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How to choose a mobility scooter or power wheelchair

REMARKABLE REPS
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students of the world

Studying abroad comes with challenges, benefits and memorable experiences

MDA For Strength, Independence & Life

MDA is leading the fight to free individuals — and the families who love them — from the harm of muscular dystrophy, ALS and related life-threatening diseases that take away physical strength, independence and life. We use our collective strength to help kids and adults live longer and grow stronger by finding research breakthroughs across diseases; caring for individuals from day one; and empowering families with services and support in hometowns across America.

Learn how you can fund cures, find care and champion the cause at **mda.org**.

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Dreaming Big

At this time of year, it seems fitting to talk about independence, new possibilities and summer dreams. We are proud to give our campers the opportunity to dream big for a week each summer. MDA Summer Camp is a place where anything is possible, and kids can live beyond limits. We believe that giving children and teens a special week to get out of their comfort zone, achieve independence and build confidence is the reason parents, families, sponsors and campers look forward to it all year. Not to mention all the friends to be made and fun to be had.

Older teens and young adults may want to dream big through a study-abroad program. Studying abroad can help them take in a new culture, learn a new language and see the world. In this issue, we explore some ins and outs of this life-changing experience (see page 24).

For individuals of all ages with neuromuscular disease, nothing provides independence and facilitates dreaming big like the right mobility aids and having access to travel. In the Thrive 365 article "Learning to Love Your Wheels" (page 16) and the feature article "Get Ready to Roll" (page 18), we look at some of the benefits and tradeoffs between mobility scooters and power wheelchairs, describe what happens at a typical mobility evaluation, and examine the transition to a mobility device. MDA is proud to participate in advocacy efforts around access to travel and complex rehabilitative technologies, and we hope you will join us and become an advocate by signing up at **mda.org/advocacy**. You'll also find more information on MDA's advocacy including MDA's Accessible Air Travel Resource Center. Connect with us on Twitter by following and tweeting **@MDA_Advocacy**.

"At MDA, our big dream is also our mission: to transform the lives of individuals affected by neuromuscular disease."
— Kristin Stephenson

Ask MDA

Remember, our Resource Center is available to answer your questions about mobility aids, summer travel and any other area in which you need assistance. Contact us at **833-ASK-MDA1** or **resourcecenter@mdausa.org**.

Dreaming big is a universal concept that resonates with people of all ages and abilities. At MDA, our big dream is also our mission: to transform the lives of individuals affected by neuromuscular disease. We will never stop reaching for new discoveries, medical and scientific breakthroughs, innovations in care, and life-changing solutions.

Sincerely,

Kristin Stephenson
Sr. VP, Chief Policy & Community Engagement Officer

summer 2018

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WHAT DO YOU THINK OF *QUEST*?

Let us know what you think of *Quest*, what we could do better and what you would like to see more (or less) of in these pages. Everyone who completes our reader survey will be entered into a drawing for a $100 Visa gift card. **Visit surveymonkey.com/r/mdaquest18 or use the QR code below to take our reader survey.**

MDA-024

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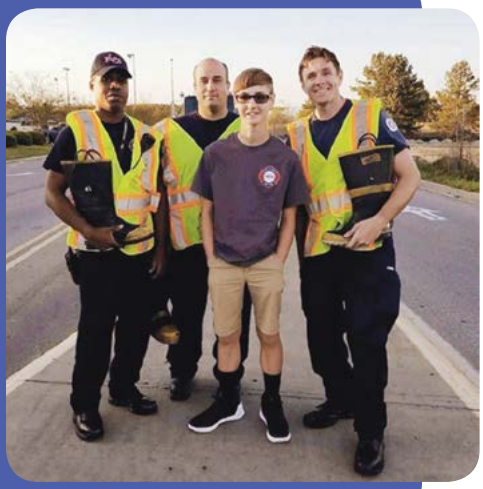
Fighting for a Cause

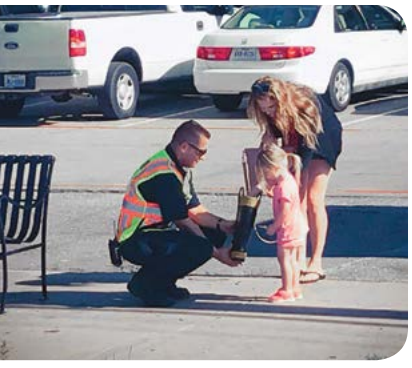
Supporting MDA is a cherished tradition among fire fighters. But their support goes well beyond Fill the Boot, a campaign where more than 100,000 fire fighters hit the streets each year asking for donations to help MDA fight muscle disease and support the families living with it. Here is a social media snapshot of some of the great work fire fighters do for our community.

Strongly.mda.org
"We tried to get families who have kids with muscular dystrophy to come by the station, have dinner with us, go down to City Hall, even join us at Fill the Boot. We want them to know that our work with MDA doesn't just end with Summer Camp or Fill the Boot. We want to be an influence in their lives."
— Daniel Buford

lexcofire *Lexington County Fire Service has been putting in work for MDA. Local Ambassador Dylan stopped by to say thank you to the men and women who have been Filling the Boot.*



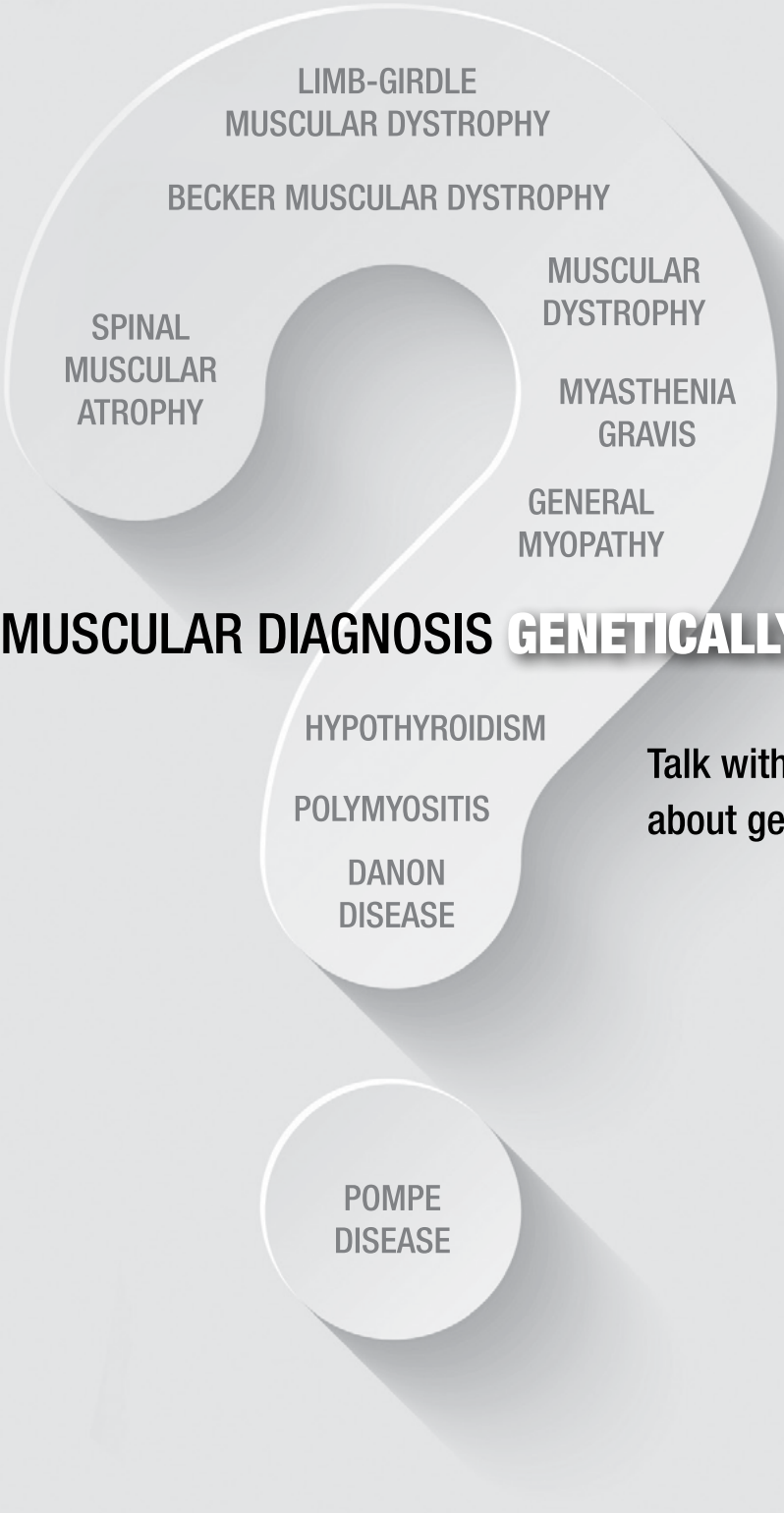
@mdawisconsin *MDA Summer Camp 2018!*



@PlanoFFs2149
#Generosity comes in all sizes during the #Plano #FilltheBoot drive.

Find Your Cause

Generous donors, event participants and dedicated volunteers are the heart of MDA's programs. They make it possible for MDA to fund groundbreaking research across diseases and support kids and adults from day one. Learn how you can get involved at **mda.org/get-involved**.



IS YOUR NEUROMUSCULAR DIAGNOSIS GENETICALLY CONFIRMED?

Talk with your MDA physician about genetic testing.

