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MDA is committed to transforming the lives of people affected by muscular dystrophy, ALS and related neuromuscular diseases through innovations in science and innovations in care. Since our inception, MDA has committed more than \$1 billion to accelerate the discovery of therapies and cures. MDA supports the largest network of multidisciplinary clinics providing best-in-class care at more than 150 of the nation's top medical institutions and serves the community through MDA Summer Camp, the Resource Center and educational conferences, events and materials for families and healthcare providers.

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A Look Back at an Eventful Year

Welcome to our first issue of *Quest* in 2019! This is a great time to reflect on the past year, which was an exciting one at MDA. Right now, we are living through unprecedented times in neuromuscular disease. Our work, across all of MDA, is paving a new path forward in terms of understanding neuromuscular disease and enabling life-changing innovations in research, treatment and care.

In 2018, many of our advocacy and research efforts came to fruition with critical impact:

- Spinal muscular atrophy (SMA) was added to the list of disorders recommended for newborn screening.
- MDA launched the neuroMuscular ObserVational Research (MOVR) Data Hub.
- The U.S. Food and Drug Administration (FDA) accepted an application for the first-ever gene therapy treatment for a neuromuscular disease.

We began 2018 by opening a dialog with key members of our community — families and neurologists. Learning from this important research led to two landmark reports:

- ONEVoice describes the needs of adults with neuromuscular disease, their caregivers and family members.
- Understanding Neuromuscular Disease Care, created with the IQVIA Institute, explores how genetic testing and precision medicine will alter the course of neuromuscular disease within the next decade.

In addition, in 2018 we doubled down on our mission to transform lives through innovations in science and care and saw significant achievements on both fronts:

- We now have 170+ active research grants covering 25 diseases.
- We launched new educational seminars for families and healthcare professionals.
- Our Resource Center responded to more than 20,000 inquiries.
- We made the MDA Summer Camp program even better by offering choices about where kids can attend.

As we focus our attention on the work ahead in 2019, we will continue to engage and unite our community to bring better outcomes, advanced treatments and more services to MDA families, caregivers and the neuromuscular medical community.

We are so grateful and inspired by our Care Center teams, sponsors, volunteers and donors for their unwavering support of our important mission. Thank you!

Sincerely,



Lynn O'Connor Vos
President and CEO
Muscular Dystrophy Association



Lynn O'Connor Vos

THANK YOU, READERS

Quest readers gave us great feedback in our 2018 Reader Survey. Thank you to everybody who completed the survey; your responses help shape the content we provide in *Quest*.

We'd like to offer special congratulations to Gail, of Lancaster, Pa., who won the survey drawing for a \$100 Visa gift card. Readers like Gail help us make *Quest* a valuable resource for the MDA community.

Here are a few survey results:
96% of readers consider *Quest* a valuable benefit of being part of MDA.
92% of readers live with a neuromuscular disease or have a family member living with one.
81% of readers share what they learn in *Quest* with family members, caregivers and others.



Issue 1, 2019

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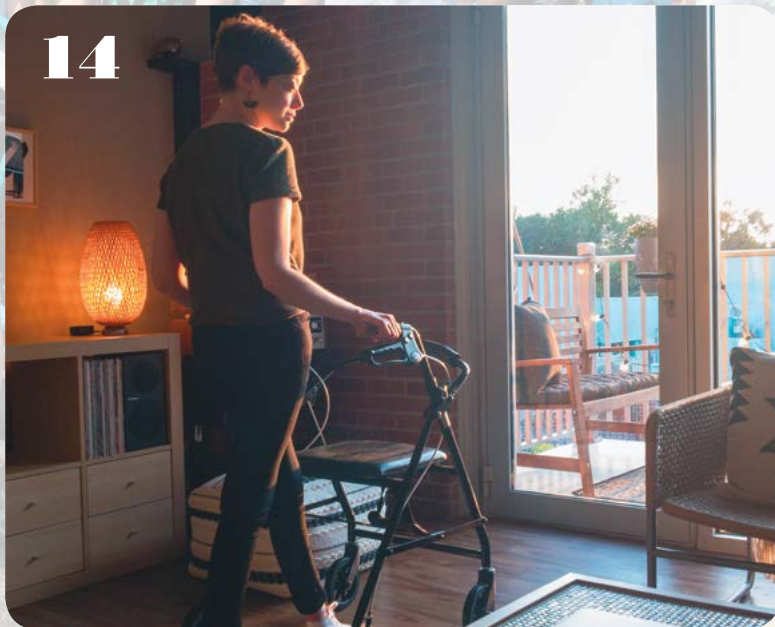
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Here's what you need to know before you visit the emergency department.



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MORE ONLINE

Get the Facts on DMD
In February, MDA released a new DMD Fact Sheet that families can share with relatives, teachers and home and childcare providers. The sheet includes an overview of DMD symptoms, treatment options and a glossary of terms. MDA strives to provide our families with everyday knowledge and support. These efforts include new MDA Engage educational events (learn more on page 4), as well as handy materials like the DMD Fact Sheet.

Visit strongly.mda.org and search for "DMD Fact Sheet."

MDA-027



The National ALS Registry: Get The Facts

The National Amyotrophic Lateral Sclerosis (ALS) Registry enables persons with ALS to fight back and help defeat ALS (Lou Gehrig's Disease). By signing up, being counted, and answering brief questions about your disease, you can help researchers find answers to critical questions.

Learn more at www.cdc.gov/als or (800) 232-4636



Who can sign-up?

Anyone with ALS

No computer? Don't worry! A family member, caregiver or friend with a computer can help you. You can also contact your local ALSA chapter or use the computer at your public library



What do I need?

- A computer with an internet connection
- 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



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



Do I need to update my information?

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



YOU JOINING



More information for research

A better understanding of ALS

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

Education in Your Community

At MDA, we believe in the power of community and the importance of building relationships with families going through similar experiences. We invite individuals living with a neuromuscular disease, as well as caregivers and loved ones, to attend MDA Engage educational events taking place across the country.

MDA Engage Community Education Seminars and Disease-Specific Symposia are one-day events empowering individuals and families with knowledge and resources. Experts in the field and individuals living with neuromuscular disease share information on topics such as best practices in clinical care, research and clinical trials, genetic information, and more. All MDA Engage events include dedicated social time to give families the opportunity to connect.

In 2019, MDA will host 18 Community Education Seminars and eight Disease-Specific Symposia in locations across the country. We hope you will join us at one.



Left and here: An MDA Engage Community Education Seminar in Minnesota

UPCOMING MDA ENGAGE EVENTS

- **April 6, 2019** MDA Engage Community Education Seminar, San Francisco, CA
- **April 12, 2019** MDA Engage SMA Symposium, Orlando, FL
- **May 17, 2019** MDA Engage Community Education Seminar, Salt Lake City, UT
- **May 18, 2019** MDA Engage ALS Symposium, Quincy, MA
- **May 18, 2019** MDA Engage LGMD Symposium, Richmond, VA
- **June 8, 2019** MDA Engage Community Education Seminar, New York, NY

*Additional events and locations are being planned. Check mda.org/care/mda-engage for the latest list.



Presenters at an MDA Engage ALS Symposium

Find an Event

For a complete list of MDA Engage events and to register, visit mda.org/care/mda-engage.

“[I was] glad to meet other people in similar situations and discuss issues and solutions related to neuromuscular disease.” –MDA Engage Community Education Seminar participant in Augusta, GA



Help Fight Duchenne Muscular Dystrophy through Clinical Research

Researchers in your area are enrolling boys with Duchenne muscular dystrophy (DMD) caused by a nonsense mutation into a research study of an investigational drug to see if it can slow disease progression.

Key Inclusion Criteria:

- Male age 5 years or older
- Diagnosed with nonsense mutation DMD confirmed by medical history and genotyping
- Able to stand, walk, climb, and descend stairs

Taking corticosteroid treatment for DMD for at least 12 months

For more information about this study, please contact:

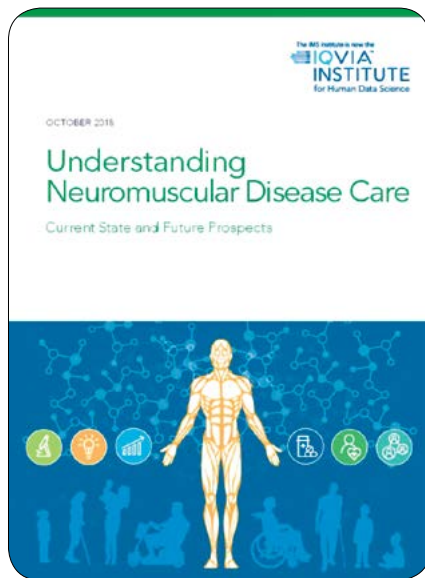
www.dmdstudy041.com



progress *now*

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

MDA Research in Action



This report concludes that recent technological advancements could radically alter the course of neuromuscular disease.

Transforming Diagnosis and Care in the Next Decade

MDA-funded report highlights current gaps in care and opportunities to accelerate therapy development


Neuromuscular diseases encompass a broad group of disorders that are individually rare but collectively impact an estimated 250,000 people in the United States. The diagnostic odyssey often takes more than a year, although improvements in the speed, price and comprehensiveness of available genetic testing are accelerating the process. Currently, treatment options for these diseases are limited. But the financial impact is staggering; costs related to neuromuscular diseases exceed \$46 billion dollars annually.

