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WINTER 2017

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IN GOOD HANDS

Finding child care you can trust

students

Speak Up

Taking on accessibility challenges in higher education



MOVING FORWARD
Celebrating FDA
approval of Spinraza

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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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Moving Forward Together in 2017

In my four years as President and CEO of this remarkable organization, I have never been more excited than I am about 2017.

As I think about the year ahead, I'm reminded of how I felt at mile 10 of the Denver Half Marathon last fall. I ran as part of MDA Team Momentum and raised funds to support our mission. My legs and hips were aching, and I was moving at a slower pace than I wanted. But when I looked down at the honor ribbons pinned to my shirt, I knew I could fight through pain and fatigue to push forward.



My inspiration for that race — and for fighting on for MDA — are all the individuals and families counting on us. These past few years, we have repositioned MDA for leadership and impact. Our Mission 2020 plan, our new branding and messaging, and our integrated and modernized fundraising are all in the service of helping MDA “go the distance” in the fight against muscular dystrophy, ALS and related muscle-debilitating diseases.

This year, we will build on the tremendous progress made in 2016 — from seeing the first drug to treat the underlying cause of Duchenne muscular dystrophy and the first drug to treat most kids and adults with spinal muscular atrophy approved by the FDA, to launching MDA's National Resource Center for families and developing new programs and services for young adults in our community.

Here are a few of the things you can expect from MDA in 2017:

- Enhanced multidisciplinary care at MDA Care Centers, along with new quality improvement initiatives, more clinical trials and other programs to give families the best care from day one.
- New research funding for projects that will speed drug development and clinical trials to the market, as well as our March Scientific Conference bringing together leaders in the field to share their research.

- A new clinical trial finder tool on mda.org.
- New offerings for young adults, including one-on-one coaching sessions, an education series, interactive online tools and more.
- Our first public policy and advocacy conference in April that will bring dozens of MDA family members together to advance policies to benefit the neuromuscular and disability community.
- A new national community advisory committee that will inform and help guide MDA's programs, offerings and direction.

We know success comes one person at a time. Thanks to families, corporate sponsors, donors, event participants, volunteers and staff, we have a strong team that enables our progress.

With the start of the new year, I encourage you to find what you are passionate about and get involved in the MDA community in a way that is meaningful for you. There are so many opportunities — from attending a conference to signing up for a Muscle Walk, volunteering with your local office or adding a donation to a fire fighter's boot — that can enable you to channel your efforts and energy into hope and progress for MDA families. You may even try something new and defy a limit you didn't think was possible.

We at MDA continue to ask for your partnership, volunteerism and financial support to make our shared progress possible.

Together, we will continue to push for cures, care for families and champion our mission to help everyone live longer, grow stronger and continue living unlimited. Together, we move forward. And, together, we will cross our finish line.

Steven M. Derks
President and CEO
Muscular Dystrophy Association

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Support MDA
Shamrocks

MORE ONLINE

READ STRONGLY

Find personal stories from people living with neuromuscular diseases, research news, fun videos and more on Strongly, the MDA blog. Visit strongly.mda.org.

SHARE YOUR STORY

The Strongly blog is home to stories from all around the MDA community. If you're interested in sharing your story on Strongly, contact us at strongly@mdausa.org.

On the cover: Dimpal Patel

MDA-07



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The Latest from the Lab

You probably enjoy visiting strongly.mda.org to read personal stories from people living with neuromuscular diseases – but did you know you'll also find updates and information straight from the mouths of the researchers who are working to uncover scientific and medical breakthroughs? Here are some excerpts from Strongly.



Current research

“Based on my recent exciting results, I will investigate the mechanism of how vitamin B3 can boost mitochondrial health to prevent the pathology in FA [Friedreich’s ataxia]. ... [This] could lead to novel and accessible therapeutic strategies.”

– from “Five Questions with Friedreich’s Ataxia Researcher Michael Huang”



Encouraging results

“We are happy to continue to provide exciting news about the Spinraza (nusinersen) program. ... Every day, we are spurred on by the stories we hear from the SMA community and are very grateful to all the families participating in our clinical studies as well as the physicians who work tirelessly to care for them day in and day out. We will continue our relentless pursuit of a treatment option for the community.”

– from “Biogen and Ionis Pharmaceuticals: A Message to the SMA Community”



MDA in the Lab

MDA funds cutting-edge research aimed at finding treatments and cures for neuromuscular diseases. Search mda.org for a particular disease or visit our Grants at a Glance page (mda.org/gaag) to learn more about the projects we’re funding across the United States and around the world.

MDA research grant recipient

“Funding from MDA will enable my lab to perform [my] research that is important for human health, and for potentially alleviating the suffering of innumerable patients affected by motor neuron disease. Without this funding, our work, and the critically important work from many other laboratories, would not be possible.”

– from “Five Questions with Researcher Steven Markus”



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References: **1.** Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guideline. *Genet Med.* 2006;8(5):267-288. **2.** American Association of Neuromuscular & Electrodagnostic Medicine. Diagnostic criteria for late-onset (childhood and adult) Pompe disease. *Muscle Nerve.* 2009;40(1):149-160. **3.** van der Beek NA, de Vries JM, Hagemans MLC, et al. Clinical features and predictors for disease natural progression in adults with Pompe disease: a nationwide prospective observational study. *Orphanet J Rare Dis.* 2012;7:88.

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progress **now**

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

ALS (amyotrophic lateral sclerosis)

Edaravone Under Review

Drug targets the oxidative stress pathway

UNDER REVIEW

If approved, edaravone would be the first drug approved to treat ALS in the U.S. in more than 20 years.

Osaka-based Mitsubishi Tanabe Pharma's new drug application for the drug edaravone (brand name Radicava) to treat people with ALS has been accepted by the U.S. Food and Drug Administration (FDA).

An FDA decision on the drug is expected by June 16, 2017. Edaravone was approved last year in Japan and South Korea for the treatment of ALS and has been approved for the

treatment of stroke since 2001.

Edaravone is thought to work by relieving the effects of oxidative stress, which has been suspected to play a role in the death of motor neurons in people with ALS. (Oxidative stress is an imbalance between the production of free radicals and the ability of the body to counteract or detoxify their harmful effects with antioxidants.) Targeting this pathway could potentially preserve motor neuron health, which

could, in turn, keep muscles functional for a longer period of time.

MDA has supported research investigating the role of oxidative stress in ALS and other neuromuscular disorders as well as therapeutic approaches to combat the damage caused by free radicals.



To read about MDA's current ALS-related research efforts, visit mda.org/gaag.



Data suggest a diet high in fruits and vegetables may be helpful for people with ALS.