SANOFI GENZYME

progress now

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

ALS (amyotrophic lateral sclerosis)

Imaging in ALS

MDA and ALS ONE team up to advance biomarker research

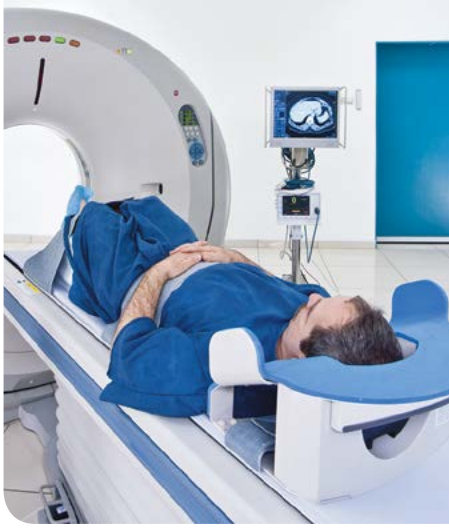
One of the biggest challenges hindering ALS drug development is a lack of biomarkers that can be used in clinical trials to signal whether a drug is working in patients. To help overcome that challenge, MDA has awarded a human clinical trial grant totaling $750,000 to ALS ONE to explore the potential for a type of imaging called positron emission tomography (PET) to measure inflammation in the brain that could serve as a biomarker for ALS.

The project, led by Nazem Atassi, M.D., MSSc, will use PET imaging to track inflammation in healthy people who carry a known ALS gene and in symptomatic people with early-stage ALS.

In previous studies, data has shown a significant increase in inflammation in the motor regions of the brain in people with ALS. It also has been found that more inflammation tends to be associated with a more advanced stage of disease progression and greater impairment of motor function. Now, Atassi and colleagues are working to build on those findings and assess the ability of PET imaging to measure the degree of inflammation in people with very early ALS symptoms and in people who carry one of the ALS genes but are asymptomatic.

Implementing PET imaging technology potentially could reduce the duration of future trials from 12 months to three months, and it could reduce the required patient enrollment from 400 participants to 30.

Results from the study could contribute to the growing body of evidence suggesting PET imaging can be used to accurately measure brain inflammation in people living with ALS.



Read more about this exciting new project at strongly.mda.org.

Charcot-Marie-Tooth disease (CMT)

Targeting Muscle Cramps

Participants needed for trial to test FLX-787-ODT

Muscle cramps can negatively affect quality of life for individuals with CMT.

Researchers are looking for people with CMT to participate in a phase 2 clinical trial to evaluate the safety and effectiveness of the investigational drug FLX-787-ODT.

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

Trial length is approximately three months, during which participants will visit with study investigators approximately 20 times. For the duration of the study, participants will record muscle cramp frequency in a diary during the 28-day treatment period. To be eligible to participate, individuals must:

1. Be at least 18 years old
2. Have a documented genetic confirmation of a mutation known to cause CMT, or clinical evidence of CMT and a genetic confirmation in a family member
3. Experience weekly muscle cramping
4. Meet additional criteria

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

To learn more about this trial, visit clinicaltrials.gov and enter NCT03254199 in the "Other Terms" search box.

Congenital muscular dystrophy (CMD)

Omigapil in CMD

Encouraging results in phase 1 CALLISTO trial

Santhera Pharmaceuticals has reported it will discuss the results of this phase 1 trial with clinical experts and regulatory authorities to prepare for a pivotal trial in people with CMD.

A phase 1 trial to test omigapil in CMD, conducted at the National Institutes of Health, is complete, and data indicate it met its primary objective. Results from the trial, also known as CALLISTO, showed a favorable pharmacokinetic profile of omigapil and demonstrated that the drug was safe and well-tolerated in children and adolescents with MDC1A and Collagen VI subtypes of CMD. ("Pharmacokinetics" refers to how the drug is absorbed, distributed and metabolized in the body.)

Omigapil is an anti-apoptotic drug designed to prevent cell death. In previous studies in a mouse model with CMD type 1A, results indicated that treatment with omigapil inhibited cell death, reduced weight loss and skeletal deformation, improved motor function, and increased life span.

Under development by Santhera Pharmaceuticals, the drug has received U.S. Food and Drug Administration (FDA) Orphan Drug and Fast Track designations for the treatment of CMD.

For more information about omigapil and the completed clinical trial, visit strongly.mda.org.

Congenital myasthenic syndrome (CMS)

Firdapse in CMS

Participants sought for phase 3 trial

Researchers are looking for children and adults with CMS to participate in a phase 3 study being conducted by Catalyst Pharmaceuticals to test the experimental drug amifampridine phosphate (brand name Firdapse).

Amifampridine phosphate is a potassium channel inhibitor designed to cause greater stimulation of muscle by prolonging nerve signals, and is expected to help treat muscle weakness in people with CMS.

The goals of the study are to determine the safety, tolerability and efficacy of amifampridine phosphate in CMS.

Study participants will receive an initial evaluation and several clinical exams. Tests will include heart rhythm (ECG), muscle testing (EMG) and questionnaires. Participation is expected to last 56 days.

Participants may be male or female, age 2 years or older, with a genetically confirmed CMS mutation. Those whose CMS has not been genetically confirmed will have genetic testing done at screening, and additional eligibility criteria will be reviewed with each patient's physician.

Trial sites are located in California, Georgia, Maryland, Massachusetts and Ohio. Travel-related costs will be provided for those who are willing to travel.

For additional information on this trial, visit clinicaltrials.gov and enter NCT02562066 in the "Other Terms" search box. There you can find contact information for the trial coordinator at each study site.

Duchenne muscular dystrophy (DMD)

Researchers think Cosyntropin may work as an alternative to corticosteroids but with less severe side effects.

BRAVE Trial

Phase 2 DMD study seeks participants

Researchers are looking for people with DMD to participate in a phase 2 clinical trial called BRAVE to evaluate the safety and effectiveness of the investigational drug MNK-1411 (Cosyntropin).

Cosyntropin, under development by Mallinckrodt Pharmaceuticals, is delivered subcutaneously (injection under the skin) and is designed to slow the loss of muscle function and promote muscle regeneration in DMD.

The expected length of participation time is about one year, during which participants will make monthly visits to the study site to undergo muscle function tests, respiratory tests and blood draws. For a portion of the study, approximately one-third of participants will be randomly assigned to receive a placebo, while others receive Cosyntropin. However, after about 24 weeks, the study will roll over into an open label extension phase in which all participants can receive the drug. In order to be eligible to participate, individuals must:

- Be between 4 and 8 years old
- Have a documented genetic diagnosis of DMD
- Be able to walk
- Meet additional criteria

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

To learn more about this trial, visit clinicaltrials.gov and enter NCT03400852 into the "Other Terms" search box, or contact study coordinator Sean Ma at 800-556-3314 or clinicaltrials@mnk.com.

SIDEROS Phase 3 Trial

Participants with DMD needed

Researchers are looking for boys and men age 10 years or older with DMD to participate in a phase 3 clinical trial, sponsored by Santhera Pharmaceuticals, to assess the safety and efficacy of the experimental drug idebenone (brand name Raxone).

Idebenone is an oral tablet. Scientists hypothesize it may improve the way muscles utilize fuel to power movement, which could delay the loss of respiratory function in DMD.

SIDEROS is designed to assess whether idebenone can slow decline in respiratory function in boys and men with DMD. It will compare the efficacy of idebenone to placebo in individuals who currently are receiving treatment with steroids (prednisone or deflazacort).

In order to be eligible to participate, individuals may be ambulatory (able to walk) or non-ambulatory, must have been taking a stable dose of corticosteroids for at least 12 months, must not need daytime ventilator assistance and must meet additional study criteria.

After completing the study, all participants will have the opportunity to enroll in an open-label extension study in which everyone will receive treatment with idebenone.

The study is expected to last 22 months. Dozens of trial sites are open across the United States, and support is available for travel and hotel accommodations.

To learn more or to inquire about participation, email SIDEROS@santhera.com, visit Santhera's website at santhera.com or visit clinicaltrials.gov and enter NCT02814019 in the "Other Terms" search box.

BreatheDMD

Expanded access program launches for idebenone in DMD

In February, Santhera Pharmaceuticals launched an expanded access program (EAP) in the United States for boys and men with DMD to receive idebenone outside of a clinical trial.

Idebenone is an oral tablet in development and testing for DMD. Scientists hypothesize it may improve the way muscles utilize fuel to power movement, which could delay the loss of respiratory function in individuals with the disease. (See "SIDEROS Phase 3 Trial" on page 8.)

Through the EAP, called BreatheDMD, eligible patients age 10 years or older who are experiencing decline in respiratory function can obtain access to idebenone at no cost.

For details about BreatheDMD, visit breathedmd.com.

If you think you or your child may benefit from enrollment in BreatheDMD, speak to your physician.

Lambert-Eaton myasthenic syndrome **(LEMS)**

FDA Approval Sought for Firdapse

Catalyst Pharmaceuticals submits New Drug Application

Catalyst Pharmaceuticals has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for the investigational drug amifampridine phosphate (brand name Firdapse) for the treatment of people with LEMS. If the FDA accepts the NDA, it will begin the review process to decide whether Firdapse will gain approval in the U.S. for the treatment of people with LEMS.

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

The NDA follows Catalyst Pharmaceuticals' announcement of encouraging results from its second phase 3 clinical trial to test Firdapse in people with LEMS.

Firdapse is a potassium channel inhibitor designed to prolong signals released from nerves and allow greater stimulation of muscles.

Catalyst Pharmaceuticals has an Expanded Access Program (EAP) for Firdapse for people with LEMS or some types of CMS. The program is an open label pre-approval safety study in which a person who meets the inclusion and exclusion criteria can receive Firdapse, at no cost, if his or her physician determines it may help improve the person's condition.

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

Myasthenia gravis (MG)

Firdapse in MG

Enrollment is open for phase 3 trial

Firdapse currently is under clinical investigation as a symptomatic therapy to treat muscle weakness in people with MG.

Researchers are looking for people with MG to participate in a phase 3 clinical trial to evaluate the safety and effectiveness of the investigational drug amifampridine phosphate (brand name Firdapse). Effects of the drug will be assessed in individuals with muscle-specific kinase (MuSK) antibody positive MG and in a smaller group with acetylcholine receptor (AChR) antibody positive MG.

Amifampridine phosphate, under development by Catalyst Pharmaceuticals, is a potassium channel inhibitor designed to prolong signals released from nerves and allow greater stimulation of muscles.

