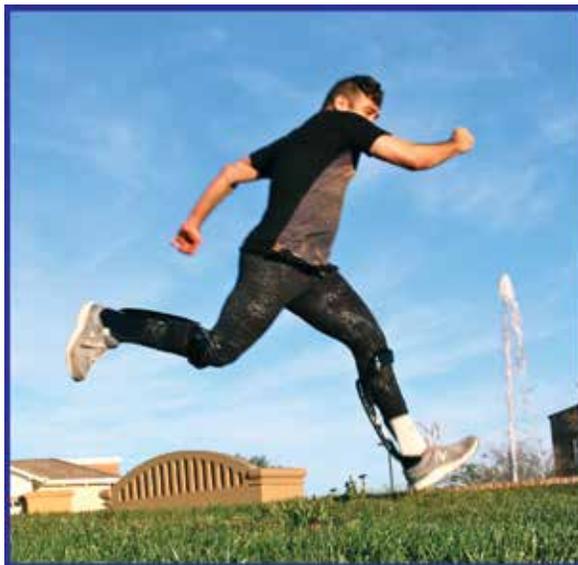
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# Who Am I?

*An educator shares how she maintains her identity throughout work-life transitions*

BY LESLIE KRONGOLD, Ed.D.



Podcasting allows Leslie Krongold to work on her own schedule.

**A**lthough I experience a range of physical symptoms due to the multi-systemic nature of myotonic dystrophy (DM), perhaps some of the emotional issues are the most difficult to deal with. Topping that list is my identity. Who I am, for much of my adult life, centered around my career.

When I was diagnosed in my mid-30s I didn't know what to expect. I had fatigue and digestive difficulties, but nothing severe enough to curtail my work life. A few years later, I realized my work situation was too demanding — I traveled 25 percent of the time, did presentations at conferences and was grooming myself to manage a team. Each day I

would come home and crash on the sofa. What I couldn't accomplish at the office seeped into evening and weekend time. I had some minor health issue every other week. I had to make a change after an ER visit left me weakened and exhausted.

I left my full-time job to join what is now called the "gig economy." Gradually, my stress level stabilized despite a diminished salary. There was still the worry of finding each project, and no work situation is without politics, but I was healthier.

In my mid-40s, I enrolled in a weekend doctoral program. Everyone else in the cohort worked fulltime, and we went through the program

at a slower pace. I loved the challenge and fantasized about the niche I would cultivate once I graduated. Each year of the five-year program became more taxing — attending morning classes was grueling, defending my dissertation proposal was hellish — but I finished. You can call me Dr. Krongold.

The experience drained me. It was as if I'd run a marathon and collapsed just as I crossed the finish line. Each month went by and my dream of the next steps slowly crumbled. I knew there was no way I could embark on the entrepreneurial project I'd envisioned. I felt lost, aimless and hoped to find something to lift my spirits.

Coincidentally, it was a brochure I found at my MDA Care Center advertising a weekend retreat about loss and transition. I was experiencing physical loss — diminished muscle strength, decreased energy — but the loss of my identity was more vexing. The timing of the retreat was perfect, and the experience provided support for life's next chapter.

I heard many horror stories from fellow DM community members about applying for Social Security Disability Insurance (SSDI) benefits and being rejected. Some gave up, while others hired lawyers to plead their

## More Online

Read stories from around the MDA community — and share your own — on Strongly, the MDA blog. Visit [strongly.mda.org](http://strongly.mda.org) to find personal stories from individuals living with neuromuscular diseases, research news and more. If you're interested in sharing your story on Strongly, email us at [strongly@mdausa.org](mailto:strongly@mdausa.org).

*“I set my goals and timeline to sync with how I feel and what I need. The creative and intellectual challenges of building a listening audience and creating a brand identity are like medicine.”*

— Leslie Krongold, Ed.D.

cases. This next step in my journey was wrought with fear and shame. What would it mean to stop working? Was I really that sick? Some days I felt awful, while other days I could forget I had a progressive neuromuscular disease. I was still ambulatory; would I be perceived as an imposter?

Thankfully, the process was swift and I was approved for SSDI benefits. Yes, I really did have a disease, and I'd made that valiant effort to continue working. But I wondered, What now? Who am I?