A new report, *Understanding Neuromuscular Disease Care*, funded by MDA and released by the IQVIA Institute for Human Data Science, shows

that advancements in genetic testing and precision medicine have the potential to radically alter the course of neuromuscular disease within the next decade.

The report illuminates the role that newborn screening, genetic testing and therapeutic interventions that target the root cause of disease will play in revolutionizing treatment and care and accelerating the development of new therapies for neuromuscular diseases. Insights gained from a survey of healthcare professionals focused on the care of individuals with neuromuscular disease are also included.

MDA has a long history of investment in all stages of research that has resulted in the discovery of genes that cause neuromuscular diseases, understanding of underlying disease mechanisms and development of rational therapies. As the umbrella organization for more than 40 neuromuscular diseases, and with the advent of our comprehensive data hub, MOVR, MDA continues to drive the advancement of research, new technologies and care strategies to galvanize both the industry and research arenas to pioneer better care and more cures. MDA has high expectations for advancing patient care and bringing disease-modifying therapies across neuromuscular diseases to patients and their families.

 **Read the full report at [iqvia.com/institute/reports/understanding-neuromuscular-disease-care](https://www.iqvia.com/institute/reports/understanding-neuromuscular-disease-care).**

Amyotrophic lateral sclerosis (ALS)

BHV-0223 Under FDA Review

Under-the-tongue formulation of riluzole could help individuals with swallowing difficulties

In November, Biohaven Pharmaceuticals announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) to review BHV-0223, a sublingual (dissolvable under the tongue) formulation of riluzole for the treatment of individuals living with ALS.

If approved, BHV-0223 would be the first formulation of riluzole to be approved by the FDA that does not require swallowing. Riluzole is currently the only FDA-approved treatment for ALS that is shown to extend tracheostomy-free survival.

The tablet would dissolve under the tongue within seconds and be absorbed into the bloodstream, serving as an important delivery alternative for people who have difficulty swallowing or cannot swallow.

Many people with ALS develop weakness in the face and throat muscles that can cause problems with swallowing, chewing, drinking and speaking. This weakness can lead to choking and aspiration, as well as difficulties with taking medications in tablet form.

Biohaven's NDA contains data from multiple trials of BHV-0223 that showed a bioequivalence with riluzole oral tablets, meaning that BHV-0223 produced the same effect and was used by the body in a similar way as riluzole. In addition, BHV-0223 has shown acceptable safety and tolerability profiles.



Read more about the development of BHV-0223 at [biohavenpharma.com](https://www.biohavenpharma.com).



Becker muscular dystrophy (BMD)

Biomarker and Outcomes Study

Researchers seek participants to provide information on their disease progression

Researchers at the State University of New York (SUNY) Binghamton's School of Pharmacy and Pharmaceutical Sciences are seeking individuals with BMD to participate in the Becker Muscular Dystrophy Biomarker and Patient-Reported Outcomes Study. The purpose is to learn more about disease progression, with the hope that information gathered will enable future biomarker-focused clinical trials of new therapeutic agents in BMD.

To participate, individuals must be age 6 or older, weigh more than 30 pounds and have a BMD diagnosis.

This is a remote study, meaning that participants will not be asked to visit a clinical site for medical evaluation. For the biomarker portion of the study, participants may schedule a visit

from a phlebotomist in their homes or another location. The

phlebotomist will collect small samples of blood and urine to be used for this biomarker analysis.

Additionally, participants will be sent questionnaires, either electronically or via mail, that will ask about their physical, emotional and social well-being as it relates to their BMD diagnosis. The questionnaires should take less than 30 minutes to complete.



For additional information, or if you are interested in participating, contact clinical coordinator Marissa Barbieri at [607-777-5970](tel:607-777-5970) or barbieri@binghamton.edu.



Charcot-Marie-Tooth disease (CMT)

Encouraging Results in CMT1A Study

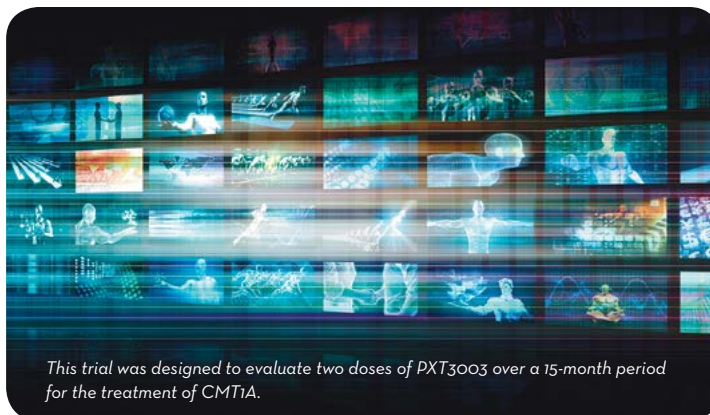
In phase 3 clinical trial, PXT3003 was safe and effective

French pharmaceutical company Pharnext SA announced encouraging topline results from its phase 3 clinical trial (PLEO-CMT) for the treatment of CMT1A.

PLEO-CMT was a 15-month, double-blind study that assessed the efficacy and safety of PXT3003 compared to placebo for the treatment of individuals with mild to moderate CMT1A. The study included 323 participants ages 16 to 65 years old.

The primary endpoint was the Overall Neuropathy Limitation Scale (ONLS), which measures disability. Pharnext reported that a reduction of 0.3 point on this scale was determined to be meaningful.

Compared with placebo, a mean reduction of 0.4 on the ONLS was observed in the trial group receiving the highest dose



This trial was designed to evaluate two doses of PXT3003 over a 15-month period for the treatment of CMT1A.

of PXT3003. Additionally, PXT3003 was safe, well tolerated and showed a similar safety profile as seen in an earlier phase 2 study.

This study provided the first evidence of PXT3003 contributing to meaningful improvement of CMT1A. Based on these results, Pharnext intends to file for market approval for the drug in the United States and Europe.

 **Read more about the PLEO-CMT trial of PXT3003 at pharnext.com/en.**

Duchenne muscular dystrophy (DMD)

DMD Gene Therapy

Sarepta trial results update



At the 23rd International Congress of the World Muscle Society in Argentina, principal investigator Jerry Mendell, M.D., of Nationwide Children's Hospital in Columbus, Ohio, shared additional data relating to Sarepta Therapeutics' AAVrh74.MHCK7.micro-Dystrophin gene therapy program for DMD.

Mendell previously had presented encouraging preliminary results for the first three

clinical trial participants. In his update, he shared micro-dystrophin results for the fourth study participant and reported positive functional results for all four patients in the trial. These participants showed improvement in several metrics, including time to rise, time to climb four stairs and time to walk 100 meters. Additionally, all four patients showed robust expression of the transduced micro-dystrophin gene. No serious adverse effects were observed.


Mendell, in collaboration with Louise Rodino-Klapac,

Ph.D., developed AAVrh74.MHCK7 specifically for DMD. The drug is designed to address the genetic cause of the disease via the delivery of highly miniaturized "micro-dystrophin" replacement genes that enable production of a functional protein to substitute for the dystrophin missing in people with DMD.

It is important to remember that while these results are encouraging, they need to be confirmed by a controlled trial.

Of note: Two other gene therapy trials are underway for DMD. Pfizer currently

is testing PF-06939926 in a phase 1 trial, and Solid Biosciences is testing SGT-001 in a phase 1/2 trial. Interim results have not yet been made available for either study.

 **For more information, visit clinicaltrials.gov. Enter NCT03375164 in the "Other Terms" search box for Sarepta's AAVrh74.MHCK7.micro-Dystrophin trial. Enter NCT03362502 to learn about Pfizer's trial, or enter NCT03368742 to read about Solid Biosciences' trial.**

Duchenne muscular dystrophy (DMD)

CAP-1002 Trial Seeks Participants

Study will assess safety and efficacy of Capricor DMD drug



Researchers at Capricor Therapeutics are looking for children and adults with DMD to participate in a phase 2 clinical trial to evaluate the safety and efficacy of CAP-1002.

An investigational cell therapy, CAP-1002 is designed to slow DMD disease progression in heart and skeletal muscle by modulating immune system activity, reducing inflammation and fibrosis (scarring), and stimulating muscle regeneration.

Trial participants will randomly be assigned to groups that will receive either CAP-1002 or placebo, which will be delivered via an IV infusion during site visits at day one and months three, six and nine.

Total study duration for each patient will be about one year, and requires nine clinic visits. At each visit, participants will undergo various evaluations and assessments, including performance of the upper limb (PUL), pulmonary function testing, the North Star Ambulatory Assessment (ambulatory subjects only), strength testing, a cardiac MRI, a physical exam, a 12-lead ECG and clinical laboratory testing.

Boys ages 10 and older and men who are ambulatory (able to walk) or non-ambulatory may be eligible to participate. In addition to meeting other eligibility criteria, participants must have:

- > Reduced upper arm strength
- > An impaired ability to walk/run
- > An established treatment regimen for systemic glucocorticoids of at least 12 months, and at least six months prior to the study at a stable dose except for weight-based or toxicity-related adjustments

Travel support for the participant and one travel companion for each visit is available.

Throughout the study, participants will continue to see their regular doctor for routine care.



To learn more or to inquire about participation, contact

Brian Fedor at

310-358-3201 or
HOPE-2@capricor.com.