Nutrition and ALS

COSMOS study results suggest a healthy diet could have positive benefits on overall function

Although ALS can be caused by genetic factors, there is growing interest in the potential influence of the environment and nutrition in ALS disease progression. Now, results from a study called COSMOS, conducted in 302 people with ALS, suggest that intake of antioxidants, carotenes, fruits and vegetables are associated with improved function at the time of diagnosis, and indicate that people with the disease should strive toward fruit and vegetable consumption when possible.

Consumption of these foods is predicted to combat a process called oxidative stress, which is thought to play a role in ALS.

The results yield important insights into the best care for people currently living with ALS, with the suggestion that a healthy diet may improve ALS patient function. In addition, they build on the body of evidence showing the importance of the nutritional care of ALS patients.

COSMOS was supported by the National Institutes of Health and by MDA through its Wings Over Wall Street gala event fundraiser.



To read about MDA's current ALS-related research efforts, visit mda.org/gaag.

MDA and Target ALS Foundation Form Partnership

Aim is to advance research and therapy development for ALS



MDA and Target ALS Foundation have announced a partnership aimed at supporting talented, young scientists who choose to pursue careers devoted to ALS research.

Through its discovery research program, MDA awards Development Grants to promising young postdoctoral fellows who are making the transition toward independent academic careers. These awards provide fellows with the support they need to craft and execute independent research projects that can boost their careers to the next level.

Target ALS has launched core facilities that enable ALS researchers access to human postmortem tissue, human stem cells and viral vectors (delivery vehicles for therapeutic genes). Access to tissue and stem cells from human patients is critical to allow researchers to compare laboratory findings from culture or rodent models to the pathological mechanisms that occur in the human disease. As part of the collaborative effort to support young ALS investigators, Target ALS will extend access to its core facilities to MDA's development grantees focused on ALS research.

Importantly, the partnership will help to fill both the therapy development pipeline and the need for talented researchers in the ALS field.



To read about MDA's current ALS-related research efforts, visit mda.org/als-research.

ALS (amyotrophic lateral sclerosis)

ALS Study Seeks Participants

Trial will test whether NPOO1 slows disease progression

Researchers are looking for people to participate in a phase 2 clinical trial designed to test whether the investigational drug NPOO1 slows progression of signs and symptoms in ALS.

Data from some studies suggests that in people with ALS there are increased levels of inflammatory (activated) macrophages, a type of white blood cell, resulting in the release of factors that damage motor neurons. NPOO1, a regulator of macrophage activation, is designed to exert its effect by converting these activated inflammatory macrophages back to their normal state.

Study investigators will measure the change from baseline

in score on the ALS Functional Rating Scale-Revised, a scale that enables physicians to evaluate an ALS patient's degree of functional impairment.

Trial participants will receive either treatment with NPOO1 or placebo and will be

required to visit the study site 23 times over the course of the study.

Trial sites are located in Arizona, California, Florida, Kansas, Kentucky, Massachusetts, North Carolina, Oregon and Texas. Travel support may be available.

 **For more information about this trial, including complete inclusion and exclusion criteria, please visit ClinicalTrials.gov and enter NCT02794857 into the search box, or see neuraltus.com/clinicaltrials.**



Duchenne muscular dystrophy (DMD)

Participants Needed: Exercise Study

Researchers are seeking boys ages 7-9 years with DMD to participate in a trial designed to assess the safety and feasibility of a home-based exercise program.

Prior studies in milder forms of muscular dystrophy and in rodent models suggest that resistance exercise may have beneficial effects for maintenance of muscle mass in DMD, but data on the safety and potential benefits of exercise in people who have DMD is lacking.

The study will last one to two weeks. All testing sessions and exams will be performed at

the trial site at the University of Florida in Gainesville. Three to six clinic visits over the one- to two-week testing period will include strength and exercise testing, along with imaging tests and a blood draw.

In order to participate, boys must be able to walk independently for at least 100 meters and climb four stairs. In addition, they must currently be receiving a form of corticosteroid treatment and meet additional criteria.

Travel and hotel accommodations will be provided for those who must travel to the test site for clinic visits.

 **To learn more or to inquire about participation, please contact Donovan Lott at djlottpt@phhp.ufl.edu or 352-273-9226. You also can find more information about the study by going to ClinicalTrials.gov and entering NCT02421523 into the search box.**



Investigators will determine whether a mild- to moderate-intensity strengthening exercise program can be safely implemented in boys with DMD.

Vamorolone Trial Volunteers Needed

Study will test dissociative steroid in boys with DMD

Volunteers are needed to participate in a clinical trial sponsored by ReveraGen BioPharma to test the safety and effectiveness of the investigational drug vamorolone in boys with DMD.

Vamorolone, a “dissociative steroid,” is an anti-inflammatory compound that researchers hope will convey the same benefits of traditional glucocorticoids, such as prednisone and deflazacort, without the unwanted side effects – including stunted growth, insulin resistance and weight gain – of those drugs.

In the two-part, open-label study, all participants will receive treatment with vamorolone. Study investigators will assess whether the drug has positive effects on muscle strength and timed function tests.

Boys ages 4–6 years old with a confirmed genetic diagnosis of DMD and who meet additional criteria may be eligible to participate.

This study is recruiting at sites across the United States and Canada, and travel and hotel accommodations will be provided for those who must travel to a test site for clinic visits.

Two MDA Venture Philanthropy (MVP) grants totaling \$1.9 million to ReveraGen supported preclinical development and

validation of the drug, and another MVP grant for \$1 million supported the phase 1 clinical trial to evaluate safety and tolerability in healthy adult volunteers.

 **To learn more or to inquire about participation, contact Andrea Smith at smitha7@upmc.edu. You also can find more information on the study by visiting ClinicalTrials.gov and typing “vamorolone” in the search box. For additional information on travel and accommodations, contact Suzanne Gaglianone at suzanne.gaglianone@reveragen.com.**



Vamorolone may positively affect muscle strength.



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Duchenne muscular dystrophy (DMD)

MissionDMD Trial Seeks Participants

Study will test FG-3019 in boys and men with DMD

Researchers are looking for boys and men, ages 12 years and older, to participate in the phase 2 MissionDMD clinical trial, sponsored by FibroGen, to test the experimental drug FG-3019 (pamrevlumab) in DMD.

FG-3019 is designed to block the activity of a protein called connective tissue growth factor, which is hypothesized to contribute to fibrosis (scarring) in DMD. Researchers will assess whether treatment with the drug reduces fibrosis in skeletal and cardiac muscle and leads to improved function in the arms and legs, breathing muscles, and heart.

All MissionDMD participants will receive treatment with FG-3019. They must attend clinic visits every two weeks for a period of two years. During clinic visits, they may undergo muscle function testing, lung function testing and Magnetic Resonance Imaging (MRI), and they may have blood drawn.