Trial length is approximately 38 days, during which participants will visit with study investigators four to six times. At study visits, participants will undergo EKG testing, and treatment effects will be measured across two MG-specific assessment scales: The Quantitative Myasthenia Gravis (QMG) and the Myasthenia Gravis – Activities of Daily Living profile (MG-ADL).

In order to be eligible to participate, individuals must:

- Be at least 18 years old
- Have a diagnosis of MG with positive serologic test for anti-MuSK antibodies or anti-AChR antibodies
- Meet additional criteria

The trial is taking place at more than a dozen study sites across the United States. Support for travel costs may be available.

To learn more about this trial, visit clinicaltrials.gov and enter NCT03304054 in the "Other Terms" search box.

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

What do I need?

- A computer with an internet connection
- An email address

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 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

Myasthenia gravis (MG)

Phase 2 Trial Seeks Participants

Study will assess safety, tolerability and efficacy of UCB7665

Researchers are looking for people with MG to participate in a multinational phase 2A clinical trial to evaluate the safety, tolerability and effectiveness of the investigational drug UCB7665. >

Trial length is approximately 18 weeks, during which participants will visit with study investigators approximately 20 times.

UCB7665, under development by UCB Pharma, is administered via subcutaneous (under-the-skin) injection and is designed to reduce the severity of muscle weakness and fatigue in individuals with MG, as indicated via the Quantitative Myasthenia Gravis (QMG) and Myasthenia Gravis – Activities of Daily Living profile (MG-ADL) scores.

Trial length is approximately 18 weeks, during which participants will visit with study investigators approximately 20 times.

In order to be eligible to participate, individuals must:

- Be at least 18 years old
- Have a diagnosis of MG
- Meet additional criteria

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

To learn more about this trial, visit clinicaltrials.gov and enter NCT03052751 in the "Other Terms" search box.

Spinal muscular atrophy **(SMA)**

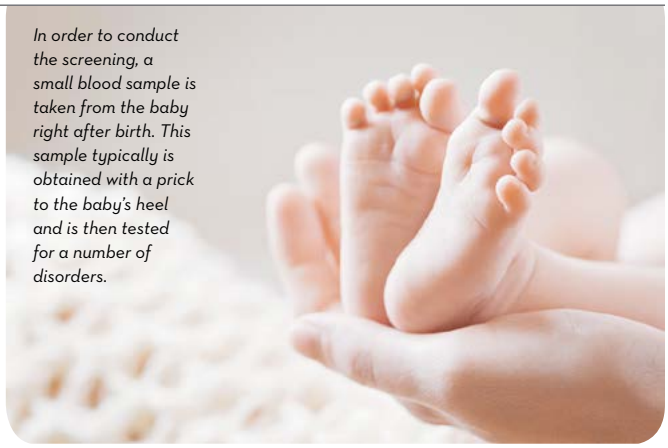
SMA Added to National Newborn Screening List

Testing in newborns could lead to early treatment, better outcomes

After the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) voted in February to add SMA to the Recommended Uniform Screening Panel (RUSP), the secretary of Health and Human Services has accepted the recommendation to officially add the disease to the RUSP.

Newborn screening is a public health program in the United States that aims to identify newborns who have certain serious and life-threatening genetic diseases that can be treated, and for which earlier treatment may contribute to better outcomes.

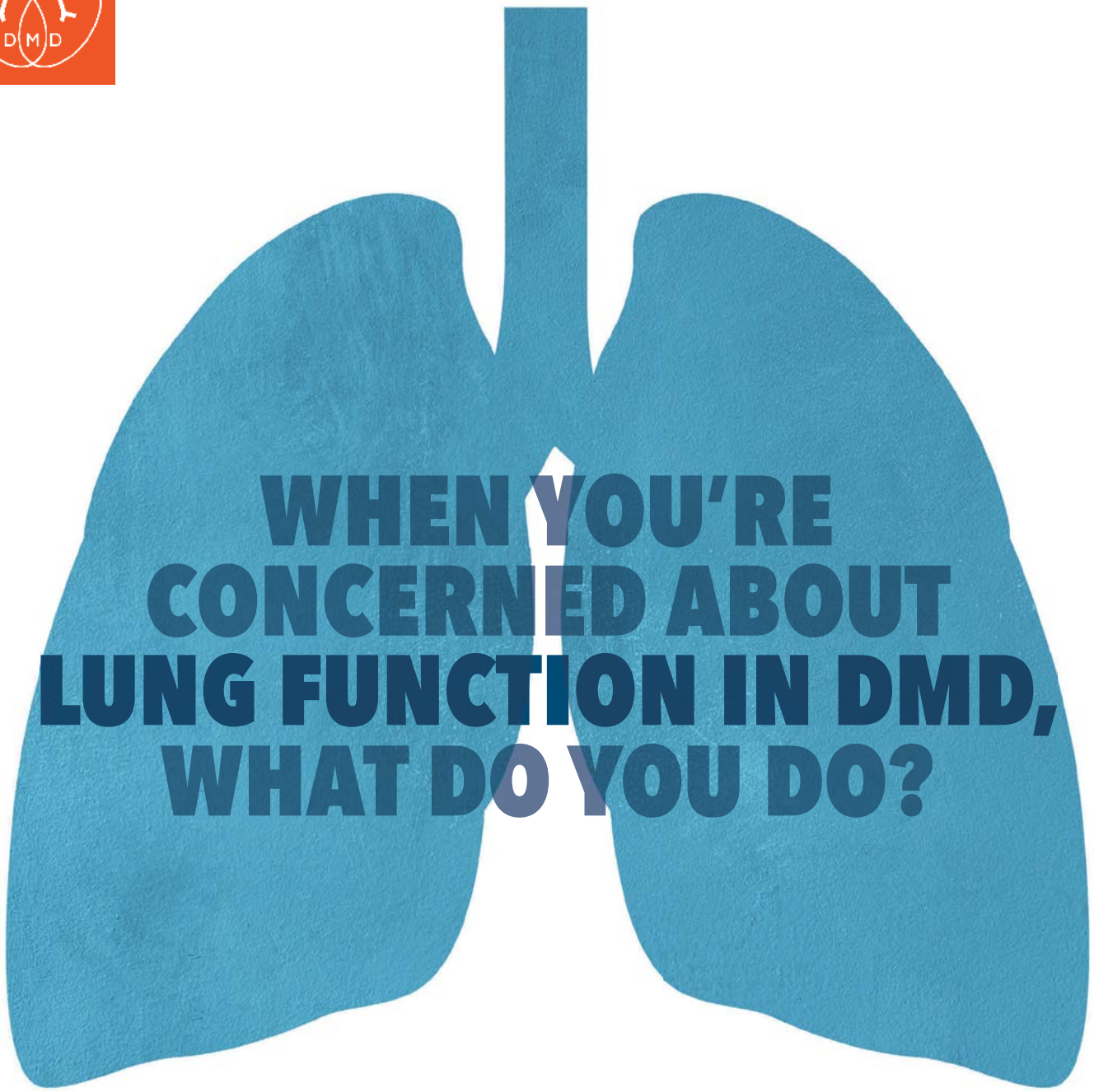
With SMA now part of the screening protocol, children identified with the disease may be able to begin immediate treatment with new drugs such as Spinraza, the first treatment for SMA that can improve both lifespan and quality of life. Research suggests that early treatment leads to the best outcomes. Newborn screening can also provide families with a genetic diagnosis—information that is often required to determine whether children are eligible to participate in promising clinical trials.



In order to conduct the screening, a small blood sample is taken from the baby right after birth. This sample typically is obtained with a prick to the baby's heel and is then tested for a number of disorders.

With the addition of SMA to the RUSP, each state will now consider adding SMA to their individual newborn screening panels. The process and timing will vary from state to state.

To read more about newborn screening for SMA, visit strongly.mda.org.



WHEN YOU'RE CONCERNED ABOUT LUNG FUNCTION IN DMD, WHAT DO YOU DO?

First, take a breath.

Lung function is important. So is feeling supported. Having access to respiratory information can make you feel empowered. On TakeabreathDMD.com, you can read about ways people with Duchenne muscular dystrophy (DMD) can help manage their lung function and their well-being. We'll provide helpful information and keep you informed about the news in DMD.

Visit TakeabreathDMD.com for respiratory information and to sign up for news updates.

santhera

Learning to Love Your Wheels

Tips for transitioning from walking to using a mobility device

BY KAREN HENRY

Twins Race and Ried transitioned to power-assisted manual wheelchairs.

Matt Curcio is a 27-year-old disability rights advocate who is living with a form of congenital myopathy. He regularly speaks and writes to increase awareness of the disability community's needs and encourage individuals with disabilities to seek the support they need to pursue their dreams.

Yet, even as he advocates for others, he has experienced his own struggles with asking for assistance. "Even at a young age, I've considered certain types of help as enablement for myself," he says. As his disease progressed, he resisted using a mobility device for as long as possible. While Curcio can still walk short distances, he now relies on a mobility scooter outside his apartment. Transitioning to the scooter was a 12-year process.

"It happened against my will," he says. "As an advocate, I'm all about people using medical devices to be more independent. As an individual, it has been a very contentious transition."

From the outside, using mobility equipment when you can no longer be independently mobile seems simple. However, when you or your child is the one facing the transition, it's not so easy. Worries about how you will be seen by others, anxiety about the rate of disease progression and fear of the unknown are common.

While there is no one-size-fits-all approach to embracing mobility equipment, individuals who have made the transition, and the caregivers and care providers who help them through it, offer some valuable advice.

"Getting the [wheelchairs] has really extended their mobility and increased their independence."
– Shannon Mosimann, mother of Race and Ried

1. Take time to grieve

Jenny Kean, LCSW, a social worker who works with children with neuromuscular diagnoses at the Ann & Robert H. Lurie Children's Hospital of Chicago, encourages parents and caregivers to give their kids — and themselves — the space to be upset and grieve. "The loss of ambulation is an emotional loss," she says. "It's healthy to grieve and cry about it."