For more information about the study, including a list of active trial sites, visit clinicaltrials.gov and enter **NCT03406780** in the "Other Terms" search box.

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Duchenne muscular dystrophy (DMD)



Clinical Trial Alert: PolarisDMD

Researchers seek boys with DMD to participate in phase 3 study

Researchers are looking for boys to participate in PolarisDMD, a phase 3 study to evaluate the safety and efficacy of edasalonexent, under development by Catabasis for the treatment of DMD.

It is thought that edasalonexent may slow down progression of skeletal and cardiac muscle disease in DMD by inhibiting the activity of a protein complex called NF-kB. NF-kB helps to activate the immune system, which leads to inflammation associated with muscle weakness and DMD disease progression.

Participants will be randomly assigned to groups, which will receive either edasalonexent or placebo.

Total study duration for each study participant will be about one year, with clinic visits every three months. At each visit, efficacy will be evaluated by the performance of the North Star Ambulatory Assessment, timed function tests and cardiac and bone assessments. Throughout the study, participants will continue to see their regular doctor for routine care.

In order to be eligible to participate, boys must:

- > Be between the ages of 4 and 7
- > Be able to walk
- > Have not used corticosteroids for 24 weeks prior to the study's start
- > Have a confirmed genetic diagnosis of DMD
- > Meet additional study criteria

Travel support is available for each clinic visit.

 For more information, visit clinicaltrials.gov and enter NCT03703882 in the "Other Terms" search box, or visit catabasis.com. To inquire about participation, contact Maria C. Mancini at DMDtrials@catabasis.com.

DMD Mobility Survey


Study will help researchers capture patient perspective in clinical trials

Researchers at the University of California Davis Neuromuscular Research Center are recruiting up to 1,000 individuals with DMD ages 5 and older, and/or their parents/guardians, to complete an online survey about movement and daily living abilities. The Duchenne Muscular Dystrophy Life-Time Mobility Scale (DMD-LMS) is a set of questions designed to help assess how easy or hard it is for individuals with DMD to complete specific movements and activities in their day-to-day life.

The goal is to provide information that can help researchers better describe ways that new therapies might benefit patients at home and in the community. Once validated, the survey could serve as a critical new outcome measure for clinical trials.

Participants will spend approximately 30 minutes answering questions about how easy or hard a movement or task (such as walking in their community, standing up from a chair or drinking from a glass) would be if the person with DMD had to complete it within the past seven days without help. Taken together, a person's responses describe how strong they are and how well they are able to perform day-to-day tasks.

Participants will complete the questionnaire in three stages: at enrollment, one month later and one year later. Parents of children ages 5 to 17 will answer questions on behalf of their children. Adolescents ages 11 to 17 with DMD will answer the same questions as their parents or guardians. Adults with DMD will answer the questions without their parents/guardians.

 For more information, and to access the study link, visit redcap.ucdmc.ucdavis.edu/redcap/surveys/index.php?s=8APYH7X7MT.



Questionnaires about mobility, when used along with clinical strength and function testing, can provide valuable information about how changes in strength affect people with DMD in day-to-day life.

Myasthenia Gravis (MG)

Administered by self-injection, zilucoplan could represent a viable, effective at-home therapy for people with generalized MG.



Results for Zilucoplan

Phase 3 trial design is underway

In December, Ra Pharmaceuticals announced positive results from a phase 2 clinical trial designed to evaluate zilucoplan (RA101495) for the treatment of generalized myasthenia gravis (gMG). Participants who received the drug showed significant reductions in both measured endpoints, the Quantitative Myasthenia Gravis (QMG) score and the Myasthenia Gravis – Activities of Daily Living (MG-ADL) score.

Zilucoplan is a synthetic peptide (a short chain of amino acids that acts like a protein) that binds to the complement component 5 (C5) protein, inhibiting its function and preventing breakdown of the neuromuscular junction.

In the trial, 44 participants received a daily injection of either 0.3 mg/kg zilucoplan, 0.1 mg/kg zilucoplan or a placebo over the course of 12 weeks. Individuals who received the higher dose achieved a mean reduction (from baseline) of 6.0 points in the QMG score compared to a reduction of 3.2 points for patients receiving placebo. Additionally, those receiving the higher dose of zilucoplan showed a mean reduction (from baseline) of 3.4 points in the MG-ADL score compared to a 1.1-point reduction for those on placebo.

Based on these results, Ra Pharma has reported it will work with regulatory agencies to approve a phase 3 trial design by end of the first quarter of 2019.



To learn more about this trial, visit clinicaltrials.gov and enter NCT03315130 in the “Other Terms” search box.

LEMS Update

Firdapse, a new drug to treat Lambert-Eaton myasthenic syndrome (LEMS), was approved in November. Learn about this and other updates in “News on LEMS Treatment and Research” on page 32.

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Spinal muscular atrophy (SMA)

Gene therapy addresses the underlying genetic cause of a disease.



Zolgensma Under FDA Review

Under Priority Review, a decision is expected around May 2019

Novartis, the parent company of AveXis Inc., announced that the U.S. Food and Drug Administration (FDA) has accepted the company's Biologics License Application (BLA) under Priority Review for Zolgensma (formerly known as AVXS-101), a gene therapy for the treatment of SMA type 1. Priority Review status requires the FDA to review the application and make a decision on whether to approve Zolgensma within six months (the typical review period is 10 months). A decision is anticipated in May 2019.

Zolgensma is a gene therapy that targets the root cause of SMA by delivering a gene to replace the missing or mutated *survival of motor neuron 1 (SMN1)* gene. The therapy is a one-time infusion.

Treatment with Zolgensma in a phase 1 clinical trial of infants

with SMA type 1 was associated with an increased survival rate compared to the normal course of the disease, and the achievement and maintenance of motor milestones that these infants normally would not be expected to achieve.

The most commonly observed side effect of Zolgensma was elevated liver enzymes.

Zolgensma currently is in phase 3 clinical trials for SMA type 1 and is also being tested in a phase 1 trial for SMA type 2. Additionally, Zolgensma is being tested in a phase 3 trial for presymptomatic newborns.

For information regarding eligibility criteria for currently enrolling or planned clinical trials, view the listings on clinicaltrials.gov or studysmanow.com, or contact medinfo@avexis.com.

Encouraging Findings for Risdiplam

FIREFISH and SUNFISH studies report interim results

Genentech has reported encouraging interim results from FIREFISH and SUNFISH, two studies investigating risdiplam (RG7916) for the treatment of SMA.

Risdiplam is an investigational drug designed to work by helping the *survival of motor neuron 2 (SMN2)* gene produce more SMN protein. The drug is given by mouth (or g-tube) and distributes widely throughout the body.

Risdiplam's development is part of a collaboration between Genentech, a member of the Roche Group, PTC Therapeutics and the SMA Foundation.

Preliminary findings from part one of the phase 2/3 FIREFISH study show that infants with SMA type 1 are meeting developmental milestones, including sitting without support. Data from the phase 2/3 SUNFISH study show improvements in motor function in individuals living with SMA types 2 and 3.

Read more about risdiplam at pioneeringhealthcare.com/sma.



Trial data were presented at the 23rd International Annual Congress of the World Muscle Society in Argentina.

ADVERTISEMENT

A close-up photograph of two hands, one from a child and one from an adult, gently planting a small green seedling into dark brown soil. The background is softly blurred, showing more soil and another seedling in the distance.

IMAGENE IF

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Lindsey Baker has lived in six apartments in the last 12 years. With each move, she has learned to adjust her living space to suit her needs.

When neuromuscular disease impacts your ability to perform daily tasks, cultivating problem-solving skills and support can help you live independently in your home

THE INDEPENDENT

mindset

BY BARBARA AND
JIM TWARDOWSKI

After Sandra Young was diagnosed with late-onset Pompe disease at age 52, she thought her life would change and she would no longer be able to do the things she enjoyed.

“It took me a year to realize I am the same person as before. I hit a stumble in the road, and it shouldn’t stop me from doing the things I want to do. I woke up and thought, ‘Girl, you can still do it, you just have to do it differently.’”
—Sandra Young

“While my friends and family were living their lives, I was sitting on the sidelines missing out,” Young says. “It took me a year to realize I am the same person as before. I hit a stumble in the road, and it shouldn’t stop me from doing the things I want to do. I woke up and thought, ‘Girl, you can still do it, you just have to do it differently.’”

What Young discovered after her diagnosis is that independent living is as much a way of thinking as it is a way of accomplishing daily tasks. Independent living is a way of looking at disability and society with the understanding that individuals with disabilities deserve to fully participate

in the world, no matter their physical limitations.

MDA advocates for independent living, and many agencies and organizations help provide the practical support those with disabilities need to live independently in their own homes. These can include state or local government agencies and community-based nonprofits, such as Centers for Independent Living or Aging and Disability

Resource Centers. (See “Navigating Independent Living Services” on page 18.)

The available services vary from one community to the next, but they might include assistance finding accessible housing and personal care attendants (PCAs), independent living skills training, peer counseling or legal aid. Each center is operated independently with funding from public and private sources, and currently, some are faced with reduced funding and higher demand for services.

The MDA Resource Center (**833-ASK-MDA1** or **resourcecenter@mdausa.org**) can help you find the independent living resources



After being diagnosed with Pompe disease, Sandra Young (above and left) moved to a first-floor apartment and found new ways to accomplish household tasks.

and services available to you, but the bottom line is that maintaining independence in your home takes initiative, problem-solving skills and the ability to network.