Researchers will evaluate whether FG-3019 is safe and effective in people with DMD and determine how the drug distributes through the body.

Participants must be nonambulatory (unable to walk), have a genetically confirmed diagnosis of DMD and meet additional criteria.

The study is being conducted at nine trial sites in the United States, and participants are eligible for reimbursement for some costs related to travel and overnight lodging associated with clinic visits.

For more information about this trial, including trial site locations and complete eligibility criteria, please visit ClinicalTrials.gov and enter NCT02606136 into the search box. If you or someone you know may be interested in participating in this trial, contact Gustavo Lorente at 415-978-1441 or glorente@fibrogen.com.



PTC has said it is willing to consider multiple pathways to advance a potential FDA approval for Translarna, including the possibility of conducting an additional clinical trial under accelerated approval.

First Appeal for FDA to Review Translarna Denied

PTC Therapeutics will appeal decision

The U.S. Food and Drug Administration (FDA) has denied PTC Therapeutics' first appeal that a New Drug Application (NDA) for ataluren (brand name Translarna) to treat some forms of DMD be accepted and that the FDA conduct a full review of the drug.

A "read-through" drug, Translarna is designed to act by changing the way muscle cells interpret genetic information, coaxing them to produce a needed muscle protein called dystrophin.

PTC completed submission of its NDA to market Translarna in the U.S. in January 2016, but in February received a Refuse to File letter from the FDA stating that the application was not sufficient to permit a review. PTC submitted an appeal in July to escalate continuing discussions with the FDA about a path toward approval for the drug.

The appeal now moves to the next supervisory level of the FDA as part of a process that may include multiple cycles of appeals to progressively higher levels of the regulatory agency.

In addition to having funded foundational work in DMD, MDA has contributed nearly \$3 million toward Translarna's development and phase 2 clinical testing.

Visit ptcbio.com for more about PTC Therapeutics' development of Translarna to treat DMD.

Myasthenia gravis (MG)

MG Drug Receives Orphan Drug Status

Amifampridine phosphate may benefit other diseases in MDA's program

The investigational drug amifampridine phosphate, under development by Catalyst Pharmaceuticals, has received U.S. Food and Drug Administration (FDA) orphan drug designation for the treatment of myasthenia gravis (MG).

Amifampridine phosphate is a potassium channel inhibitor designed to prolong signals released from nerves and allow greater stimulation of muscles. The drug currently is under clinical investigation as a symptomatic therapy to treat muscle weakness in people with MuSK-antibody positive myasthenia gravis. It also has shown promising results in a phase 3 trial that tested it in people with Lambert-Eaton myasthenic syndrome (LEMS), and currently is under investigation as a treatment for children with congenital myasthenic syndromes (CMS).



Financial incentives can help encourage the development of drugs for rare diseases.

FDA's orphan drug designation qualifies the sponsor of the drug for various development incentives, including tax credits for qualified clinical testing.

MDA has supported previous studies of amifampridine phosphate in children with CMS.



For more about Catalyst Pharmaceuticals' development of amifampridine phosphate to treat neuromuscular diseases, visit catalystpharma.com.

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Myasthenia gravis (MG)

Thymectomy in MG

Surgery to remove the thymus gland a validated treatment

In an MDA-supported worldwide study, researchers found that thymectomy (surgical removal of an organ called the thymus) reduced muscle weakness and lowered the need for drugs that suppress the immune system in people with MG.

In the trial, which was conducted in 67 centers across 18 countries, 126 participants between the ages of 18 and 65 years were treated either with surgery and prednisone (an immunosuppressant steroid) or with prednisone alone.

Trial investigators found that the combination of surgery and prednisone treatment reduced overall muscle weakness more than the prednisone-only treatment.

The researchers also found that, when compared with participants who were treated only with prednisone, participants who had surgery:

- required lower daily doses of prednisone to control the disease;
- had less need for additional immunosuppressant drugs; and
- experienced fewer adverse events (such as hospitalization).

The results from this trial will help inform clinicians and patients about the potential benefits of surgery for MG and lead to improvements in care for people with the disease.



The thymus gland is located behind the sternum and between the lungs.



To learn more about MDA's current MG research efforts, visit mda.org/gaag.

RYR-1 myopathies



MDA Partners with RYR-1 Foundation

Alliance will advance research and clinical care for RYR-1-related myopathies

MDA and RYR-1 Foundation have announced a partnership aimed at advancing research and clinical care, raising awareness, and improving education of patients, medical professionals and the public about RYR-1-related myopathies.

The partnership represents a key step in MDA's commitment to form collaborative relationships with other organizations working on the same diseases MDA covers.

Central core disease, centronuclear myopathy, multiminicore myopathy and other diseases caused by a mutation in the ryanodine receptor (RYR-1) are among the neuromuscular diseases MDA fights as an umbrella organization.

Partnership goals include working together to enhance the quality, quantity and scope of basic, translational and clinical research; bolstering the scientific review process; and increasing collaboration among investigators.



To read about MDA's current RYR-1-related research efforts, see a list of our current grants at mda.org/active-grants.

Spinal muscular atrophy (SMA)

Nusinersen (Spinraza) Approved!

SMA drug could help all ages, all forms of SMA

Biogen and Ionis Pharmaceuticals announced Dec. 23, 2016, that the U.S. Food and Drug Administration (FDA) has approved nusinersen (brand name Spinraza) for the treatment of SMA.

Spinraza has been tested both in blinded and open-label studies, and in patients with types 1, 2 and 3 SMA. Interim results from the late-stage ENDEAR trial in infants with type 1 SMA demonstrated strong evidence for efficacy, prompting premature closure

of the placebo-controlled trial in order to transition all participants to the open-label SHINE trial, in which all babies receive treatment with the drug. Similar early results were noted in the phase 3 CHERISH trial to test Spinraza in children with the type 2 form of the disease. As with ENDEAR, the CHERISH trial was stopped to allow participants to join the open-label study.

Spinraza is a disease-modifying antisense drug, one of a class of experimental

FDA approval for Spinraza comes just three months after Biogen submitted a new drug application to market the drug in the United States.

therapeutic molecules designed to target genetic instructions at the RNA stage (an intermediate step between DNA and the protein manufacturing stage inside cells). It is designed to increase production of the needed SMN protein.

MDA has funded foundational work in SMA and supported Adrian Krainer at Cold Spring Harbor Laboratory in New York for early-stage development of Spinraza.

FDA approval of Spinraza allows for widespread access to the drug for kids and adults with SMA across the United States and makes the drug the first FDA-approved treatment for the life-threatening disease.

Visit mda.org/spinraza for updates and to learn more about how to access Spinraza. Read strongly.mda.org/fda-approves-first-ever-treatment-sma to learn more.