Keeping the lines of communication open can help family members successfully navigate the grieving process.

"The unknown piece is very scary," she says. "Helping kids talk openly about their muscles and how they are changing in language that makes sense to them can be very powerful. It helps prepare them for what is to come."

2. Keep kids involved

For years, doctors advised Shannon Mosimann to put her 13-year-old twin sons Race and Ried in wheelchairs. Rather than forcing wheelchairs on her sons, who are both living with Duchenne muscular dystrophy (DMD), she asked them if they wanted to continue walking.

"I let most of these decisions come from them," she says. "If they said they wanted to keep on walking, I supported them."

Ann Motl, a 27-year-old litigation attorney with Charcot-Marie-Tooth disease (CMT), says she would have liked to have been offered the same choice. When she was in middle school, she was told that she had to use a mobility scooter during recess.

"I didn't feel like I needed it at the time," she recalls. "I wish I would have been the one to make that decision."

3. Know your options

Motl says it can been helpful to keep in mind that transitioning to a powered mobility device does not always close the door on walking. "Some people can still do a combination of typical walking, walking with a mobility aid, and powered mobility," she says. "Check out your options. It was easy for me to start with a scooter and then transition to a customized wheelchair as things got more difficult." (Learn how to choose a power mobility aid in "Get Ready to Roll" on page 18.)

Race and Ried currently have enough arm strength to propel their wheelchairs, so they are using power-assisted manual wheelchairs.

"There's a motor on the back equipped with a switch," Ried says. "It can help when we're going uphill, or if we're tired and can't wheel ourselves, we can turn it on so all we need to do is steer."

4. Change your perspective

Some might view the transition to a mobility aid as losing freedom, but clinging to that perspective is limiting. The transition can be life-changing in a positive way. It can allow an individual to maintain independence, reduce risk of injury and continue living life to the fullest.

Wheelchair use has given Race and Ried more freedom. "Before they got their wheelchairs, they couldn't go to the mall, they couldn't go to the zoo and they couldn't go to the store," Mosimann says. "Getting the chairs has really extended their mobility and increased their independence."

Once Race felt his newfound freedom, he gained a new perspective. "The most important things are on the inside," he says. "Focus on the lungs and heart, because you could live without your legs, but you can't live without those organs." Q

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

GET SOME SUPPORT

Perhaps one of the most helpful things you can do for yourself or your child when transitioning to the use of a mobility aid is to find a support network. This might be in the form of a support group or, for children, attending MDA Summer Camp.

"Seeing other children who are in wheelchairs and happy and doing well lets kids know that they are not alone," says social worker Jenny Kean, LCSW. "I think that's why MDA Summer Camp is so powerful for children."

Here for You

Learn how MDA empowers families with services and support to help them thrive and stay independent, including summer camps for kids, support groups, equipment assistance and more, at **mda.org/services**.

get READY

TO ROLL

How to choose a scooter or power wheelchair

BY KAREN HENRY

For many people with neuromuscular disease, there comes a point when walking becomes too difficult, and it's time to start thinking about power mobility aids. This typically boils down to choosing between a mobility scooter and a power wheelchair.

Given the progressive nature of neuromuscular disease — not to mention the financial investment involved with purchasing a power mobility aid — it's important not only to select something that will meet your functional needs now and in the future but also to advocate for features that will provide you with the best long-term quality of life.

MOBILITY SCOOTERS VS. POWER WHEELCHAIRS

Mobility scooters are designed primarily for outdoor use and are steered with handlebars. Power wheelchairs, on the other hand, are designed for indoor and outdoor use, are highly customizable and maneuverable, and are steered with a joystick or an alternative input control, such as a switch or head array.

If you are considering making the move to power mobility, a good first step is to contact your insurance provider and ask what types of power mobility aids are covered by your policy, how much the insurance company will pay for a power mobility aid and how often they will pay for a new one. Most insurance companies cover mobility aids once every five years; however, there are exceptions. Knowing your insurance provider's coverage may help you make your choice.

For example, for someone with a fast-progressing neuromuscular disease, there may be only a short window of time during which a mobility scooter can be used, and insurance may not pay for a power wheelchair less than five years after paying for a scooter. In this case, a power wheelchair may be a more practical choice.

THE MOBILITY CARE TEAM

Your MDA Care Center physician will typically provide you with a referral to get a formal mobility evaluation and seating assessment. "Evaluations are typically led by the primary therapist," says Angie Kiger, MEd, CTRS, ATP/SMS, clinical strategy and education manager at Sunrise Medical in Alexandria, Va.

Kiger advocates for an interdisciplinary approach, where your MDA Care Center physical therapist (PT) or occupational therapist (OT) works in coordination with a complex rehabilitation technology (CRT) supplier who is credentialed as an assistive technology professional (ATP). ATPs are trained in analyzing the needs of people with disabilities, assisting in the selection of appropriate assistive technology and providing training in the use of the selected devices.

Kiger recommends working with a wheelchair supplier who is also certified by the National Registry of Rehabilitation Technology Suppliers (NRRTS). "Working with qualified professionals is key," she says. "The more credentials someone has, the greater the likelihood they have invested in learning their craft and maintaining it."

GETTING AN EVALUATION

A typical mobility evaluation consists of three parts: an interview, a physical evaluation and a feature match. Kiger likens the initial interview to a first date: "We want to make sure we get to know the person as much as possible."

During the interview, the mobility team will ask questions about your neuromuscular disease, your preferences and goals, how your mobility changes as the day progresses, and what your mobility looks and feels like on your best and worst days.

"Being honest about what things feel and look like at 5 p.m. is really important," she says. "The team that's with you in that moment is only seeing you right then. They can't see what your entire day looks like."

After the interview, the team will conduct a physical evaluation. During the evaluation, the PT or OT and the CRT supplier will take measurements and assess your functional mobility.

"The individual will be asked to do various tasks, like sitting on the edge of a mat to check trunk stability and range of motion," Kiger says. "The therapist and suppliers are looking to see if and how much support is going to be needed."

Mobility scooter cushions do not provide a lot of support for the back and neck, so if you need additional support to maintain a healthy posture throughout the day, a power wheelchair with a customized seating system may be more appropriate.

When a family chooses a power wheelchair, Kiger moves on to what she calls a feature match. During this process, specific features are selected based on individual needs. For example, if the client has a form of neuromuscular disease that impacts hand function and fine motor skills, alternative control mechanisms can be designed. For those who are not able to shift their body weight, power seating allows the user to tilt the chair for pressure relief. A headrest is beneficial for those who cannot hold their head in a proper position, and lateral supports can help maintain proper leg positioning.

Vovanti Jones, a 30-year-old physician living with limb-girdle muscular dystrophy (LGMD), uses a mobility scooter and a power wheelchair interchangeably. She prefers using her scooter for

Q&A WITH THE MDA RESOURCE CENTER: FINANCING MOBILITY

When it comes to paying for power mobility equipment, it can be difficult to figure out what options are available beyond insurance. We asked Gina Olson, national director of the MDA Resource Center, about alternative funding sources.

Q *Do insurance providers offer financial assistance outside of what is covered in the policy?*

Some insurance companies have foundations or grants that can help with co-pays. It's important to ask about these programs before purchasing mobility equipment because many won't retroactively pay for equipment.

Q *Where else can I look for help to pay for mobility equipment?*

These resources can be particularly helpful for funding features that insurance typically won't cover:

- Some foundations and grant programs help pay for medical equipment.
- You may have access to state and county waiver programs.
- If you are currently employed or a student, state or local vocational rehabilitation services may be able to help.
- Some hospitals offer financial assistance programs.
- Some manufacturers offer rebates.

Q *Which resources are available to me?*

There's no one answer to this question because resources vary by location and individual information. The MDA Resource Center (**833-ASK-MDA1** or **resourcecenter@mdausa.org**) can help identify funding organizations and programs in your area and find out if there are qualifications you must meet to apply.

work because it's more compact and easier to maneuver. However, her power wheelchair is more comfortable for spending long periods of time seated.

"The power wheelchair is better when you're not walking at all because it gives you a better seating position," she says. "You have more options, like leg elevation and recline."

Jones' power wheelchair seat has an elevate function, which raises her seat so that she can reach items on shelves.

OTHER CONSIDERATIONS

There are a number of practical considerations that help inform the decision-making process, including lifestyle, living environment, transportation issues and finances.

Vovanti Jones uses a mobility scooter in her work as a physician.

"The power wheelchair is better when you're not walking at all because it gives you a better seating position. You have more options, like leg elevation and recline." — Vovanti Jones

"I don't want to show someone something that I know they could never afford. But at the same time, I might not know if they have another source of income that may help them afford something different."
— Angie Kiger

As an assistive technology professional, Angie Kiger helps individuals select power mobility aids.

From an environmental standpoint, you, your caregiver and your mobility team will look at whether the power mobility aid will be used primarily in a home or work environment, indoors or outdoors, and whether the surfaces it will roll on are primarily finished, paved or rugged. If the mobility aid will need to be transported to and from various locations, the team may also ask if you are able to afford an accessible vehicle.

"A full-blown power wheelchair can be difficult to transport if the user does not have the proper equipment," Kiger says, "so if the family is looking to transport the wheelchair, they have to think about a wheelchair-accessible van. They also have to think about things like accessible housing and ramps. If those things are financially out of reach, we might recommend funding programs or resources to assist with getting the proper equipment. Ease of transportation is sometimes what draws individuals toward a mobility scooter. Some can be folded up, making them really convenient for travel.

"It's very important to talk with your team about what is medically appropriate for you," she continues. "Utilizing any device without the proper support or positioning can eventually cause more harm than good."

Your mobility team will present all types of mobility aids and features that are clinically indicated. Depending on your situation, financial considerations may have a significant impact on the type of mobility aid you ultimately select.