Whether you dream of living in your first apartment or need extra help to stay in a home you own, the solutions for establishing a self-sufficient environment are unique to every individual. Here, experts and those like Young who are working every day to maintain their independence offer some advice.

1. LET KIDS TRY

Independence begins as an idea, and parents play a key role in how their children envision the future.

“Instill in children a belief that they have the capacity to be mature and in charge of their own lives,” says Michileen Oberst, LCSW, a social worker at the MDA Care Center at Stanford University in Palo Alto, Calif. “Allow young adolescents to have more and more responsibility so they develop decision-making skills.”

For many young adults, attending college may lead to living away from their family

for the first time. In addition to the typical adjustments every freshman makes — dealing with homesickness, building a network of friends, managing their academic load — students with neuromuscular diseases must find accessible housing, navigate the college campus and sometimes secure caregiving support.

Carol Lipari, who lives with facioscapulohumeral muscular dystrophy (FSHD) and uses a power wheelchair, moved hundreds of miles from her home in Illinois to attend the University of Arizona. “My mom always encouraged me to do what I wanted and find my own way,” she says.

Living in a dorm and later an apartment meant that starting at age 18, Lipari was hiring and supervising her own PCAs.

“I think it is a little harder to stand up for yourself and self-advocate when you’re that young,” Lipari says. “But I wanted to be as independent as possible, and that meant knowing how to manage those professional relationships and keep them strong — it’s a good skill to have.”

Today, Lipari lives in a high-rise apartment in

“I wanted to be as independent as possible, and that meant knowing how to manage those professional relationships [with PCAs] and keep them strong — it’s a good skill to have.”

—Carol Lipari

Chicago and works as a resource coordinator for MDA. She has three PCAs who alternate coming to her apartment every morning and evening to assist her with bathing, meal prep and household chores.

When she isn’t working, Lipari goes to museums, Broadway shows and rooftop bars with friends. She also writes and performs standup

Carol Lipari (below) loves living in the middle of everything in downtown Chicago. Her apartment search centered on finding a bathroom (lower left) that would accommodate her power wheelchair with ample dimensions, open space under the sink and grab bars.



NAVIGATING INDEPENDENT LIVING SERVICES

The maze of services designed to help people with disabilities live independently varies in every area. Typically, recipients of potential services or funding must fit specific criteria based on their age, disability, military service, county, income level and more.

A good place to begin a search is with an Aging and Disability Resource Center (ADRC). These organizations – established in many communities across the country – are a gateway to a broad range of services for people at all income levels. An Area Agency on Aging (AAA) assists older adults who wish to remain in their homes. They might coordinate services such as housekeeping or meal delivery. Find your local ADRC and AAA by calling 800-677-1116 or visiting eldercare.gov.

Centers for Independent Living (CILs) are an often-underutilized resource that assists people of all ages. These community-based, cross-disability nonprofit agencies are run by people who have disabilities, and they provide services to support independent living, including skills training, peer counseling and information on local transportation services for individuals with disabilities. To find CILs in your state, visit ilru.org and click on “Directory of CILs and Associations.”

Keep in mind, “Aging and Disability Resource Center,” “Area Agency on Aging,” and “Center for Independent Living” are general terms. Each organization is operated independently, and the actual names of the organizations in your community may be different.

A handy list of additional resources – covering a range of needs from assistive technology to housing – can be found at acl.gov/help.

comedy around the Windy City. “For me, seeking out these opportunities comes naturally,” she says. “Don’t be afraid to try something new.”

2. GET HELP

In her job as a social worker, Oberst sees a variety of ways people living with neuromuscular disease attain the help they need.

Living on the West Coast, where housing prices are soaring and PCAs are in high demand, Oberst has clients who have switched from hiring a team of PCAs

to a live-in caregiver. Providing a rent-free home for the caregiver is one way to substantially reduce the cost of full-time care.

Not everyone needs a full-time caregiver, but many people with neuromuscular disease could use assistance with certain tasks. Oberst finds local senior centers often have a pool of volunteers. Churches and other religious institutions also might have volunteers willing to lend a hand.

“Until you connect up with what’s in your community, you don’t know what you will find,” Oberst says. Identify your specific needs, like shoveling snow, running errands or cooking meals. Once you are armed with a list of needs, contact the directors of local organizations and ask for the help you need. “Take the initiative to sit down and meet. Sometimes little miracles work out,” Oberst says.

Young, a New Jersey resident, has also explored the resources in her community that help with daily tasks. When doing laundry became too taxing, she signed up for a weekly service that picks it up and drops it off at her home. She orders groceries online from Walmart and picks them up at a nearby store without getting out of her car. Young enjoys baking, but weak shoulders make removing food from the oven difficult, so she calls her upstairs neighbor for assistance. Of course, she bakes him a pie to show her gratitude.

3. THINK OUTSIDE THE BOX

Lindsey Baker, healthcare communications manager at

MDA, was trained as a journalist, and the creativity she’s cultivated throughout her life helps her live independently with Charcot-Marie-Tooth disease (CMT).

CMT has weakened her extremities, making it difficult to walk and use her hands. In the last 12 years, she has lived in six apartments, and with each move, she has had to adjust her living space to suit her needs and find workarounds for her changing abilities.

Baker’s current apartment in Omaha, Neb., is not accessible, but it’s her favorite, with high ceilings and a sunny balcony. Before moving in, Baker visited the unit eight times, brainstorming on how to adapt the standard shower so it would work for her. After consulting with her father, a retired architect, and clearing her changes with the building management, she had a special shower bench designed and installed, along with a thin grab bar because her hand contractures don’t allow for a wide grip.

A minimalist, Baker finds her compact apartment with its short walk between the bedroom and bathroom a good fit. She keeps her belongings neatly arranged and off the floor to avoid tripping. To control the lights and stereo, she purchased an Amazon Echo. In the kitchen, her workhorse appliance is a food processor/chopper/blender with only one big button to push.

Two years ago, Baker stopped driving. Now, she hires someone to take her out for errands once a week. Her lack of a pincher grip makes picking things up difficult. When she drops a piece of



When you're armed with initiative and problem-solving skills, you can build a network that helps you live independently in your home.

Lindsey Baker (above) enjoys her apartment's sunny balcony. She adapted the bathroom with a shower bench and thin grab bar, and she's learned which tools are especially handy in the kitchen.

paper on the floor, she uses the sticky side of a Post-it to pick it up.

4. BUILD YOUR NETWORK

When you're armed with initiative and problem-solving skills, you can build a network that helps you live independently in your home.

For example, Young, who moved into an accessible first floor garden apartment when the stairs in her house became too difficult, works from home as national manager of foundations for MDA and is an

expert at research. When she needed an adjustable bed, she began searching for assistance and found Patient Services, Inc. (patientservicesinc.org), an advocacy organization for certain chronic diseases, which provided funding for the bed.

MDA Care Centers (mda.org/care/mda-care-centers) are also an important part of your network. Team members can help problem-solve for obtaining equipment and meeting other needs.

"I'm the go-to person in my family and among my

friends," Young says. "They all say, 'Ask Sandra. She'll know what to do.'"

Though she exudes confidence, Young acknowledges that remaining independent feels like a battle sometimes. Fortunately, it's one you don't have to fight alone. **Q**

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.

More Online

Read more of the important lessons Lindsey Baker has learned about living independently on the Strongly blog. Go to strongly.mda.org and search for "Living Independently with CMT."

Think outside the cubicle to find solutions to common accessibility challenges in the office

BY ELIZABETH MILLARD



MAKING THE workplace ACCESSIBLE

A decorative graphic consisting of several overlapping, wavy dashed lines in light green and grey. Scattered throughout the graphic are various small icons: a lightbulb, a square, a triangle, a diamond, and a circle.

When Josh Moser transitioned from college to a career 10 years ago, having Duchenne muscular dystrophy (DMD) was a concern he wasn't quite sure how to handle. He felt nervous talking about his needs, he recalls, worried that it might sabotage his job prospects.

Fortunately, the financial services firm where he was temping appreciated his work and asked him to apply for a full-time position as a processing associate. After he accepted the offer, the company worked with him to make sure he could perform his job duties effectively.

For instance, one of the first accommodations his company made was raising his desk space so his wheelchair could fit underneath. They installed a speech-to-text program on his

computer to help him avoid fatigue from typing. The company also engaged a healthcare provider for a contracted personal care assistant who works alongside Moser to assist with numerous tasks, such as putting on a headset for phone calls, taking restroom breaks and performing other duties.

"It's nice to have the assistant because it helps me to focus on the job," Moser says. "I feel fortunate to be in a supportive environment where there's a personal care plan."



Josh Moser, who works at a financial services firm, has a workspace tailored to his needs.

TOP TIPS

Moser, who was promoted to a client process health associate, feels confident that as his needs change, his company will continue to make sure the workplace is accessible to him. For example, increasing muscle weakness in his arms has made it harder to feed himself, so Moser's personal care assistant added that task to his lunchtime routine.

Adaptability and tailoring solutions to individual needs is crucial for helping individuals with neuromuscular disease thrive in an office environment, says Rafael Salazar, OTR/L, founder of Rehab U Practice Solutions, an education and consulting firm.

"The focus for every company should be on making the workplace less stressful," he says. "For those with neuromuscular disease, that just requires different approaches."