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In Good Hands

How to find specialized child care for kids with neuromuscular diseases

BY CHERYL ALKON



All parents need time to themselves, but for BJ Mirabile of Winchester, Mass., finding child care for her daughter Katie, who has spinal muscular atrophy (SMA), often

proved difficult. “When she was younger, we had some neighborhood sitters and sitters through early intervention,” Mirabile says. But early intervention services are for babies and toddlers

with developmental delays or disabilities. Now Katie is older, and she uses a power wheelchair, BiPAP (bilevel positive airflow pressure) machine and MIC-Key gastric feeding tube. “I think people get scared and they say, ‘I don’t know anything about that,’” Mirabile says.

It is true that a lot of child care providers don’t have experience with children with complex needs. But it is possible to find people who are willing to learn. Finding a child care provider who is a good match for your family just takes a little savvy about where to look — and some patience and ingenuity.

BEGIN THE SEARCH

A lot of parents turn to child care websites to search for sitters. According to Sarah Berg, director of marketing at sittercity.com, approximately 3 percent of the sitters on the site note that they have experience with children with

WRITING THE MANUAL

What should go in your child care provider training manual? Start with these questions. Write down the answers and any other details about your child’s care or routine.

- What medical equipment does your child use and how will the provider operate them?
- Does a provider need to be able to lift your child in and out of a wheelchair?
- Do they need to know how and when to turn your child during sleep?
- Do they need to put on and take off braces?
- Should they know how to perform CPR on a child with complex medical needs?
- Does your child need medications, and when should they be administered?

FIVE TIPS FOR MAKING YOUR CHILD CARE PROVIDER MATCH

- 1. Use your support network.** Consider asking family members or neighbors who already know your child if they can provide child care for a night out.
- 2. Widen your network.** Tap into Facebook or Yahoo groups for parents who have children with the same diagnosis as your child. Ask how others find care and if they can recommend providers they know.
- 3. Ask questions.** Once you have potential candidates, interview them over the phone and then, if you like what you hear, in person. Find suggested interview questions and other advice here: sittercity.com/parents/find-child-care/find-a-special-needs-caregiver.
- 4. Check references.** Always check a provider's references and visit the Dru Sjodin National Public Sex Offender Website (nsopw.gov) to see if the candidate has been charged with these types of crimes. Background checks are also important, and some child care websites include that service.
- 5. Let your child have a say.** Ask your child how he or she feels about a child care provider — both before you hire a new provider and going forward. Some providers haven't been a good match for BJ Mirabile's daughter, Katie — such as one who would hover when Katie wanted to lie down without feeling watched. "But those who work well become a part of our family," says Mirabile.

complex disabilities, such as muscular dystrophy or spinal cord injury. "Depending on the level of care needed, if a family is willing to provide some hands-on training, that can definitely help them increase their chances of finding the right fit," she says.

Sometimes it can be easier to find the right person through a more specialized search. Parents may find helpful information from programs designed for those living with disability, such as their local parent information center (parentcenterhub.org) or the National Respite Network and Resource Center (archrespite.org).

Joseph Ban, co-founder of Denver-based Specialized Sitters (specializedsitters.com), regularly hires child care providers to work with children with complex needs. He recommends reaching out to the therapists or health care providers you already see for your child's care, as well as the special education department in your school district, to ask if they can recommend potential child care providers. Find out if the paraprofessionals or others at your child's school are available. "You won't get the therapist making \$150 an hour to watch your child for \$15 an hour, but the intern working there might," he says.

Also, consider contacting universities nearby, as they often have physical therapy programs or special education degrees, and students may be interested in part-time work.

BE HANDS-ON

You know your child best. When working with a new child care provider, be clear about what you need. It can be helpful to write down everything you want them to know — and how to do each task — in a notebook or training manual.

The first time your provider comes to work, it can take a few hours to train them in person. "When I train them, I don't go out for the first night, so they are comfortable with the equipment," Mirabile says. "I have them do it solo one or two times when they are with me."

Be patient, as some people will catch on more quickly than others. "Family members who have seen me care for Katie and nursing students get it," says Mirabile. "Some concepts are easier to understand if they have experience with a senior parent or a child of their own."

While this process takes time, it's worth it to be able to take a break. "When I have found a great personal care assistant, I know that my daughter's care is in the right hands," says Mirabile. "I can relax and rejuvenate for when I'm needed to care for her." **Q**

Cheryl Alkon is a freelance writer based in Massachusetts.

You're Not Alone in Your Journey

MDA is here every step of the way, ready to provide resources and guidance, support groups and ways to connect with others online. Contact your local MDA office to find support groups and other events in your community at mda.org. (Type your ZIP code into the "Find MDA in Your Community" box.) Be sure to connect with MDA on Facebook ([@MDANational](https://www.facebook.com/MDANational)) and Twitter ([@MDAnews](https://twitter.com/MDAnews)).



Sarah Bellish, here with her sorority sisters, is active on campus and in her sorority at Baldwin Wallace University.

advocate



Taking on accessibility challenges in higher education

BY CHARMAINE DYMOND

For many young adults, the college experience is a rite of passage and an exciting time for growth. This time comes with additional challenges and opportunities for the 9 percent of college students with disabilities in the United States. For some, it's not just about the lessons that come from living away from their parents and families, making decisions about their future or navigating the world on their own. It's also a chance to learn to advocate for themselves and others.

Learn More

Browse the new Young Adults Program website at mda.org/young-adults, which includes a collection of resources that are especially helpful for young adults with neuromuscular conditions. Resources cover everything from education and employment to independent living.

While the Rehabilitation Act of 1973 and the Americans with Disabilities Act of 1990 require higher education institutions to provide academic adjustments and accessible housing, equal access goes beyond classes and dormitories.

“I think the accessibility of social settings is especially important for students,” says Alexandra McArthur, MDA’s national director of young adult and community engagement. “How do they fully participate in college life if they’re not able to get in the front door of the party or gathering?”

BECOMING AN ADVOCATE

Some problems with accessibility stem from geography — think sprawling campuses or lots of hills — or the age of the buildings. “A lot of higher education

institutions are older, and some of them have done a better job of retrofitting than others,” McArthur says.

Sarah Bellish, who has spinal muscular atrophy (SMA), discovered this during her freshman year at Baldwin Wallace University (BWU), founded in 1845 in Berea, Ohio. “Our buildings are very old,” says the sophomore, who uses a power wheelchair. Bellish is dedicated to advocating for improved accessibility at BWU. She’s come up against

inaccessible dining halls, malfunctioning elevators and a lack of accessible bathrooms.

“I was at a musical — we have a really great theater department — and needed to go to the restroom. But there was not a single accessible restroom in the entire building. That was crazy.”