BEING YOUR OWN ADVOCATE

The process of getting a power mobility aid can be overwhelming, particularly for those who are newly diagnosed or are getting their first piece of equipment. Working with qualified professionals can help to ensure you get mobility equipment that will meet you or your child's individual needs. Keep in mind that you may need to advocate for features that provide you with the best quality of life and be prepared to fight for them if necessary.

Shannon Mosimann says it took one year for insurance to approve manual wheelchairs equipped with power-assist motors for her 13-year-old twin sons Race and Ried Martinez, who are both living with Duchenne muscular dystrophy (DMD). (See "Learning to Love Your Wheels" on page 16.)

"I went to the doctor and said, 'Look, this is for their quality of life,'" she recalls. "We got them approved, but it was not easy."

If your health insurer does not provide authorization for coverage, you can ask the provider to reconsider its decision. You also have the right to appeal the decision and have it reviewed by a third party. "No" isn't always a final answer. Q

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

Ready to Help

Contact the MDA Resource Center at **833-ASK-MDA1** or **resourcecenter@mdausa.org**. Our trained resource specialists are available Monday through Friday, 8 a.m. to 5:30 p.m. Central time, to answer questions and provide one-on-one support and resources. They typically answer questions within 24 hours of a request, or on the next business day.

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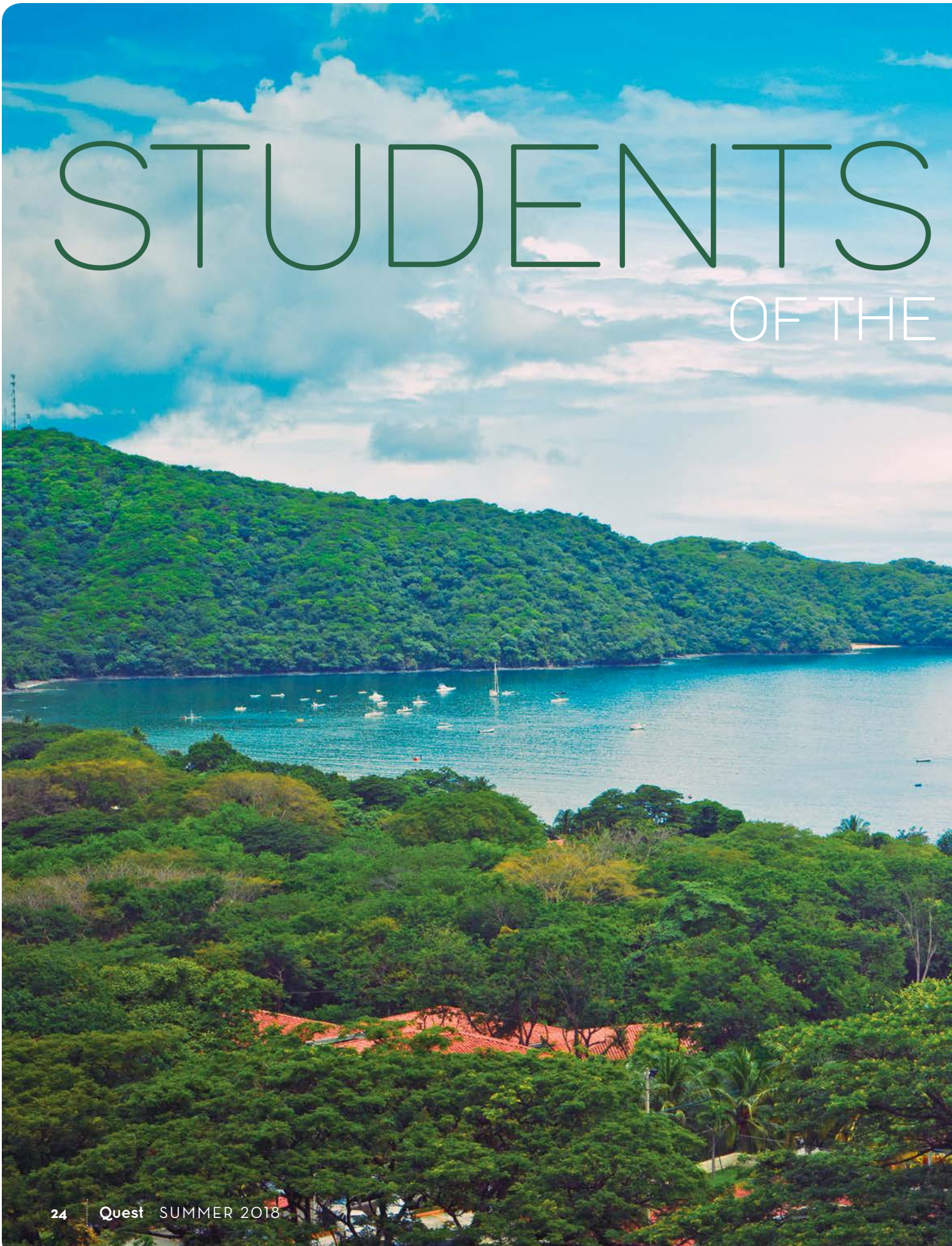
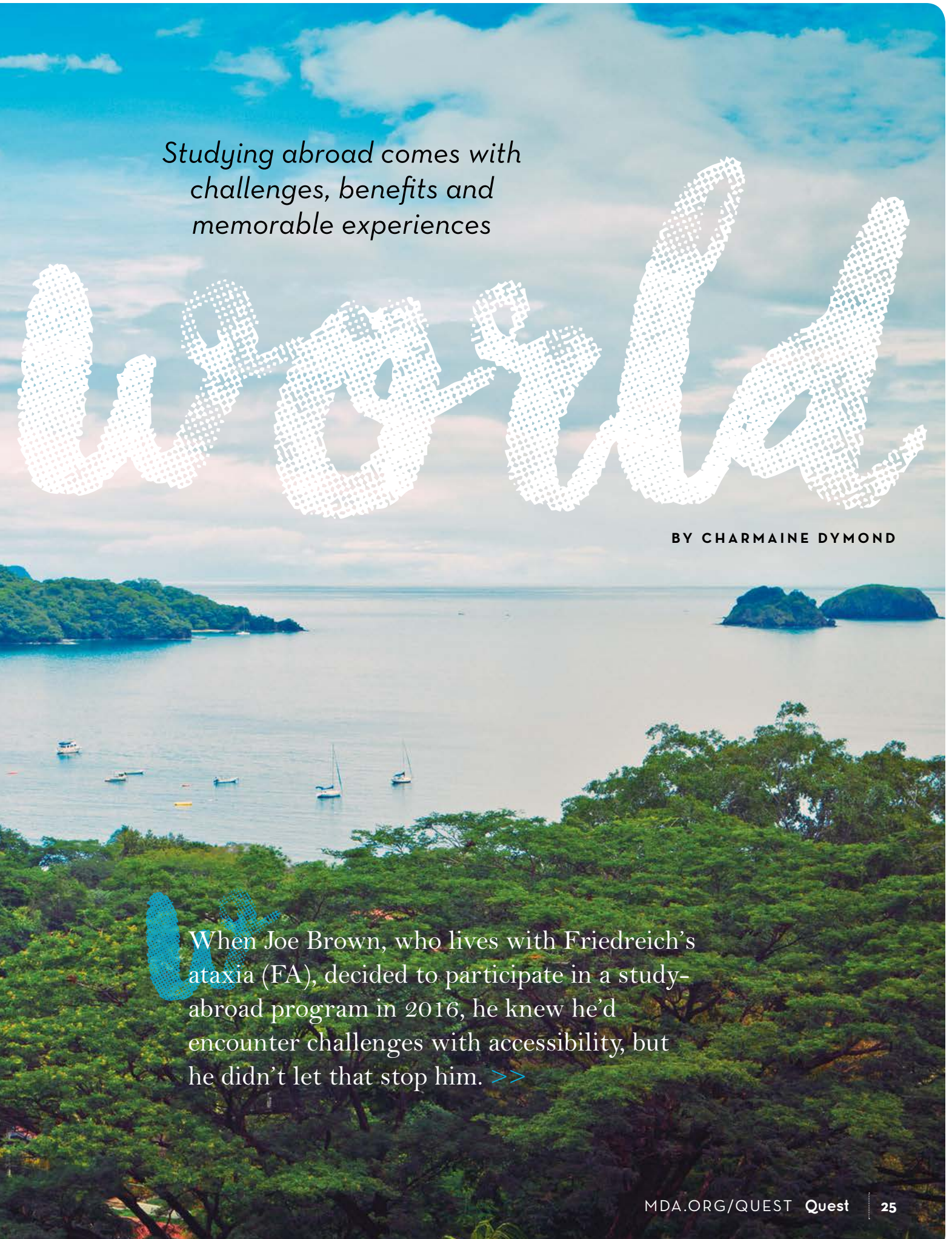


Studying abroad comes with challenges, benefits and memorable experiences

Abroad

BY CHARMAINE DYMOND



When Joe Brown, who lives with Friedreich's ataxia (FA), decided to participate in a study-abroad program in 2016, he knew he'd encounter challenges with accessibility, but he didn't let that stop him. >>

Joe Brown (seated) and his personal assistant visited the Burj Khalifa in Dubai.

"One of my favorite visits was the Dubai campus of Zayed University. We got to see how very similar people of the same age are, even though we are on the complete opposite sides of the world."

— Joe Brown

"On the last day, we were going to go dune bashing and have an evening meal in the desert. Bringing a power wheelchair to the desert is not feasible," Brown says. "The program leaders asked me if I wanted to participate, since I would be without my wheelchair. I think my response was something like, 'Strap me to the roof and put sand goggles on me if you have to!'"

The trip, a weeklong visit to Dubai and Abu Dhabi in the United Arab Emirates, was organized by the University of North Carolina's Kenan-Flagler Business School as part of Brown's undergraduate studies.

From touring the Burj Khalifa, the world's tallest building, to visiting the Sheikh Zayed Grand Mosque, Brown's trip was jam-packed with memorable experiences. "One of my favorite visits was the Dubai campus of Zayed University," he says. "We got to see how very similar people of the same age are, even though we are on the complete opposite sides of the world."