Although specific accommodations and assistive technology will vary for each person, these tips can help employees and their companies work together to find solutions for workplace accessibility challenges:

- Ergonomic assessments are a good place to start. Occupational therapists are adept at evaluating a workspace, as well as other office areas, and coming up with recommendations, Salazar says. They have expertise in knowing how changes like desk heights, specialized keyboards and voice activation can help employees with physical challenges, he notes.

- Consider a breadth of simple accommodations. There are numerous adaptive equipment options, and sometimes, just switching to a different keyboard can be a huge advantage, Salazar says. For instance, a keyboard might be split into two sections, require less pressure on the keys or be compatible with a communication device.
- Work with IT for computer control panel access. Companies often don't allow employees to make changes to basic control panel functions on company computers, but some changes can improve accessibility, according to Holly Cohen, OTR, program manager of assistive technology at NYU Langone's Rusk Rehabilitation. For instance, changing the mouse click speed to a slower setting might allow someone with muscle weakness in the hands or arms to take more time when using the mouse. Looping the IT department into the discussion early on can be useful for making control panel changes or adding assistive technology.
- Focus on what an employee can do. One aspect of workplace accessibility that Cohen likes to drive home is thinking about what an employee is able to do, not what he or she can't do. "Even if the person can only move one finger, there are many adaptive equipment options that let him or her control everything with that one finger," she says. "Focusing on limitations can make the process feel counterproductive."

WHAT IF YOU HAVE TO EVACUATE?

While considering desk configuration, software and door access are helpful for your everyday work, don't forget to plan for emergencies.

Lindsey Baker, a healthcare communications manager at MDA who lives with Charcot-Marie-Tooth disease (CMT), recalls a previous job where she worked in a high-rise building. When she asked about the office plan for an evacuation, she was told that everyone else could leave via the stairway, and they would inform first responders that she was left behind. When she balked at that solution, they suggested she crawl down the stairs instead.

"People tend to think that if they meet ADA requirements for a workplace, they're good to go," Baker says. "But you have to think about evacuation in advance. You can't plan for a fire when the building is burning."

Schedule a meeting with HR to talk specifically about an evacuation plan, advises Kelli Reiling, OTD, OTR/L, clinical assistant professor of occupational therapy education at the University of Kansas Medical Center. Focusing on just that topic will keep the plan from getting pushed off into the "we'll discuss it someday" category. She also suggests being proactive – rather than asking what the company can put in place, research some options so you have a starting point for the conversation. Contact your local fire department as well, as they may have ideas that neither you nor the HR reps may have considered.

"Much as you would with other types of accommodations, the more options you can bring to a discussion, the better," Reiling says. "That creates an atmosphere of working together on a solution."

- Think beyond the work-space. Do the office doors open in or out? Would they be difficult to push for someone using a walker? Are the bathrooms ADA compliant? Are there obstacles along the path from an individual's office to a meeting room or break room? These are the kinds of questions to ask for a truly accessible workplace, says Kelli Reiling, OTD, OTR/L, clinical assistant professor of occupational therapy education at the University of Kansas Medical Center. Even if an office meets ADA

requirements, that doesn't mean it automatically works for someone with a neuromuscular disease. "Employers should be coming up with strategies that work for the employee's entire day," she says. "That means at a work station, but also during lunch, going to the bathroom, coming in from the parking lot, or going to a meeting. You need to think of the full breadth of activities that happen so an employee feels comfortable and doesn't have to keep coming up with work-arounds."

START THE CONVERSATION

The most useful first step to better accessibility is to simply begin the conversation. Contrary to his fears when he first joined the workforce, Moser has learned that employers are willing to listen when he voices his needs. He advises others with neuromuscular disease to advocate for themselves.

"It can be hard to talk about, because you don't want to stand out as different," he says. "But at the same time, if there's something that can make you better at your job, your company will want to know."

Some companies may have resources you might not have known about. For example, the financial services firm where Moser works has an accessibility team, according to company spokesperson Alyssa Thornton.

"This team helps us stay abreast of and implement accessibility enhancements to ensure access and functionality for all," she says. [Q](#)

Elizabeth Millard is a freelance writer in St. Paul, Minn.

Advocacy at Work

MDA is dedicated to advocating for policies and programs that improve the lives of people living with neuromuscular disease in the workplace and beyond. Learn about MDA's advocacy and how you can get involved at mda.org/advocacy.

ADA REQUIREMENTS

The Americans with Disabilities Act requires state and local governments, businesses, and nonprofit organizations to follow certain rules to ensure that individuals with disabilities are treated on an equal basis.

Find resources for learning about and asserting your rights at:

- ada.gov
- adachecklist.org
- adata.org

WHAT COULD SPINRAZA UNLOCK?

The **first** treatment for children and adults with spinal muscular atrophy (SMA)

SPINRAZA was evaluated in a well-controlled study of 126 individuals with later-onset (Types 2 and 3) SMA. The results are supported by an open-label study of 28 individuals with later-onset (Types 2 and 3) SMA, aged 2 to 16 years at first dose. Limitations of the open-label study included differences in dosing compared with the approved regimen and the lack of an untreated group.



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Individuals taking SPINRAZA continued to improve or maintain motor function over nearly 3 years, regardless of their level of function before starting treatment.



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Visit [SPINRAZAevent.com](https://www.spinrazaevent.com) or call **1-888-615-4343** to connect with your local SMA community.

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 respiratory infection, fever, constipation, headache, vomiting, 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.

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

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

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



Lauren
age 20
 Later-onset (Type 3) SMA
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Individual results may vary based on several factors, including severity of disease, initiation of treatment, and duration of therapy.

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

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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 respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome (headache related to the intrathecal procedure).
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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.

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

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 or 1-844-4SPINRAZA (1-844-477-4672).

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BE

prepared

What you need to know before you visit the emergency department

BY DONNA ALBRECHT

When a medical emergency occurs — and the patient is a person with a neuromuscular disease — it's not just getting to the emergency department (ED) quickly that's critical. It's also critical to ensure the ED staff understands the patient's particular needs.

“Many emergency department physicians have a limited background in neuromuscular disease,” states Lisa Wolfe, M.D., associate professor of medicine in the Division of Pulmonary Critical Care and Sleep Medicine at the MDA Care Center at Northwestern University in Chicago. Unfortunately, many people don't discover this potential problem until they've arrived for emergency care.

WHAT'S AN EMERGENCY?

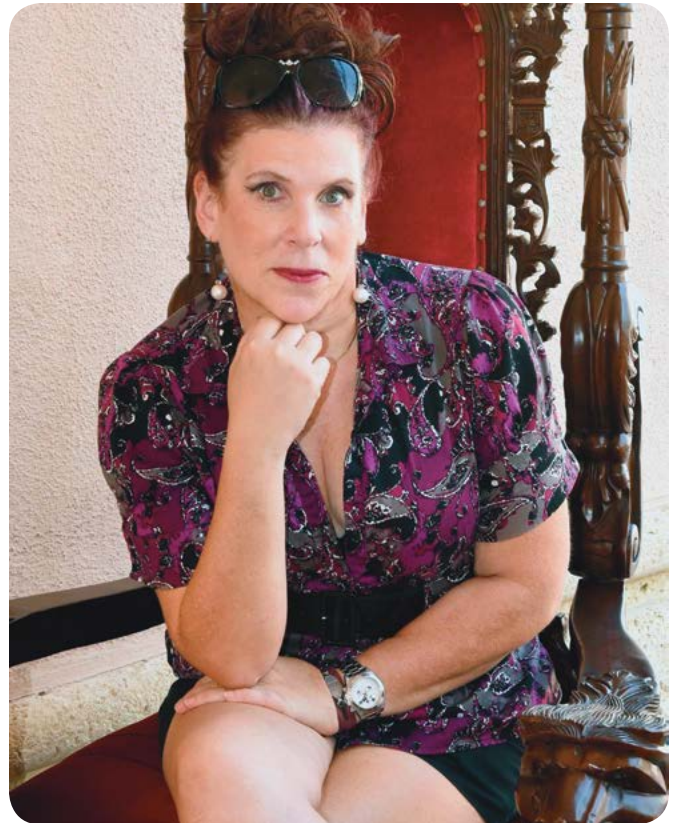
Respiratory problems are the top reason that people with neuromuscular diseases go to the ED. Dr. Wolfe explains that pneumonia can occur on its own, or aspiration of food particles can cause smoldering infection or acute airway blockages needing emergency care.

Other common reasons people with neuromuscular disease visit the ED include:

1. Flu-related complications
2. Trauma from falls or accidents
3. Cardiac issues, such as associated cardiomyopathies
4. Gastrointestinal issues, such as constipation
5. Orthopedic issues, including muscle or joint pain and hip subluxation

EXPECT THE UNEXPECTED

In the event of an emergency, “most people who have neuromuscular conditions go to a community ED first,” says James Naprawa, M.D., a



Frances Kiperman

pediatric emergency medicine physician at UCSF Benioff Children’s Hospital in Oakland, Calif. Because most ED staff have little contact with neuromuscular conditions, they might overlook signs of distress or misinterpret the problem.

“The kids I see are typically adolescents coming in with breathing difficulties,” Dr. Naprawa says. “They tend not to look very distressed because of the weakness in their muscles. They may not be coughing. They can appear surprisingly well.”