Bellish submitted an online work order drawing attention to this particular problem, but she didn’t stop there. “I was contacted immediately saying that they were going to fix it.

“My passion became working on disability policy as well as working with people with disabilities to help them learn early on what they need ... to navigate [higher education] as an adult with a disability.” — Jean-Marie Lawrence



Jean-Marie Lawrence graduated from the University of Tennessee at Chattanooga in May 2010.

Then I talked to our [uni-
versity] president, who I've
spoken to quite a few times
about issues. After I talked to
him, that was when the ini-
tiative to check every single
building came into play.”

KNOW YOURSELF

Understanding your needs is
a critical first step to becom-
ing a successful advocate.
“The challenges that I faced
weren’t just on campus,”
says Jean-Marie Lawrence, a
graduate of the University
of Tennessee at Chattanooga
who has limb-girdle mus-
cular dystrophy (LGMD).
Though she worked with
the school’s vocational
rehabilitation counselor, “I
was struggling to get the
personal assistance services
I knew I needed,” she says.

This experience fueled
Lawrence’s passion to change
the system. Since graduating
in May 2010, she has been
pursuing her second master’s
degree while working as a
transition and academic coach
at the nonprofit Journey Edu-
cational Services.

“I saw a lot of issues, and I
knew that I couldn’t change it
on the outside; I needed to get
to the inside,” says Lawrence.
“My passion became working
on disability policy as well
as working with people with
disabilities to help them learn
early on what they need in
terms of university services,
personal knowledge and
advocacy skills to be able to
navigate the new terrain as
an adult with a disability.”

Lawrence recommends
starting early by speaking
with disability services at uni-
versities you’re considering

Dimpal Patel enjoys campus life at the University of North Carolina at Charlotte.



to “get a really good feel for
how committed they are to
their students and advocating
for their students, or helping
their students learn how to do
that for themselves.”

KNOW YOUR RIGHTS

“Your college or university has
to make certain accommoda-
tions, and if they’re making
that very difficult, then you
need to know how to bring in
different support systems or
even legal action, if needed,”
McArthur explains.

Bellish says she sometimes
struggles with feeling like
she’s always asking people
for things, but she reminds
herself that her requests are
reasonable. “You’re allowed
to push a little bit until you

*“I used to be nervous talking on
the phone or talking to people I
didn’t know. Advocating for myself
has allowed me to break away
from my comfortable, shy self and
speak out.” – Dimpal Patel*



“I definitely had to do a lot of self-advocacy, saying, ‘Hey, that’s not going to work for me. Let’s do it this way, and here’s why.’”

– Owen Kent



Owen Kent is a senior at the University of California, Berkeley.



TRANSITIONING TO HIGHER EDUCATION

MDA is dedicated to improving the lives of kids and adults living with neuromuscular diseases. Supporting individuals as they pursue their educational goals and learn to be self-advocates is one way we can help.

“We take our role in advocacy seriously,” says Alex McArthur, MDA’s national director of young adult and community engagement. “Ultimately, we want to support our young adults in achieving whatever their dreams are, whether that includes living on campus at the university, going to community college right next door or not going to college. We are here to help and provide resources and referrals to make those dreams a reality.”

To find out what resources are available to help you with your transition to higher education, contact your local MDA office or the MDA National Resource Center at ResourceCenter@mdausa.org or **800-572-1717**.

actually see the changes that you need. It’s not like you’re asking for anything crazy. You’re just asking for something that’s going to make your life a little bit better.”

McArthur cautions that self-advocacy isn’t just about making demands. “It’s asking questions like, How do you think we can work on this together? How would you propose we make this accessible? It’s really about being as clear as possible about the challenge, and then working together to find a solution.”

SPEAK UP

Letting others know about your rights and needs often elicits a surprisingly positive response. “A lot of times, people want to help. It’s just that they have absolutely no idea what you’re going through or what to expect,” says Bellish.

Dimpal Patel, a senior at the University of North Carolina at Charlotte who has a form of muscular dystrophy, kept quiet her freshman year when the desk and toilet in her dorm were too low to use comfortably. “But when I was talking to my disability services coordinator at the end of the year, I asked them to fix it for next year, and he said they could have fixed it this year if I’d told them earlier,” she says. “I didn’t know [the university] would be that accommodating.”

This experience helped Patel find her voice. “I used to be nervous talking on the phone or talking to people I didn’t know,” she says. “Advocating for myself has allowed me to break away from my comfortable, shy self and speak out. I have learned how to stick up for myself.”

THE GREATER GOOD

Whether it's asking for changes to your dorm room or advocating for accessible bathrooms all across campus, speaking up for yourself helps others, too.

Owen Kent, a 24-year-old filmmaker with SMA in his senior year at the University of California, Berkeley, is grateful for the efforts that came before him, which have made it easier for him to be an active leader in campus organizations. "I'm fortunate that when I started in a lot of positions, they were already somewhat accessible," he says. "That's not to say they were set up for it. I definitely had to do a lot of self-advocacy, saying, 'Hey, that's not going to work for me. Let's do it this way, and here's why.'"

For example, as president of his campus housing co-op, one of his duties was to lead the meetings while taking notes. "I wasn't going to be able to type fast enough to take notes, so I suggested that we have someone else do that," he says. "It worked really well, and even now that I'm not president, that's how it's done."

Although not all accessibility problems will be fixed so easily, in Kent's and many others' experiences, by being clear about your needs, knowing your rights and finding the right person to talk to, change is possible.

"Maybe you're the first person in a wheelchair to be living on campus," says McArthur. "What an opportunity for the institution to learn how to accommodate successfully, right? And what

"You're allowed to push a little bit until you actually see the changes that you need."

— Sarah Bellish

an opportunity for you to learn how to coordinate your care and to figure out how to request help. I think all of this can be in the spirit of learning, and that's what college is all about." [Q](#)

Charmaine Dymond is a freelance writer in Halifax, Canada.



Sarah Bellish makes a friend at a sorority event that was moved to an accessible building.

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8 Some individuals consider exercise a four-letter word, while others can't seem to get enough. Whatever your feelings, the research shows that physical activity is good for everyone. Inactivity can lead to secondary health problems, such as high blood pressure and diabetes. A lack of exercise can quickly put on the pounds. Weakened muscles burdened with added weight is a double whammy.



*Physical activity is good for every body –
and it may be easier to stay active with
a neuromuscular disease than you think*



“If Natalie does too much, she can be down for days. ... It’s all about finding the balance and adjusting accordingly.”

– Nicole Dion, Natalie’s mom

There is no one-size-fits-all exercise prescription for people living with neuromuscular diseases. One person might be climbing mountains, while another needs assistance to climb into bed. The degree to which an individual can be active depends on a wide range of variables, even for those who have the same disease.