A GOOD MOVE

Hugo Trevino joined his first study-abroad program as an undergraduate student at the University of Illinois. "After I went to Costa Rica for the first time, I just became addicted and I wanted to travel abroad again," he says. He participated in programs in China, Hong Kong and Taiwan as an undergraduate, then continued his global adventures in Vietnam as a graduate student at Loyola University Chicago, which he chose partly because the program requires students to study abroad.

Trevino, who lives with spinal muscular atrophy (SMA), now works as an

WHERE IN THE WORLD WILL YOU GO?

A successful study-abroad trip aligns with your academic interests as well as your abilities and taste for adventure. Answer these questions to find the right experience for you.

- **Academic and personal interests:** What are you looking for in a study-abroad experience personally and academically? What countries, cultures or activities interest you?
- **Accessibility:** How accessible is your host country? Will your disability prevent you from participating in certain events, and if so, how will you feel about that?
- **Comfort level:** How adventurous and flexible are you? Would you prefer an English-speaking country? What are your must-haves?
- **Cost:** How will you pay for it? Will you need to pay for extra equipment or a personal assistant's travel and living expenses? What funding is available?
- **Credit:** Will your studies be accepted for academic credit at your university?
- **Health:** What access to medical services do you need, and can your host country provide that? Will you need to bring prescription medicine with you? How might living abroad affect your health and well-being?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Support for Young Adults

MDA is committed to supporting young adults with neuromuscular disease as they pursue education and seek to live beyond limits. Learn about our resources and how to connect with the young adult community at **mda.org/young-adults**.

MDA resource coordinator, and he feels that his study-abroad experience has helped him find jobs. "One of the things I always say whenever I'm getting interviewed is, 'If I was able to study abroad in all of these countries, I'm not going to have a hard time just coming into work every day.' They laugh about that."

Studying abroad helps students develop useful professional and personal skills, such as adaptability, communication, cultural awareness and confidence. This can help launch careers, according to Matthew Rader, assistant vice president of student affairs and dean of students at IES Abroad, which runs study-abroad and internship programs around the world. "Career benefits of studying abroad include increased hireability, jobs secured more quickly after graduation, higher starting salaries and higher acceptance into grad school," he says.

Hugo Trevino has participated in multiple study-abraod programs, inclulding this trip to Hong Kong.

"One of the things I always say, whenever I'm getting interviewed, is, 'If I was able to study abroad in all of these countries, I'm not going to have a hard time just coming into work every day.'"

— Hugo Trevino

PLAN IT OUT

If you're interested in studying abroad, the first step is to talk with your university's study-abroad office, which can advise you on credit transfer options, financial aid and application processes.

"My advice would be to not go about it alone," says Brown, who depended on the study-abroad department to do the bulk of the planning. "My school had tour guides on the ground in Dubai, and they found out things for us like what is accessible and where to get needed medical equipment. They had plywood ready at one place that did not have a ramp."

Mobility International USA (MIUSA) can help with this planning. Its National Clearinghouse on Disability and Exchange, sponsored by the U.S. Department of State's Bureau of Educational and Cultural Affairs, offers a web resource library with tip sheets and success stories, as well as a free information and referral service.

"People have connected with our office to understand how they can study abroad with different types of disabilities, including physical and sensory disabilities," says Monica Malhotra, MIUSA program manager. "We can offer support and resources to both the student and programs on how they can prepare for an inclusive program. This includes identifying various funding options, connecting with disability organizations abroad and tips for using personal assistants abroad."

ADVENTURES AWAIT

Your university's study-abroad office should be your first stop for information, but these resources can also help:

- Abroad With Disabilities empowers students with disabilities to go abroad and offers a scholarship. **abroadwithdisabilities.org**
- Diversity Abroad is a global community of diverse students and programs. **diversityabroad.com**
- IES Abroad runs study-abroad and internship programs around the world. **iesabroad.org**
- Mobility International USA (MIUSA) offers support and advice for studying abroad with a disability. **miusa.org**
- The U.S. Department of State's Bureau of Educational and Cultural Affairs offers scholarships and exchange programs. **eca.state.gov**

FACING CHALLENGES

Each journey comes with its own challenges. For Trevino's trips to Asia, for example, he decided to swap his power wheelchair for a manual one — lighter and foldable — after being warned of accessibility issues.

Overall, his experiences were positive. "I have zero regrets and feel lucky to have been able to experience every single trip," he says. But Vietnam proved to be more inaccessible than Trevino had expected, and it began to take a physical toll on his personal assistant. Trevino had to explain to his professor that though his assistant was there to help make things accessible, he had limits.

"He's not a robot. If he ends up hurting his back, the trip will be over for me," Trevino says. "We had to turn down some of the visits toward the end of the trip."

Brown also had to miss a scheduled class outing because of accessibility problems, but for him it resulted in a positive experience when he was able to arrange a visit to the stadium of the Al-Nasr Sports Club instead. He was even able to meet with a marketing representative for the soccer team, a perfect fit for his academic interest in sports management.

"Dubai is fantastic, but it personally made me feel lucky to live in the United States given my disability," Brown says. "I expected some challenges as far as accessibility, and I got them. But I had a blast regardless." Q

Charmaine Dymond is a freelance writer in Halifax, Canada.

MEET MDA'S NATIONAL ambassadors

BY CHRIS ANSELMO

Faith Fortenberry

There was a palpable buzz in the air on March 12 at the kickoff of the 2018 MDA Clinical Conference in Arlington, Va. The announcement of the new MDA National Ambassador was one of the most-anticipated elements of the day, and the crowd was excited to learn that this year, MDA has not one but two National Ambassadors: 6-year-old Faith Fortenberry of Waco, Texas, and 17-year-old Justin Moy of Concord, Mass.

These poised young spokespeople represent individuals and families living with neuromuscular disease

The new ambassadors took the stage without a hint of nervousness in front of an audience of more than 700 people, many of whom were clinicians and researchers. Faith's mother, Leeann, addressed the crowd first, sharing Faith's journey with spinal muscular atrophy (SMA) and how her life has been positively affected by Spinraza, one of the breakthrough drugs approved by the FDA in recent years. When her mom finished, Faith introduced herself and thanked the crowd for helping kids like her.

Next, the audience enjoyed a video from the 2012 MDA telethon featuring a 12-year-old Justin. Afterward, Justin took the stage and threw out a quip about how much he has grown up since that telethon. Although he started with a moment of levity, the rest of Justin's speech left the room breathless. He shared his personal story about how he chooses to ignore the limitations of living with congenital muscular dystrophy (CMD), and how he looks forward to the opportunity to inspire others and spread hope throughout the country.

By the end of their introductions, it was clear to everyone in attendance that Faith and Justin would make ideal ambassadors.

Justin Moy

MDA'S SIGNATURE PROGRAM

Faith and Justin, MDA's 41st and 42nd National Ambassadors, continue the tradition of MDA's signature program. They take over for Joe Akmakjian, who served a two-year term as MDA's first young adult National Ambassador.

MDA National Ambassadors play an essential role in representing the thousands of people living with neuromuscular disease and spreading the word of MDA's life-saving mission. Throughout the year, they'll travel around the country to forge better connections with MDA families, engage with

FUN FACTS ABOUT FAITH AND JUSTIN

FAITH FORTENBERRY

1. She loves to play softball, cheer, sing and dance.
2. Her favorite school subject is math.
3. If she has a favorite chore, it would be loading the dishwasher.
4. Her favorite food is mac and cheese.
5. The place she is most excited to visit as a National Ambassador is New York City.

JUSTIN MOY

1. He collects pins.
2. He has traveled to 39 states and six countries, his favorite of which is Thailand.
3. His favorite food is sushi.
4. He enjoys having fun outdoors, especially adaptive biking and skiing.
5. He loves to sing. This year, he was in his high school's production of "Guys and Dolls."

Faith Fortenberry's bubbly personality comes through on a trip to Disney World in 2017.

MDA's partners and champion improved services for individuals living with neuromuscular disease. In addition, Faith and Justin will share their experiences with the MDA community in blog posts and on social media.

"We are honored to have Faith and Justin, and their families, represent MDA as National Ambassadors this year," says MDA President and CEO Lynn O'Connor Vos. "These young individuals have incredible and different stories to share. I know that they will do a fantastic job for MDA during this exciting time in our organization's history, as we seek to build on the momentum of recent therapeutic successes, further our mission to provide the best quality of care and empower individuals and their families to Live Unlimited."

FAITH FORTENBERRY

Many MDA Clinical Conference attendees met Faith the night before the announcement, when she sped around the ballroom of the Hyatt Regency Crystal City in her power wheelchair handing out cups of water to passersby. With her jovial personality and boundless energy, Faith quickly made friends. "She loves talking to new people," Leeann says.

Faith lives in Waco with her parents, Leeann and Don. Diagnosed with SMA at 17 months, Faith has used a power wheelchair from a young age. As she grew, Faith had limited use of her arms and was frequently hospitalized with respiratory infections. That all changed last May, when Faith began receiving treatments of Spinraza, the only FDA-approved drug for SMA. The results have been dramatic.

"Our little girl went from being hospitalized every three months — we almost lost her the first time — to no respiratory stays since she started Spinraza," Leeann says. Faith can now open her markers at school, roll over in bed and pull herself up in her stander, things she could never do before. "She has so much more independence," Leeann says.

Spinraza, which was the product of years of MDA-backed research, has helped redefine what is possible in Faith's life. "You can always do anything that you want to do," Faith says.

Spinraza has given kids like Faith this message of hope that the Fortenberrys are excited to share with MDA partners throughout the country.

JUSTIN MOY

MDA's Live Unlimited motto is a core ideal of Justin's life. The recent high school

A chance to advance DMD therapy

Join a study of respiratory function in Duchenne muscular dystrophy (DMD).