RIGHT PLACE, WRONG CARE

Emergency department (ED) doctors and nurses who are unfamiliar with neuromuscular diseases might miss signs of distress or misinterpret them. Here are issues to watch for in the ED:

Appearance: A patient whose muscles are floppy can be subjected to a full spinal workup looking for injuries, especially if they aren’t able to speak

for themselves and aren’t wearing medical alert jewelry.

Communication: Friedreich’s ataxia (FA) can cause slurring of speech, and spinal muscular atrophy (SMA) or amyotrophic lateral sclerosis (ALS) can make speech difficult to understand, leading doctors to unnecessarily test for drug or alcohol abuse.

Respiratory distress: Emergency medical staff’s immediate reaction may be to give oxygen, but if the problem is weak respiratory muscles, the extra oxygen could dangerously suppress breathing. In some cases, an ED physician may want to do a tracheostomy when all that is needed is to provide noninvasive supportive care while the underlying respiratory infection or obstruction is resolved.

Anesthesia: People with certain inherited neuromuscular diseases (Becker muscular dystrophy, Duchenne muscular dystrophy, myasthenia gravis, SMA and central core disease, among others) are at increased risk of a dangerous reaction to anesthesia called malignant hyperthermia. Talk with your healthcare providers about whether you have a specific risk.

“The kids I see are typically adolescents coming in with breathing difficulties. They tend not to look very distressed because of the weakness in their muscles. They may not be coughing. They can appear surprisingly well.”
—Dr. Naprawa

In 2018, Dr. Naprawa co-authored a paper that appeared in *Lancet Neurology* on new care considerations for Duchenne muscular dystrophy (DMD), which led MDA to co-publish the new *Duchenne Guide for Families* (find it at mda.org under Care & Services in MDA’s Resource List for Families). Dr. Naprawa found that working on the paper opened his eyes to the nuances of caring for neuromuscular disease patients in crisis. Now, he is quick to recognize muscle weakness, and he is aware that these patients may be taking steroids to prolong their muscle strength, can have abnormal heart rates and may experience problems that are rare in other patients.

Frances Kiperman, a Florida resident who lives with myasthenia gravis (MG), has dealt with a lot of

misunderstandings in the ED over the years. One involved a physician who thought that her legs looked OK, despite the fact that she could not stand independently. The doctor pushed her wheelchair up to a gurney and told her to get on. As she leaned on the gurney, still using her chair for support, he abruptly pulled the chair back, thinking

he was moving it out of her way. “I managed to punch him on the jaw as I fell to the floor,” she says.

But not all surprises in the ED are bad. Years ago, when my oldest daughter, who had spinal muscular atrophy (SMA), was home from the hospital after having a tracheostomy, the surgical trach became blocked. At the ED,

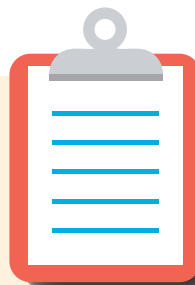
DO IT NOW!

Going to the emergency department (ED) means you or your loved one has a medical situation that can’t wait. Here are three ways to prepare in advance to get ED staff up to speed on your needs as quickly as possible:

- 1. Keep your important medical information in one place.** This should include information on your condition, a list of your medications and a list of your healthcare providers with contact information, including on-call numbers and how to contact key doctors in an emergency. The International Ventilator Users Network has a thorough healthcare form that covers everything from medications and vent settings to typical vital score readings and positioning instructions. Find this resource at ventusers.org/vume/PatientInfo.pdf.
- 2. Practice with your doctor.** At your next appointment with your care team, ask what kinds of situations might come up in the ED and what information an ED physician would need when treating you. Find out where your doctors have hospital privileges so you can go there if you have a choice.
- 3. Have an advance directive.** An advance directive, or healthcare directive, lets your medical providers and caregivers know the amount of medical intervention you are willing to accept. Ask your MDA Care Center team about setting up an advance directive.

Once you have documented this information, give copies to your medical providers, family and caregivers. Also tuck a copy in your backpack or purse and keep images of the forms on your phone.

And don’t forget to review the information at least every three months, or whenever your medications or treatments change.



Expert Care

MDA Care Centers provide expert clinical care for individuals living with muscular dystrophy, ALS and other neuromuscular diseases at more than 150 of the top healthcare institutions across the United States. To find an MDA Care Center, visit mda.org/care/mda-care-centers.



Toria Tozer with her husband, David

the physician took the time to teach me how to change the trach. Under his guidance, I removed the surgical trach and replaced it with a removable, cleanable one. This lesson helped me handle subsequent blockages at home and probably saved us many more trips to the ED.

ADVOCATE AND EDUCATE

Any time you visit the ED, it is critical that you or a loved one has your medical information on hand. You can do this by having a print-out or cards (some people laminate them) that you keep in your purse or backpack, a photo of that information on your phone and a medical alert pendant or bracelet with your diagnosis and emergency contact. Your caregivers should all know how to access your medical

“I was proud of my ability to handle myself in what could have become an even more dangerous situation.”

—Toria Tozer

information in case you cannot speak for yourself.

Having a friend or advocate with you in the ED is also valuable for the practical help they can give you for small tasks like reaching the call button and holding drinking cups. You and your advocates need to be aware that their presence with you in the ED is a privilege, not a right. If a buddy, spouse or parent causes a commotion or is verbally abusive to staff, they will be sent away.

Advocating for yourself is an important skill to develop as well. Oregon native Toria Tozer, who lives with SMA, recounts a time she landed in her local ED with a gallbladder infection that led to sepsis.

“I had been in the ED three times prior,” she says. “They finally decided I needed my gallbladder out, and I requested a 45-minute transfer to an academic hospital in Portland. The doctor argued with me, saying I’d be dead by the time I got there. I told him last time the hospital tried to do surgery, I aspirated due to my small airways from SMA, and if I didn’t transfer, they’d probably kill me anyway.”

In the end, Tozer was transferred to the hospital she requested, and she learned the power of self-advocacy. “I was proud of my ability to handle myself in what could have become an even more dangerous situation,” she says.

BE PREPARED

If at all possible, bring your own medical equipment to the ED, especially bipaps or other respiratory support items. Some hospitals may not have access to equipment such as a cough assist machine, and, Dr. Naprawa points out, patients and caregivers feel more comfortable with the equipment they’re accustomed to.

Dr. Wolfe cautions that some hospitals may not allow unfamiliar equipment to be used. In that case, she advises asking if the hospital can provide equipment that is similar to the patient’s and a respiratory therapist trained on that equipment.

Along with a list of current medications that should be included with your medical information, try to bring along your actual medications, especially if some of them are specifically for your neuromuscular condition. The hospital pharmacy may not have what you need on hand when you need it.

Before you put this magazine down, write down the important information listed in the sidebar — don’t put off documenting your physical needs.

Having that information when you arrive at the ED’s sliding doors will help the staff inside get you the right care. It could be the thing that saves your own or a loved one’s life. **Q**

Donna Albrecht is a health writer who lives in Northern California with her husband and border collie.



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News on LEMS

Treatment and Research

A Q&A with researcher Stephen Meriney, Ph.D.



on LEMS-model mice. *Quest* asked him about the latest news in LEMS.

What does the treatment landscape for LEMS look like today?

There is no cure for LEMS, as scientists have not yet figured out how to selectively stop the autoimmune attack on motor nerve terminal calcium channels and other nerve terminal proteins targeted by LEMS. Therefore, symptomatic treatments for neuromuscular weakness that results from LEMS are favored.

A potassium channel blocker called 3,4-diaminopyridine (DAP) was discovered in the 1970s and '80s to be an effective LEMS therapy. By blocking some potassium channels, DAP increases acetylcholine release.

Since its discovery, DAP has been synthesized by compounding pharmacies in many countries and permitted in the U.S. through the "compassionate use" program. A private company in New Jersey called Jacobus Pharmaceutical has been providing DAP at cost, or for free, to many patients for years. As a result, DAP has been the symptomatic treatment standard for more than two decades.

Although less common, some patients may receive treatment with an

In Lambert-Eaton myasthenic syndrome (LEMS), the immune system attacks the connection between nerve and muscle — the neuromuscular junction — and interferes with the ability of nerve cells to send signals to muscle cells.

Specifically, the attack targets the calcium channels on nerve endings that are required to trigger the release of acetylcholine, a chemical messenger that triggers muscle contraction. With

fewer calcium channels, the nerve endings release less acetylcholine. With low levels of acetylcholine, muscles do not contract normally, resulting in muscle weakness.

Stephen Meriney, Ph.D., professor in the department of neuroscience and co-director of the Center for Neuroscience Graduate Program at the University of Pittsburgh, recently completed an MDA-supported research project focused on testing a new calcium channel modifier

About LEMS

Learn more about Lambert-Eaton myasthenic syndrome (LEMS) at mda.org under "About Neuromuscular Diseases."

acetylcholinesterase inhibitor (to prolong the lifetime of acetylcholine after release), by plasmapheresis (to filter out antibodies from the blood), with immunosuppressant drugs or through the administration of intravenous immunoglobulin (which modifies the immune response).

Most recently, a phosphate salt of DAP — Firdapse — was created with a more stable shelf life. Firdapse was approved by the European Medicines Agency (through BiMarin Pharmaceutical). The U.S. Food and Drug Administration (FDA) approved Firdapse in November 2018 (through Catalyst Pharmaceutical), and Catalyst predicts the drug will be available in the United States in 2019.