Whatever your abilities, incorporating physical activity into your life can help you manage your disease by maintaining flexibility, strength and mental fortitude. Before starting any physical activity or exercise program, consult with your MDA Care Center physician and physical therapist for guidance.

BREAK THE BARRIERS

Committing to a more active lifestyle can be challenging for individuals with neuromuscular diseases, especially for those with a new diagnosis or those who have not yet embraced exercising. A variety of potential obstacles can waylay the best intentions: lack of accessible facilities and equipment, cost, time or the need for someone else’s assistance.

Pennie Naylor, 60, has never considered herself athletic, but she always enjoyed walking. “Walking was my therapy. I would just walk and walk and solve my problems and the world’s problems,” Naylor says. Charcot-Marie-Tooth disease (CMT) has deteriorated the muscles in her hands and feet. “Walking one step for me is equal to someone else walking four or five,”

she adds. “What few steps I have, I give to my dog.”

Now, Naylor rides a stationary bike twice a week. “It’s a replacement for walking. You can kind of get lost in it,” she says.

DO WHAT YOU LIKE

Staying active is a lifelong commitment. You’ll be more likely to stick with an exercise routine if it suits your abilities and your interests.

Owen Kent, 24, was tired of traditional physical therapy sessions. Kent has spinal muscular atrophy (SMA) and uses a power wheelchair. To start something new, he turned to his physical therapist at his local MDA Care Center, Tina Duong, who helped him locate an aquatic therapy program within a 10-minute drive of his home. For Kent, returning to the pool is motivating. As a child, he swam often and always enjoyed the water.

Duong, a physical therapist at Stanford University School of Medicine, tells her patients, “Focus on what you like to do, because exercising isn’t an easy thing to keep doing.”

To set yourself up for success, she advises talking with your medical team about the kinds of exercise you might be able to do. “Individuals need to be their own advocates and let their therapists know they want to maintain an active lifestyle that is feasible beyond visiting the [MDA Care Center],” says Duong.

JUST MOVE

Melissa Fox, a physical therapist at the MDA Care Center located at the University of Virginia, explains that when

JOIN THE FITNESS COMMUNITY



“Most of us get a lot of joy out of going out,” says Tina Duong, a physical therapist at the MDA Care Center located at the Stanford University School of Medicine. “Finding a group exercise or activity that works for you could be fun.”

Follow these five tips to find exercise classes or activities that meet your unique needs:

- Talk with your local MDA office to find community resources. Some hospitals, rehabilitation facilities and senior centers offer wellness programs or classes designed for people with disabilities or chronic medical conditions.
- Join support groups and ask other members what they do to stay active.
- Search the National Center on Health, Physical Activity and Disability’s website (NCHPAD.org) for programs in your state.
- Find adaptive fitness programs through Disabled Sports USA (disabledsportsUSA.org), which has information on 45 adaptive sports, from archery to yoga.
- Attend one of the eight Abilities Expos (abilitiesexpo.com) to learn about adaptive sports and inclusive recreation in the region.

people continually sit, their muscles and tendons contract, which can cause pain. To avoid this problem, it is imperative to move and explore assistive technology like a mobile stander or standing wheelchair.

Of course, being active doesn’t mean you have to do

100 pushups or spend hours sweating at a gym. According to Fox, anything that gets you moving can count as exercise, even active video games, such as tennis or race car driving.

For 13-year-old Natalie Dion, mitochondrial myopathy limits her activity level

and requires constant monitoring between exertion and resting, explains her mom, Nicole. The disease weakens muscles and causes fatigue.

At school, Natalie plays on a volleyball team, which is good for her coordination. The coach allows her to skip



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“Pilates is phenomenal. I’m in the beginners’ class, and I’ll always be in the beginners’ class. I don’t progress like other people do, and that’s OK.” – Pennie Naylor

PHOTO COURTESY OF CARLA SHIFFLETT POSTURE STUDIO



Pennie Naylor credits pilates classes with strengthening her core and improving her balance.

the pregame running and has her play in short bursts. “If Natalie does too much, she can be down for days,” Nicole says.

Keeping her daughter active is important for her health, and Nicole sneaks in a variety of physical activities to make exercise fun. The family frequently splashes in the pool

or turns on music and dances around their house. Outside, Natalie enjoys bouncing for five or 10 minutes at a time on her trampoline. Indoors, Natalie uses a jump rope, climbs the stairs or does airplane stretches on a large exercise ball. Last year, loom bracelets were the rage, and Natalie worked on her finger

dexterity while making rubber band jewelry.

Figuring out how much Natalie can physically do varies from day to day. “It’s all about finding the balance and adjusting accordingly,” says Nicole.

PRACTICE MODERATION

Most neuromuscular diseases are progressive and frequently cause fatigue. Performing daily tasks such as dressing, gardening and housework may be an adequate amount of physical activity for some.

“We teach people a lot about what we call ‘energy conservation,’ which is how to manage your energy throughout the course of the day,” says Fox. “It’s not going to be the same amount you had before your diagnosis or the same amount as an average person.”

Think about what your day entails and pace yourself. For example, don’t spend the morning at the gym if you are scheduled to attend a party that evening. Loading your

STAY MOTIVATED



Everyone occasionally needs a carrot to keep plugging away at an exercise program. Use these tips to stay active.

- **Use the buddy system.** Knowing someone is waiting for you at yoga class can be a powerful incentive.
- **Write how you feel after exercising in a fitness journal.** Read this journal when enthusiasm is waning.
- **Reward yourself with a new exercise outfit or piece of equipment.** How about a waterproof iPod Shuffle for the pool?
- **Try something different.** Join a new class or buy a video game that gets you moving.
- **Listen to an audiobook only when you exercise.** You’ll be eager to hear the next chapter.

dishwasher might be a more productive way to expend energy than lifting weights.

Gauging how much you can do means listening to your body. If you feel worse after an activity, you might be overworking your muscles. Individuals with neuromuscular diseases should not overexercise. "For example, if you do too many squats, you're more likely to fall later in the day because your legs are tired," says Fox.

BE ADAPTABLE

When you're ready to be more active, remember to start small and build up gradually. Try new activities to keep your routine fun. And most importantly, continually re-assess your fitness goals with the help of your physician to make accommodations for your changing body.

Four years ago, Naylor joined a Pilates class and credits her twice-weekly sessions with strengthening her core and improving her balance. "Pilates is phenomenal. I'm in the beginners' class, and I'll always be in the beginners' class. I don't progress like other people do, and that's OK," says Naylor.

"The key is to adapt," she says. "I don't look at what I can't do anymore. Instead, I look at what I'm able to do." 

Barbara Twardowski has Charcot-Marie-Tooth (CMT) disease and uses a power wheelchair. Jim, her husband, is a registered nurse. Both have degrees in journalism. They live in Louisiana and write about accessible travel, health and lifestyle, and related issues.