The *SIDEROS* study

is a clinical trial that will study whether a therapy called idebenone is safe and effective at delaying the loss of breathing function in boys and men with DMD.

The study will compare the efficacy of idebenone to placebo in those currently on steroids (either prednisone or deflazacort).

The therapy

Idebenone is an oral tablet developed by Santhera Pharmaceuticals, a specialty company focused on developing novel treatments for DMD.

Who can participate?

- Males with DMD, any mutation
- Age 10 or older
- Ambulatory or non-ambulatory
- On corticosteroids for at least 12 months
- Forced Vital Capacity between 35% and 80%

Find out more about the study or who can participate at SiderosDMD.com or by emailing us at sideros@santhera.com.

santhera

Keep Up with Faith and Justin

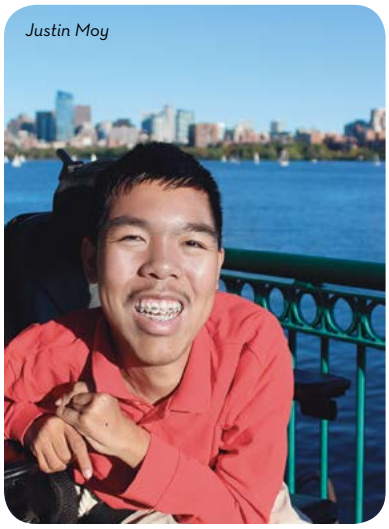
During their year as National Ambassadors, Faith Fortenberry and Justin Moy will share their experiences with the MDA community. Keep up with them by connecting with MDA on Facebook (**facebook.com/MDANational**), Twitter (**@MDAnews**) and Instagram (**mda_usa**). Also, look for their updates on the Strongly blog at **strongly.mda.org**.

graduate will be attending college in the fall with the hopes of becoming a researcher.

"I want to become a biochemist," Justin says. "I want to go into academic research or work for a pharmaceutical company so that I can help find a cure for my disease."

Justin was diagnosed with CMD at the age of 6 months and has always needed a wheelchair to get around, but he has never looked at life in terms of what he cannot do. This attitude is a testament to his parents, Prow Sarnsethsiri and Chris Moy, who have always encouraged him to dream big. "He can put forth a message that with our challenges we can still live life and pursue opportunities," Prow says.

MDA has been a major factor in Justin's life, providing him and his family with resources and support, along with exciting opportunities such as MDA Summer Camp. "Summer Camp is one of the best experiences," Justin says. "It provides independence and was a great opportunity for me to meet other kids with conditions similar to mine."


Justin Moy

At MDA Summer Camp, Justin met counselor Alex Funez, who became his good friend. Recently, Alex pushed Justin in a half marathon and accompanied him on a trip to Japan.

As a National Ambassador, Justin is excited to use his platform to empower others. "It is my belief that anyone, no matter their abilities, should take advantage of every opportunity that comes their way," he says. Q

Chris Anselmo is a Connecticut-based freelance writer living with Miyoshi myopathy. He chronicles his journey with the disease at ***sidewalksandstairwells.com****.*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SPINRAZA® (nusinersen) injection 12 mg/5 mL

The first and only treatment for children and adults with spinal muscular atrophy (SMA)[1]

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

INDICATION

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

IMPORTANT SAFETY INFORMATION

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

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

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

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

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

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

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IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

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

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

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

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

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

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

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

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

Chipping In

A conversation with PGA Tour golfer Morgan Hoffmann about his muscular dystrophy diagnosis

Morgan Hoffmann

In 2017, PGA Tour golfer Morgan Hoffmann surprised the sports world by revealing that he had been diagnosed with facioscapulohumeral muscular dystrophy (FSHD). Hoffmann made the announcement in an article for The Players' Tribune, where he reflected on his career, his feelings about the diagnosis and his thoughts about the future. Recently, *Quest* followed up with Hoffmann.

What was your awareness of neuromuscular disease before you were diagnosed?
My awareness of muscle atrophy began in 2012 right after I left college. It started with my right pectoral atrophying from my sternum toward the underarm. The atrophy was small in the beginning, maybe 1-by-2 inches. For the next five years it progressed throughout almost the entire right pectoral. During that time I traveled to countless doctors and specialists in North America. They conducted MRIs, EKGs, EMGs, nerve concussion tests, CAT scans, a stomach biopsy, etc. Long story short: No one could find a cause or a diagnosis.

Why did you decide to share your experience?
I decided to write The Players' Tribune article because I knew that I could help and reach many people with this

"incurable" disease. I figured if I kept to myself it would be very selfish. With my platform, I knew I could make a difference, and The Players' Tribune was very supportive in making my message heard. The reason I have "incurable" in quotes is because I believe it most certainly is curable, we just need to search in the right places.

What kind of feedback have you received since revealing your diagnosis?

I have been so lucky with the most incredible feedback from writing the article and announcing my health condition to the public. I am so humbled and amazed with how nice and supportive people from all ends of the earth have been. Countless families, friends and strangers contacted me through email, text, calls and social media. I'd say one-quarter of them either have a neuromuscular disease or know someone close to them who has been diagnosed. Many individuals have told me their stories and offered advice with how to cope. Some have even come forward for the first time, electing me to be the first to know. I have made sure to reply and offer my advice, comfort and nutrition beliefs to every single person who has reached out to me.

How have your fellow golfers reacted?

My fellow golfers have been a very good support system. We hosted our first Morgan Hoffmann Foundation event at the Palm Beach Boat Show several months ago and many of them showed up and even bid on our auction items. It is really cool to see how our PGA Tour community is so helpful, philanthropic and caring.

How important are family and loved ones in keeping you going?

I have been extremely lucky with the friends I've grown close to over the years. I don't really call them friends anymore; they are now my family. I am so grateful to have an amazing support system from my whole family. The energy and love that I have received has been out of this world. It is a huge factor in keeping me going to find a cure, make a difference and shine light on neuromuscular disease.

Learn more about the Morgan Hoffmann Foundation at morganhoffmann.org. To learn about MDA's research across diseases to uncover breakthroughs for treatments and cures, visit mda.org/research.

A Room of His Own

A high school graduate with DMD gets the voice-activated room of his dreams

Robbie Ivey's bedroom doesn't just display his interests; it listens to his voice.

"It helps me to not be so needy with things around here. I can basically stay up as late as I want, and it allows me to do things at my pace."
— Robbie Ivey

When Robbie Ivey, a 19-year-old soon-to-be college student from Iron River, Mich., and his mother, Carrie, first met Bill and Deb Weis, they had an idea but they didn't know how to make it happen. They explained to the couple that they were looking for a way for Robbie to live more independently with Duchenne muscular dystrophy (DMD) by operating powered objects in his room, like the lights, TV and bed.

"We asked if they knew anyone who had that capability," Carrie says. "Bill called us a couple days later and asked if we wanted to meet and if we could bring Robbie's bed remote. I found out he worked for Microsoft for 35 years."

Bill had recently retired from a 40-year career in the computer industry, and as a longtime MDA supporter, he was the perfect person to take on this project. After visiting with Robbie and getting an idea of his needs, Bill created a voice-activation system for Robbie's room in less than two weeks. He customized consumer devices such as Amazon Echo, Google Home, Google Chromecast and others to make it work. The setup allows Robbie to use his voice to operate his TV and apps like Netflix, change the position of his bed, control a fan, turn his lights on and off, and even make phone calls.

"It helps me to not be so needy with things around here," Robbie says. "I can basically stay up as late as I want, and it allows me to do things at my pace. And it allows my mom to take a break, too, and not be in here every five minutes helping me with stuff."

The technology came at a perfect time for Robbie, as he is preparing to start college at Oakland University in Auburn Hills, Mich. In the fall, Bill will go with Robbie's family to re-create the voice-activation system in his dorm room. As a passionate Green Bay Packers fan, Robbie intends to study business and sports management with a goal of one day being the general manager of the Packers.

While Robbie has a lot on his plate these days, he still takes time to attend MDA events. Last year, he worked with Bill and Deb to bring the first Toast to Life Upper Michigan MDA Gala to life. From being a former MDA Ambassador for Upper Michigan and Northeastern Wisconsin to attending Muscle Walks to making lifelong friends at MDA Summer Camp, Robbie and his family have seen the positive effect MDA has, even beyond introducing him to Bill and giving him a more independent lifestyle.

For more detail about Robbie Ivey's room, read Bill Weis's blog post on strongly.mda.org. Visit limitedmobility.solutions to see more of Bill's projects and learn about bringing voice-activation technology to your home.

Bill Weis and Robbie Ivey (seated)

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Fishing for a Cause

This fishing team hopes to hook a cure for neuromuscular disease

FFMD boats proudly display the MDA logo.

Paul Robertson has a clear goal in mind when he dreams about the impact his organization, Fishing for Muscular Dystrophy (FFMD), can achieve in the future.

"Ultimately, we want to be responsible for a $1 million-per-year contribution to MDA," Robertson says matter-of-factly. Although ambitious, it is a goal that FFMD is successfully building toward as it establishes itself as a fundraising powerhouse in the fishing industry.

Paul Robertson (above right and below left) has turned his passion for fishing into a fundraising powerhouse.

Robertson's commitment to MDA is a personal one: The 50-year-old has been living with Limb-girdle muscular dystrophy (LGMD) for many years, experiencing progressive muscle weakness, especially in his legs. The day after Christmas last year, Robertson suffered a broken pelvis in a fall, but he was determined to heal so he could get back in the captain's chair as soon as possible. "I missed the January tournament but made our February one," Robertson says. "I was itching to get back."

At its core, FFMD is a fishing team of six to 10 people who compete in offshore fishing tournaments throughout the country on a boat proudly adorned with the FFMD and MDA logos. The team has fished in more than 50 tournaments in the last two years and has reached the podium 12 times, winning as much as $41,000 in a single tournament. A share of the winnings goes to MDA. "I like to say that we are one fish away from hitting the big six-to-seven-figure paydays," Robertson says with a laugh.