These regulatory approvals are great news for LEMS patients who were having trouble obtaining DAP previously but have been met with concern from other LEMS patients who were obtaining DAP for little or no cost. It is not yet clear how access to Firdapse for all LEMS patients will be managed. However, Catalyst has indicated that it will work with all patients to ensure access.

What are the most important recent advances in LEMS research?

The most exciting advances are:

- Approval of Firdapse, a stable form of DAP for the symptomatic treatment of LEMS
- Identification of diagnostic criteria that can predict cancer risk for LEMS patients

- Reports that LEMS improves small cell lung cancer prognosis
- Development of new calcium channel gating modifiers

What are researchers exploring now?

Current research includes clinical studies of LEMS patients to advance our understanding of disease progression and association with cancer; studies using a LEMS mouse model to understand in more detail how LEMS antibodies alter the transmitter release site, and the further refinement and preclinical testing of new calcium channel modifiers that hold promise as potential therapeutics.

Are there any promising new treatments in the pipeline?

One new treatment in preclinical development is a small molecule that holds calcium channels open longer. Animal studies have shown that when this calcium channel modifier is combined with DAP, the magnitude of acetylcholine release in LEMS-model mice can be completely restored to normal levels. This combination therapy approach has potential to be a next-generation treatment for LEMS and other conditions with reduced neurotransmitter release.

Why is it important to continue to fund LEMS research?

Current symptomatic treatment, with DAP or Firdapse,



Stephen Meriney, Ph.D.

Most recently, a phosphate salt of DAP — Firdapse — was created with a more stable shelf life. ... The U.S. Food and Drug Administration (FDA) approved Firdapse in November 2018.

usually is not sufficient to allow patients to return to normal activity (due to dose-limiting side effects). Therefore, individuals need more options for treatment (either add-ons or novel treatments). In order to develop such treatments, basic science studies in LEMS that can illuminate potential new targets must continue to be funded. [Q](#)



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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 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**.

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



access MDA


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

Ready for Anything

Download MDA's emergency checklist for disaster preparedness

Are you and your family prepared if a disaster strikes? With the start of summer comes the potential for more weather-related catastrophes such as hurricanes, tornadoes and floods. Make sure you are ready for the unthinkable with the help of MDA's Preparing for Emergencies: A Checklist for People with Neuromuscular Disease.

The checklist can be used to prepare a personalized emergency plan, recognize potential hazards in your home, assemble an emergency supply kit and devise an evacuation strategy. The list also includes questions to ask local agencies, employers and teachers to make sure you and your loved ones can get assistance in an emergency at home, school and work.

 To download and print the checklist, go to mda.org/emergency-resources and click on the link for "Preparing for Emergencies: A Checklist for People with Neuromuscular Disease."



Facebook, Friends and Fundraising

MDA raises almost \$1 million on the social media network



“I set the goal for \$1,000. We ended up hitting that and it didn’t take long,” he says.

CROWD SOURCING

Facebook fundraisers are a growing source of donations for MDA. In 2018, nearly \$1 million came in from hundreds of supporters on the social media network.

This unique way to collect funds allows Facebook users to create a fundraising page explaining the charity they are supporting and why it is important to them, then supporters can make donations directly through the page.

FUND WITH FRIENDS

Christina Van Pelt, who lives in Paso Robles, Calif., and has an 8-year-old son with Duchenne muscular dystrophy (DMD), is no stranger to MDA fundraising. She participates in Fill the Boot and raised more than

When Ethan Och decided to host a Facebook fundraiser for his 21st birthday last year, there was no question of what organization he was going to support.

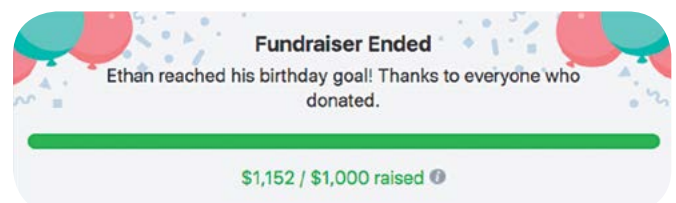
“What came to mind above all other things was MDA because they’ve helped me do cool things over the years,” he says. Diagnosed with spinal muscular atrophy (SMA) when he was 1 year old, Och fondly

remembers the time he spent at MDA Summer Camp during his childhood. In addition, MDA assisted his family in getting vital pieces of medical equipment, as well as offering priceless support and education over the years.

Now, Och is an aerospace engineering major at the University of Minnesota and has an extensive Facebook network that he was sure would be supportive. Even so, he was surprised at the turnout for his birthday fundraiser.

In 2018, nearly \$1 million came in from hundreds of supporters on the social media network.

Ethan Och hosted a Facebook fundraiser to benefit MDA for his 21st birthday.





\$10,000 for a recent Central Coast Muscle Walk – and she has hosted at least four Facebook fundraisers for MDA.

So far, every goal she has set on the social media site has been surpassed. “If I set it at \$300, I will raise about \$500 to \$600,” she says.


Not only do her Facebook friends donate, but they share her fundraising page so their friends will see it and increase donations. With this amount of support, she says she is going to continue hosting Facebook fundraisers.

“I don’t only do it for me or


my son, I do it for every child that needs my help,” she says. “We need to find a cure for these kids.”



Christina Van Pelt gets lots of support from her Facebook friends every time she hosts one of her fundraisers.

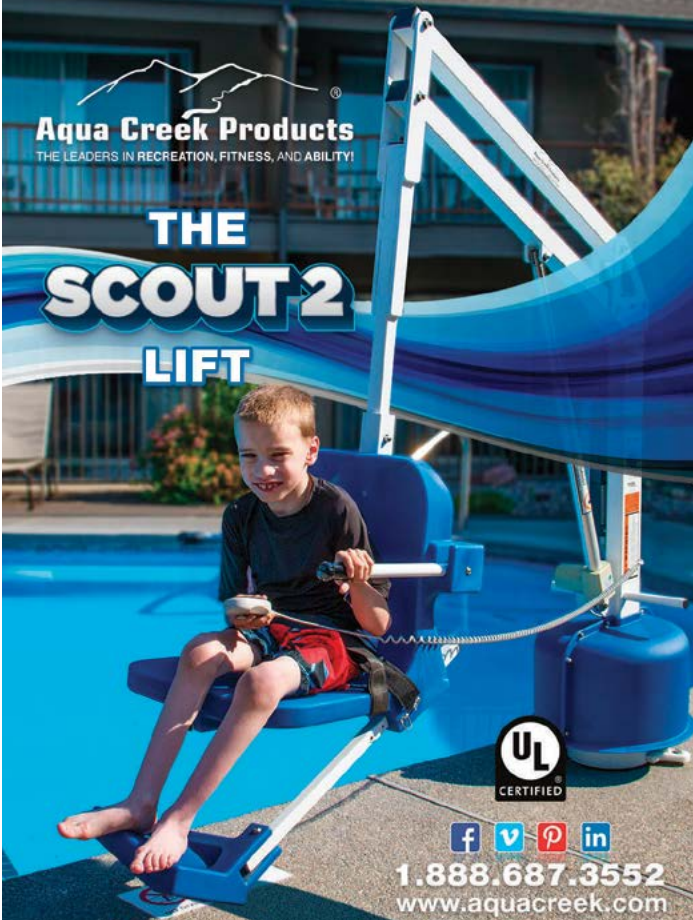
 **To create your own Facebook fundraiser for MDA, visit [facebook.com/MDAorg](https://www.facebook.com/MDAorg).**


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Dutch Bros presents a check to MDA during ALS Awareness Day at the Arizona Diamondbacks game.

Coffee and a Cure

Drink One for Dane on May 10

Get your caffeine fix and join MDA's quest to end amyotrophic lateral sclerosis (ALS) on Friday, May 10, at Dutch Bros Coffee's annual Drink One for Dane event.

Now in its 13th year, this one-day event is Dutch Bros' largest annual charity fundraiser. Last year's event raised more than \$1.3 million for MDA. Across seven states, more than 300 of the drive-through coffee chain's locations will donate all proceeds to fund MDA's cutting-edge research and support families affected by ALS and other muscle-debilitating diseases.

Drink One for Dane was inspired by Dutch Bros co-founder Dane Boersma, who

passed away from ALS in 2009. His legacy of kindness and selflessness lives on in this event and in all of the fundraising Dutch Bros does for MDA – an effort that now totals more than \$4.8 million.


"It is because of inspirational programs like Drink One for Dane that we are able to fund innovations in science and care for people living with ALS. These investments lead to better treatments and will ultimately unlock the cure," says MDA President and CEO Lynn O'Connor Vos.



The Dutch Bros team in action during Drink One for Dane



"Broistas" spread the mission of ending ALS during Drink One for Dane.

 Find Dutch Bros Coffee locations in Oregon, Washington, Colorado, California, Arizona, Nevada and Idaho at dutchbros.com.