As fate would have it, shortly after my “retirement,” I was offered a part-time job at a small patient advocacy organization; the modest compensation didn't conflict

with SSDI benefits. For nearly four years I had the opportunity to — on a small scale — live out my graduate school fantasy of launching support groups and training facilitators around the country. Interacting with my DM community, empowering them to take on this new charge, filled my heart.

The once-small organization grew, gaining staff and board members — and subsequently, more stress. They wanted me to assume more responsibilities, but I couldn't. I realized it was time to move on, and so I did. Would I finally sit back and become a couch potato and eat my well-deserved bon-bons?

I don't think so.



Two years ago I launched a podcast, which I view as an evolution of my support group facilitation work and self-care health treatment. It's a labor of love. I set my goals and timeline to sync with how I feel and what I need. The creative and intellectual challenges of building a listening audience and creating a brand identity are like medicine. No ill side effects since I can bow out on any day to rest.

Although I miss earning a salary, I have a solid grip on my identity. The extrinsic rewards of a paycheck have been swapped with positive feedback from listeners and a gradual increase in audio downloads. I wake each morning — late morning — and have a strong sense of purpose. [Q](#)

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*Leslie Krongold, Ed.D., 56, lives in Alameda, Calif. After 20 years of facilitating support groups and training support group facilitators, she's taken her passion to a podcast and blog at [GlassHalfFull.online](#).*



# Taking the Stage

*Lexi Buckalew uses her graduation to send a message of determination*



*Clockwise from left: Lexi Buckalew poses with the Penn State Berks mascot, receives a heartfelt congratulations from her family, and shows off her personalized graduation cap.*

**W**hen Lexi Buckalew thought about graduating from Penn State Berks, in Reading, Pa., last year, she always envisioned herself walking across the commencement stage. Buckalew, who has congenital muscular dystrophy, has limited mobility and primarily gets around using a wheelchair, so making her dream a reality took some planning.

"I wanted people to see that having a disability doesn't matter," she says. "If you put your mind to something, you can do it."

Buckalew worked with a friend to practice walking up the ramp to the stage. When graduation day arrived, she was excited, but also nervous. However, once she was actually on the stage walking to receive her diploma, she felt the energy of the crowd.

"You didn't even get to hear my minor because everyone was screaming," says Buckalew. "I was proud of myself, but I also felt relief and satisfaction; it was joyful."

Buckalew, 22, earned her bachelor's degree in marketing and management and is now doing social media marketing for a chiropractic practice. She says walking at her graduation was not just about achieving a personal goal, but also about setting an example for other students with disabilities.

"Originally, I wanted to walk for myself, but earlier in the year we had another student in a wheelchair come in, and it became more for everyone," she says. "I don't want people with disabilities to think they don't have the chance to go to college or to have the future they want. I'm proud that I graduated, and I want to see other people with disabilities graduate." **Q**

## **Bright Futures**

Explore MDA's young adult resources, covering everything from education and employment to independent living, at [mda.org/young-adults](http://mda.org/young-adults).



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Falling  
Difficulty climbing stairs  
Weakness  
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Droopy eyelids  
Slurred speech

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**If you or someone you know has one of these conditions, your help is needed.**

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This study is continuing to enroll patients ages 2 years and older who have a body weight greater than or equal to 10 kg (22 lb) and who have been diagnosed with CMS, including those with certain genetically confirmed defects. The study is being conducted in Atlanta, Baltimore, Boston, Columbus, and Los Angeles. All travel-related costs will be covered for the patient and a companion.

### MuSK-MG Patients

This clinical study, to be conducted in the United States, is now enrolling patients ages 18 years or older and diagnosed with MuSK-MG. All travel-related costs will be covered for the patient and a companion. For study sites, please go to <https://clinicaltrials.gov/ct2/show/NCT03304054>, which will list study sites as they are approved, or contact us as noted in Learn More.

### LEMS Patients

Patients ages 18 years or older with a confirmed diagnosis of LEMS may be eligible to enroll in the Expanded Access Program (EAP).

### Learn More

For more information about our clinical studies in **CMS** or **LEMS**, contact Catalyst at [EAP@catalystpharma.com](mailto:EAP@catalystpharma.com) or call **1-844-347-3277**. For more information about **MuSK-MG** contact Catalyst at [MuSKMG@catalystpharma.com](mailto:MuSKMG@catalystpharma.com) or call **1-844-347-3277**. More information about our trials is also available at [www.catalystpharma.com](http://www.catalystpharma.com).

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