Another FFMD effort is a boat raffle to benefit MDA. In the most recent raffle, a 24-foot boat was generously discounted by manufacturer Everglades Boats, who also built and donated a number of the boats the FFMD team uses in its tournaments. The team raised $200,000 in gross proceeds from the raffle by selling tickets at boat shows, events and online.

In addition, Robertson has co-chaired the Washington, D.C.-area Muscle Team Gala, with his FFMD employees and contacts playing a big role in making the fundraiser a success. In two years, the gala has raised more than $900,000 for MDA.

Although a fundraising force, Robertson most enjoys the impact FFMD has had on children and families with neuromuscular disease. "We've had multiple events where we've had MDA families attend," Robertson says. "When I take a child out on the boat and see the smile on their face, when I can tell them there's hope and that you can do anything you put your mind to, it's just a really neat thing."

Looking toward the future, Robertson sees FFMD's current impact as just the beginning. "When I take a step back and I take a look at what we've done in two years, I know we've done a lot," he says. "But I am a raise-the-bar kind of guy and think we have still a long way to go."

From forming a fishing team to hosting a bake sale, you can fundraise your way to help kids and adults living with neuromuscular disease. We'll give you the tools to make the fundraiser a success and cheer you on every step of the way. Learn more at mda.org/yourway.

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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 consumer brief summary of the full FDA-approved product information on the next page.

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**.

PTC THERAPEUTICS™

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**.

Boots Filled

IAFF celebrates its 100th anniversary

Fire fighters with boots in hand collecting money to help kids and adults living with muscular dystrophy are some of the most iconic images associated with MDA fundraising. Since the partnership between MDA and the International Association of Fire Fighters (IAFF) began in 1954, Fill the Boot drives have raised more than $630 million, making the IAFF MDA's largest fundraising partner. This year, IAFF is celebrating its 100th anniversary.

Fire fighters meet some of the kids impacted by their Fill the Boot drive.

Jessica Hetzel, MDA's 2016 Michigan State Ambassador, visits Warren Fire Fighters Union Local 1383 Station 2.

A few decades after its founding in 1918, the IAFF became a key partner to MDA. While the overall effect of the IAFF's fundraising efforts for MDA can't be overstated, one of the most important aspects of Fill the Boot is how it connects fire fighters to their local communities. Kevin Faddis, Union President of Local 065 in Knoxville, Tenn., has seen the impact Fill the Boot has had in his 22 years in the union and eight years as its president.

"We're supposed to be a labor organization, but I always say that we're part of the community because we're out in the community working," Faddis says. "Fill the Boot is part of >

that. The cause hits close to home because we've got a member whose son has muscular dystrophy and we've helped him throughout the years, but we also see who it impacts at conventions." Their annual Fill the Boot fundraiser brings in $25,000 to $30,000 in just one day.

"I'm proud to be a part of an organization where we put people before ourselves every day," Faddis says. "We're working to help people in the community, and that goes hand-in-hand with MDA fundraising."

IAFF's 100th anniversary gives Faddis a sense of pride because Local 065 was one of the original unions in IAFF. Faddis recently visited Washington, D.C., to re-create a photo of the first Local 065 union president taken at IAFF's founding in 1918.

"He went to Washington, D.C., [from Knoxville] in 1918 with no interstates because he believed in something," Faddis says. "That's why we try to honor the people who went before us."

For more information about Fill the Boot and MDA's partnership with IAFF, visit firefighters.mda.org.

IAFF Indianapolis Fire Department Local 416 kicks off a Fill The Boot campaign.

Aging (Sort of) Gracefully with SMA

A writer reflects on the lessons and challenges of middle age

BY MICHAEL P. MURPHY

I'm still a night owl whenever possible … I still listen to old-school punk and speed metal (the Sex Pistols, the Clash and Motörhead are on heavy rotation).
— Michael P. Murphy

Recently, when a new homecare nurse arrived for training, Dad looked out the window and said, "She's a young one."

"Well, Dad," I replied, "We're at the point where everybody's young." Despite my sarcasm, there was more than a little truth in this statement.

Once I turned 45, evening nurses started putting me to bed at 11 p.m., after I had spent decades writing until the wee hours of the morning with the lights turned down and music blasting in my headphones, and then reading a few chapters of a book before turning in. I rebelled at the curfew, and I managed to sneak in the occasional all-nighter, but the next day I'd be running on fumes. This was my first indication that I'm not a young man anymore.

I'm 51 now, which is barely considered middle-aged nowadays. To a ventilator-dependent quadriplegic with type 2 spinal muscular atrophy (SMA), type 1 diabetes and one functioning lung, however, that's quite an accomplishment.

First and foremost, I attribute my longevity to the love and dedication of my dear parents, who've sacrificed pretty much everything to keep me healthy and happy, and who continue to care for me as much as possible even though they're not getting any younger either. Next come improvements in ventilator technology to keep my weak lung pumping.

Despite these advantages, I can't deny that my body no longer performs as well as it used to. Gone are the days when I could be in my wheelchair for 10 hours at a stretch without batting an eye, as was common during family vacations "up North" (you have to be from Wisconsin to understand) or when I attended science-fiction conventions. Ah, those were the days!

Now, after only four or five hours of being strapped into one position, my back,

shoulders and neck beg me to stop the torture and I'm forced to stretch out in bed, where I feel drained to quarter-strength for the rest of the day. It's taken me years to accept the fact that there's only one way to deal with these limitations: Surrender to them.

I'm Irish, so "surrender" isn't in my DNA, but when one's adversary is an aging body further weakened by a relentless neuromuscular disease, the only way to keep the machine running smoothly is to know when to throttle back and allow the engine to cool. I've tried repositioning every half-hour, but movement only causes my pain levels to spike. I had a chest X-ray recently, and I felt as if I'd been hit by a truck after just a few minutes of lying on top of the film plate. SMA will not be tested.

Age certainly hasn't granted me any great wisdom; I'm still the happy hedonist that I've always been. But I'm more comfortable with these new limitations because technology affords me pleasant — or at least acceptable — alternatives to many of the things I enjoyed in my younger days.

Can't fall asleep at bedtime? I'll settle in and listen to an audio book.

Can't attend that science-fiction convention? I'll join in the discussion with fellow fans on social media.

Can't make it to the theater to see that new superhero movie? I'll stream it in a few months. That's cheaper and easier, anyway.

This last fact is rather disturbing to me: I find myself enjoying television, which I've always considered the last resort of the bored and unimaginative. When not writing, reading will always be my favorite pastime, but I must admit that it's also relaxing to kick back in bed and watch a movie or binge watch a TV series. With streaming devices, it's now possible to watch whatever you want whenever you want and not be at the mercy of what the networks decide to show at any particular time.

Simple steps like these allow me to function at near-peak performance most of the time. I also take pride in being mature enough to adapt to my limitations and not do anything that will set me back for days.

Let me be clear: I am not, by any stretch of the imagination, ready to join the Hallmark Channel and Geritol club just yet. I'm still a night owl whenever possible, I still read and collect comic books ("Batman," "The Punisher" and "Hellboy" are among my favorites) and I still listen to old-school punk and speed metal (the Sex Pistols, the Clash and Motörhead are on heavy rotation). Because kids today seem to listen only to rap (yeesh!) and corporate-engineered pop divas and boy bands (blech!), my musical tastes make me an old man, a label I wear like a badge of honor.

My hair is pretty much a memory now, my beard seems to turn grayer by the day and my voice — when it even works — is reduced to little more than a raspy whisper. Considering the fact that I've spent my life hounded by a

Michael P. Murphy

neuromuscular disease that's determined to kill me, though, these are small prices to pay. Like my Nana always said, "It's hell getting old, but it beats the alternative." Q

Michael P. Murphy is a writer with several self-published, electronic works of science fiction, horror, thriller, action/adventure and poetry to his credit. He lives with his parents in Wisconsin.

Get Personal

Read stories from around the MDA community — from personal perspectives to research news — on MDA's Strongly blog, at **strongly.mda.org**. If you're interested in sharing your story, contact us at **strongly@mdausa.org**.

Bringing It Home

Philadelphia Phillies slugger Rhys Hoskins reconnects with MDA

Above: Rhys, Jayme and their dog Rookie greet MDA families at a Muscle Walk.

Above and lower right: Rhys Hoskins (in red) and his girlfriend, Jayme (to his left), meet with MDA families before a Philadelphia Phillies game.

Philadelphia Phillies left-fielder Rhys Hoskins burst onto the Major League Baseball scene in 2017, ending the season with 18 home runs in 50 games. Taking his newfound celebrity to heart, Hoskins wanted to make a difference, and he decided to partner with MDA because its cause is close to his heart.

In high school, Hoskins volunteered at MDA Summer Camp and bonded with his camper, Charlie.

"It turned out to be a great idea," he says. "It really hooked me. I raved so much about the camp to my girlfriend that she joined me the next year as well."

Seeing firsthand how MDA Summer Camp had such an impact on the campers — not to mention the counselors — inspired Hoskins to make MDA his charity of choice.

Since making his MLB debut in August 2017, Hoskins has made a tremendous impact for MDA, participating in local events and befriending several MDA families along the way. In May, he emceed the MDA Muscle Walk of the Greater Philadelphia Area. Leading the event and sponsoring a Muscle Walk team was an opportunity to continue his support for an organization that provided him with so many wonderful memories as a teenager.

"What has stuck out for me working for MDA is the relationships I've built through the organization," he says. "The kids are awesome. The families are awesome. The relationships I fostered with some of the counselors and kids when I was at Summer Camp still mean a lot to me. And above all, being able to bring joy to these kids is so cool to see." Q

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CMS Patients

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

MuSK-MG Patients

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

LEMS Patients

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

Learn More

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

Catalyst Cares

A pharmaceutical company focused on developing treatments for rare neuromuscular and neurological diseases, Catalyst is dedicated to providing education to patients and physicians. For more information about our work, please visit **www.catalystpharma.com**.

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