Progress in Motion

MDA's 2019 Clinical & Scientific Conference

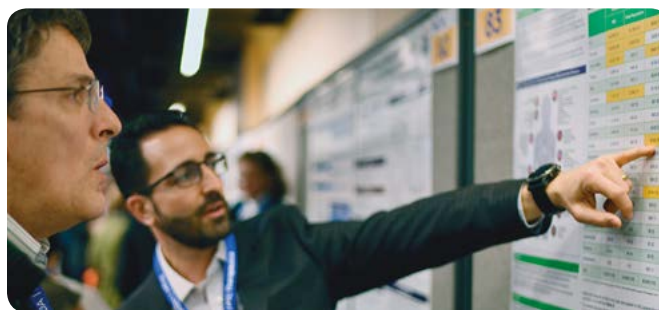
This year will be an exciting one for MDA as we converge our long-standing Clinical and Scientific conferences into an

inaugural combined annual meeting, themed "Progress in Motion." The conference will present a unique opportunity



MDA National Ambassador Faith Fortenberry attended the 2018 MDA Clinical Conference.

for attendees to learn, be inspired and share ideas with experts from academia, government and industry while supporting MDA's mission to transform the lives of individuals living with muscular dystrophy, ALS and related neuromuscular diseases.



MDA's Clinical & Scientific conference is targeted toward scientists and clinicians rather than patients, families and caregivers. However, you can keep up with the findings from the conference in a few ways:

- > Look for our blogs on strongly.mda.org.
- > Follow us on social media via the tag [#MDAconference](https://twitter.com/MDAconference).
- > Read about conference highlights in an upcoming issue of *Quest*.



To learn more about MDA's inaugural Clinical & Scientific Conference and watch a video of highlights from the 2018 MDA Clinical Conference, visit mda.org/conferences/2019-clinical-and-scientific-conference.

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A resource developed for patients and caregivers whose lives have been impacted by Duchenne muscular dystrophy

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December 2018 | MAT-DMD-0358

‘What Happened to You?’

A young man with SMA answers this question by becoming a student leader

BY JOHN SCURTO



“I wanted people to look past my chair and see me simply as one of them.”

—John Scurto

TRYING TO BELONG

Throughout my childhood, I spent more than 100 days in the pediatric intensive care unit of my local hospital because of multiple surgeries and repeated battles with pneumonia.

After I became medically stable, I was able to attend elementary school regularly. Because I spent so much time interacting with adults in the hospital and with home health nurses, I struggled to connect with people my age.

Seeing my peers run around and enjoy the slides and monkey bars while I sat and watched from my wheelchair was tough. In my mind, I imagined breaking free from my chair and partaking in the amusements alongside them.

Whenever I glanced in the mirror, I did not see my wheelchair. It is an accessory I use to accomplish the task of mobility. I wanted people to look past my chair and see me simply as one of them.

“What happened to you?” The question became familiar to me as a child while the young, curious faces stared at my power wheelchair.

I wanted so badly for my peers to see me as a “normal” kid, yet there was nothing I could do to mask my differences.

I have spinal muscular atrophy (SMA), a genetic condition caused by a mutation that affects muscle use. Only 1 in 11,000 people are born with this condition.

SMA robbed me of my ability to walk and progressively weakened the muscles in my legs, arms and hands. Additionally, SMA makes my swallowing and breathing more difficult. My condition has forced me to rely on a power wheelchair since the age of 4 and on the assistance of others for my daily physical needs.

Each time a person asked what happened to me, it made me feel as if I was part of a rare, nonhuman species.

Once I got to high school, I was looking forward to making lots of new friends. I had a desire to show people that having a physical disability does not mean I suffer from any cognitive deficiencies, a common misconception.

I was excited for a new chapter in my life.

Throughout the four years, I watched as my peers ate and socialized with each other in the cafeteria while I sat alone with my nurse feeding me lunch. I lacked the self-confidence to approach and initiate conversations with others. Every day, I hoped for someone to join me, but no one ever did.

It saddened me that my uniqueness of having a disability overshadowed my similarities to my peers. I felt like I was punished for having a disability.

Despite the social isolation during my high school years, I graduated in the top 10 percent of my class with a near-perfect GPA. This academic success led to membership in multiple honor societies and a full college scholarship.

FINDING MY PLACE

I eagerly awaited the new beginning I would have as an undergraduate at Florida Atlantic University (FAU) in Boca Raton. I refused to continue the nonexistent social life.

I had a profound desire to finally make friends and grow as a person.

In my first year of college, I completed two leadership programs that provided me with an opportunity to discover what leadership means

to me: helping others achieve their desired goals.

I then received an invitation to join the National Society of Collegiate Scholars. Less than a year later, I had the honor of serving as the organization's vice president. This acceptance from my peers marked one of the first times I truly felt valued and not judged by my disability.

During my junior year, I accomplished my most fulfilling achievement to date: I became an Elite Owl. Elite Owls are leadership and service ambassadors who passionately promote leadership development by facilitating workshops and mentoring students.

Through this organization, I have presented in front of hundreds of students at leadership conferences. While I have always dreaded public speaking, I ultimately gained the confidence to be a presenter for large audiences.

The welcome I felt from the students in Elite Owls forever changed my life. For the first time, I felt like I had a lot of friends. They exemplified the true meaning of leadership, and I want to make that same impact in other people's lives.

Shortly after, I was named the valedictorian of the two-year University Honors Program at FAU, graduating with a 4.0 GPA. I took on this ambitious aspiration and all of my other college achievements to demonstrate that an individual can accomplish anything, no matter what.

BECOMING A LEADER

While there are many tasks I am unable to perform, I have

done so much, both despite having a disability and because of my disability. Just because someone is physically or mentally unable to do something does not mean that he or she should be treated differently; we all have characteristics that make us unique.

I made it my life's purpose to serve as an example for disabled and nondisabled individuals to show that we all face obstacles in life, visible or not, and the way we respond to our situations shows who we are as individuals.

I may have SMA, but it is not who I am. My life is defined by the choices I continue to make to be someone who can inspire others.

I now find myself asking the same question that people used to ask me when I was younger: "What happened to you?"

The answer: I chose to embrace and accept my disability.

Instead of letting situations I cannot control hinder my life, I decided to achieve the high ambitions I dreamed of to demonstrate the power of actualizing a vision.

Through perseverance and tenacity, I have become a role model and leader, lighting the path for others to follow in pursuit of their dreams, so we can better ourselves and the world we live in. **Q**

John Scurto, 21, was diagnosed with SMA in 2017. He is currently a senior at Florida Atlantic University in Boca Raton, majoring in business management and minoring in leadership studies.

Make a Difference

Sharing your journey can be daunting, but it can make a world of difference to someone going through similar struggles. If you'd like to share your story with Quest, email us at quest@mdausa.org.

MVPs and All-Stars

Derek Jeter is chosen as MVP at New York Muscle Team Gala



Clockwise from above: MDA Chairman of the Board R. Rodney Howell, M.D., and Derek Jeter; the New York Giants' Mark Herzlich; MDA National Ambassador Faith Fortenberry and Jeter

Photos by: Getty Images; Greg Morris

Thanks to MDA Muscle Team MVP Derek Jeter, the New York Muscle Team's 22nd annual gala on Dec. 3 was truly an all-star event. Living up to its theme "Dream Big," the event featured a fireside chat with Jeter, moderated by New York Muscle Team committee member and legendary sportscaster Russ Salzberg, who was also the host of the event. Jeter's life was touched by neuromuscular disease recently, when the co-author of his *Baseball Genius* book series, Tim Green, publicly announced his diagnosis with amyotrophic lateral sclerosis (ALS).

Since 1996, celebrities, athletes and real estate executives have gathered at this annual gala to raise money to end neuromuscular disease, support families and send kids to MDA Summer Camp. Since its inaugural year, the star-studded event has raised more than \$20 million. The gala also recognized several honorees, including Richard C. Heller, president of Titanium Scaffold Services, LLC, and the Hamad family of Staten Island, who has lost three sons to Duchenne muscular dystrophy (DMD) and continues to raise awareness and funds in pursuit of a cure.

"Producing this event for the 22nd year shows the real estate community's deep commitment to our goals of funding research for treatment and cures and sending kids to camp for the best week of the year," says Stacie Spitzkoff, executive director of MDA New York Metro. "Having Derek Jeter and so many incredible professional athletes from the Giants, Jets, Mets and more professing their support for our mission and our families never ceases to inspire us." **Q**

Special Events

MDA events are a fun way to join your community in raising vital funds and awareness to support families living with neuromuscular diseases. Learn about events in your community at mda.org/get-involved/participate-in-an-event.

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INDICATIONS AND USAGE:

FIRDAPSE is a potassium channel blocker indicated for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.

IMPORTANT SAFETY INFORMATION**CONTRAINDICATIONS**

FIRDAPSE is contraindicated in patients with:

- A history of seizures
- Hypersensitivity to amifampridine phosphate or another aminopyridine

WARNINGS AND PRECAUTIONS

Seizures: FIRDAPSE can cause seizures. Consider discontinuation or dose-reduction of FIRDAPSE in patients who have a seizure while on treatment. FIRDAPSE is contraindicated in patients with a history of seizures.

Hypersensitivity: If a hypersensitivity reaction such as anaphylaxis occurs, FIRDAPSE should be discontinued and appropriate therapy initiated.

ADVERSE REACTIONS

The most common (> 10%) adverse reactions are: paresthesia, upper respiratory tract infection, abdominal pain, nausea, diarrhea, headache, elevated liver enzymes, back pain, hypertension, and muscle spasms.

To report **SUSPECTED ADVERSE REACTIONS**, contact Catalyst Pharmaceuticals at 1-844-347-3277 (1-844-FIRDAPSE) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.



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