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*These strategies can help put
common sleep obstacles to bed*

BY SHAILA WUNDERLICH

getting you *zzz*

ZZZs

Sixty percent of Americans have trouble sleeping, according to the National Sleep Foundation. For individuals with neuromuscular diseases, that number is likely higher, because disease-related factors, including limited mobility and respiratory problems, can make it difficult to achieve deep, restful sleep.

Research shows that proper sleep is important for maintaining physical and mental health. But if it's your health that's preventing you from getting a good night's sleep, you might feel stuck. Fortunately, by identifying what's keeping you up, you can develop strategies to put those sleep obstacles to bed.

PHYSICAL DISCOMFORTS

Mark Eisenberg, 23, has Duchenne muscular dystrophy (DMD). Since his diagnosis at age 4, he has gone from sleeping on his stomach to lying on his back. "It's easier to breathe and move," Eisenberg says. "Gravity does all the work."

But being limited to one sleeping position raises its own challenges. If a limb begins hurting or an arm is twisted awkwardly, it can be difficult to adjust or turn. "I get in one position, and I kind of have to stay there," he says.

Further complicating positioning issues are neurological ones. "Many individuals with neuromuscular conditions may have decreased sensation, so they can't sense where their joints are in space," says Kim Dohm, an occupational therapist with the MDA Care Center at the University of Missouri. "Maybe you need to move your foot an inch to the left, but you're not even sure if it's your foot that's uncomfortable."

Give them a rest: Comfortable bedding and an adjustable, low air-loss mattress can help prevent discomfort. For example, Eisenberg recently traded in a lumpy four-year-old mattress for a new adjustable model with special foam.

"There are fewer bumps and grooves to get stuck in," he explains. "I can raise the bottom if I need to move my leg, or raise the top if I need to slide down on my pillow," he says.

Talk to Your MDA Care Center

Be sure to speak with your MDA Care Center team if you experience any sleep difficulties. They may be able to help tailor strategies to your needs.

SLEEP INTERRUPTIONS

During sleep, we often drift between sleep cycles, and even a good night's sleep may

SLEEP ASSISTS

Accessory storage. Keep bedtime essentials close at hand. Simple Sheets' Storage Pocket (\$8.99, simple-sheets.com) attaches to the side of the mattress.

Adjustable beds. Invacare's Full-Electronic Homecare Bed (\$1,768, invacare.com) raises electronically and has three layers of special foam for comfort and support. Assured Comfort Beds also makes adjustable beds with a high/low feature (\$3,750 to \$7,700, assuredcomfortbed.com) that can fit into most existing bed frames.

Grabbers. These long-handled helpers take the anxiety out of simple tasks like scratching an itch or moving a blanket. Duro-Med's lightweight 32-inch Reacher Grabber (\$9.67, amazon.com) has an ergonomic trigger handle and a magnetic strip for picking up metallic objects.

Respiratory aids. Provent's tiny nasal device (proventtherapy.com) mimics the benefits of a CPAP machine without all the bulky equipment. Requires a prescription.

Smooth, clean bedding. Simple Sheets' Velcro-adhering top sheet (\$45.99 for a four-pack, simple-sheets.com) keeps bed sheets smooth and is easy to change when dirty.

Supportive pillow. Contour Products' CPAP Pillow 2.0 (\$49.95, contourliving.com) is shaped to avoid putting pressure on a breathing mask.

Turning mattress. ProBed's Freedom Bed (\$4,000 to \$15,000, pro-bed.com) automatically rotates sleepers who need assistance with turning and relieving pressure on different parts of the body.



Mark Eisenberg

“Sometimes I’ll wake up because my body is in an awkward position or in lots of pain. And it will require my mom rolling me over or propping me up for 20 minutes until the sensation disappears.”

– Mark Eisenberg

include small awakenings. But when a sleep cycle is interrupted, it is harder to fall back asleep.

For individuals with neuromuscular diseases, positioning issues and pain can be common culprits of sleep interruption.

At his home in New Jersey, Eisenberg manages most nights with minimal interruption. But

on the nights he does need help, the experience can be tedious. “Sometimes I’ll wake up because my body is in an awkward position or in lots of pain,” he says. “And it will require my mom rolling me

over or propping me up for 20 minutes until the sensation disappears.”

Give them a rest: When an individual needs a caregiver’s assistance to turn, draw sheets can make the process smooth and quick. For even less disruption, consider a turning mattress that automatically shifts the sleeper periodically throughout the night to help change position and minimize the risk of developing pressure sores.

ENVIRONMENTAL OBSTACLES

According to the National Sleep Foundation, the optimal bedroom environment is cool, dark and quiet. But for some conditions, or as diseases progress, bulky or noisy equipment must be present in the bedroom.

“Many people have some device, like an oxygen concentrator or a BiPAP,” Dohm says. “And all that requires more equipment, more assistance and more time to accommodate.”

Give them a rest: Use a white noise machine, fan or air purifier to mask unwanted noise. No matter what your bedroom environment is, consider practicing meditation, which is a powerful tool for promoting relaxation. Eisenberg works with his respiratory therapist on breathing techniques. “By focusing on my breathing, I can allow the other disruptions to fade,” he says. **Q**

Shaila Wunderlich is a freelance journalist in St. Louis who has worked for a variety of magazines, journals and newspapers for nearly 20 years.

Catch Your Breath

For individuals with neuromuscular diseases, nighttime respiratory problems can be responsible for sleep disturbances. Learn more about testing and treatment for sleep-breathing problems in “Not Enough ZZZzzzs?” at mda.org/quest.

HEALTHY SLEEP HABITS

Everybody needs good sleep habits. Optimize your sleep by following this advice from the National Sleep Foundation:

- 1. Stick to a schedule.** Try to go to bed and wake up at the same time every day, even on weekends. This helps to regulate your body’s clock.
- 2. Practice a relaxing bedtime ritual.** Having a relaxing routine right before bedtime helps separate your sleep time from activities that can cause excitement or stress.
- 3. Be active every day.** Physical activity helps promote sleep, but avoid anything vigorous close to bedtime. Exercise circulates endorphins in the body, which may keep you awake.
- 4. Make your bedroom your sanctuary.** Design your environment to establish the conditions you need for sleep. This includes keeping the temperature between 60 and 67 degrees, masking noises, and blocking out light and other distractions.
- 5. Sleep comfortably.** Make sure your mattress and pillows provide the support and comfort you need.

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A GOOD *beginning*

Why newborn screening is important for every baby



For the first time in MDA history, we are seeing the development of specific drugs and therapies for the diseases in our program — many of which are the result of decades of MDA research. With potential new therapies in or nearing the approval stage, we are able to help infants with these diseases in a way never before possible: through a public health program called newborn screening (NBS).

Here, R. Rodney Howell, M.D., chairman of MDA's Board of Directors, shares his thoughts on NBS and what it means for families with neuromuscular diseases.

Why is newborn screening (NBS) important?

We currently screen all newborn babies in the United States for more than 30 conditions for which treatments are available. Early detection allows for earlier treatment, which can prevent or dramatically reduce the symptoms of these diseases.

How can NBS for neuromuscular diseases make a difference?

There are exciting new therapies that have been approved — and others currently under consideration or soon to be considered for review — by the U.S. Food and Drug Administration (FDA). In September 2016, the FDA granted accelerated approval to eteplirsen (brand name Exondys 51) for the treatment of some forms of

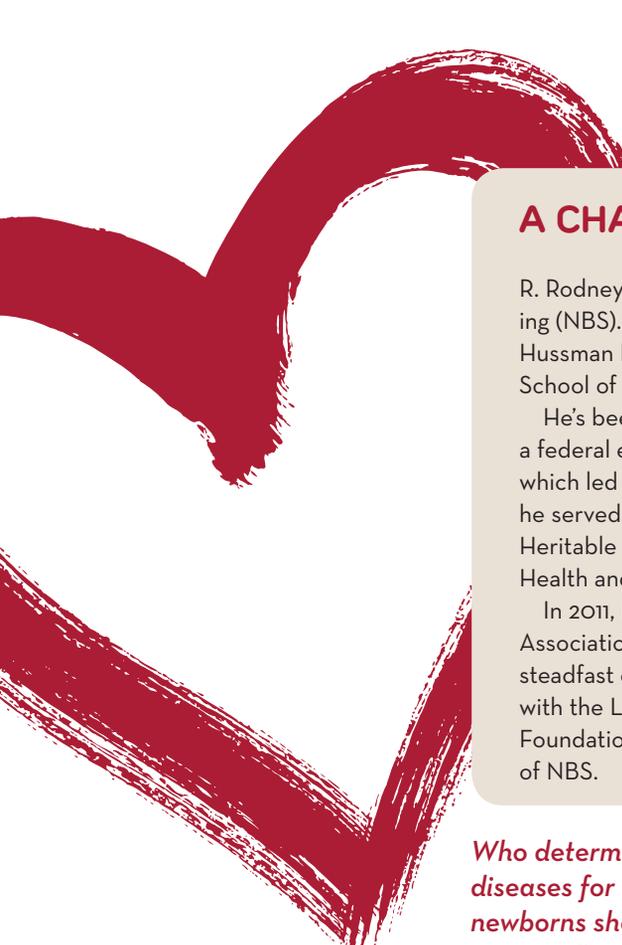
Duchenne muscular dystrophy (DMD), making it the first drug on the market for DMD that could modify the disease itself. Exondys 51 is expected to help about 13 percent of those with DMD by slowing progression of the disease. Notably, for this drug to have its greatest impact, it must be started before the destruction of existing muscle occurs, as it is not expected to help in the production of new muscle.

In another exciting development, a new drug for spinal muscular atrophy (SMA) was approved by the FDA Dec. 23, 2016. Nusinersen (brand name Spinraza) has shown promise in slowing or halting the progression of the disease, with results from clinical trials showing that babies treated with the drug were able to achieve motor milestones, such as sitting up and crawling, that they otherwise wouldn't be expected to achieve.

We must work very hard to get into place NBS for these and other conditions as drugs to treat them become available.

Learn more

To learn more, visit mda.org/newborn-screening.



A CHAMPION OF NEWBORN SCREENING

R. Rodney Howell, M.D., is recognized as one of the world's experts on newborn screening (NBS). Howell is professor of pediatrics, chairman emeritus and a member of the Hussman Institute for Human Genomics at the University of Miami Leonard M. Miller School of Medicine.

He's been heavily involved in implementing NBS in the United States. In 2003, he led a federal expert panel tasked with standardizing screening protocols across the country, which led to the expansion of screening programs the following year. From 2003 to 2011, he served as founding chair of the congressionally mandated Advisory Committee on Heritable Disorders of Newborns and Children (ACHDNC), advising the secretary of Health and Human Services on genetic testing in children.

In 2011, Howell received the Champion of Newborn Screening Award from the Association of Public Health Laboratories in recognition of "outstanding leadership and steadfast commitment to the health of newborns and children." In 2012, he was honored with the Lifetime Achievement Award by the American College of Medical Genetics Foundation for his contributions to medical genetics, especially for his work in the area of NBS.

Who determines the diseases for which newborns should be screened?

There are a variety of important factors used to determine which disorders are screened for in the newborn period. A federal advisory committee (the Advisory Committee on Hereditary Disorders in Newborns and Children, or ACHDNC) that consists of clinicians, researchers, public health experts, patient advocates, disease experts and newborn screening leaders, examines data and evidence on each disease that is submitted for consideration. It then makes formal recommendations on whether a specific disorder should be added to

the Recommended Uniform Screening Panel (RUSP), a list of diseases that ACHDNC recommends be screened for in newborns.

Because NBS is a public health program that falls under the authority of the individual states, the final decision on whether to test for a specific disease is up to each state. These decisions often rely heavily on the expert recommendations contained in the RUSP.

What rules or criteria must be met for a disease to be added to the RUSP?

Not every disorder meets the criteria required to be included on the list. While

the committee considers a number of factors when determining whether a disease should be included on the list, key factors include the following:

- The disease must be very serious.
- The disease must be identifiable in the newborn period with a reliable test that is suitable to use for every baby in the country.
- A treatment must exist that can benefit or cure babies affected by the disease.

Why do some states screen for additional diseases that are not on the list?

The states usually follow the RUSP; however, some states have added conditions to the list. This approach is not recommended, because these additional diseases may lack effective diagnosis and treatment options. Results from such testing can provide families with false hope and, ultimately, with less than optimal outcomes.

“Because NBS is a public health program that falls under the authority of the individual states, the final decision on whether to test for a specific disease is up to each state.”

– R. Rodney Howell, M.D.

Some states do not screen for diseases that are on the RUSP. Why not?

Often, we assume that if a disease has been added to the RUSP, states will automatically start testing newborns immediately for the disease. However, each state must take specific action to add the disease to its own screening panel. This implementation phase can be complicated, expensive and can take a significant amount of time.

In 2016, California took an important step by passing a state law that says any condition recommended by the ACHDNC will be added to the state's screening panel without having to first be approved by the state's advisory panel. This law is expected to help make the implementation phase faster and more straightforward. The California state advisory panel will continue to have a very important role in establishing exactly how a condition will be tested, as well as how babies will be followed up and treated — all of which are critical to a good outcome for the babies.

What is MDA's role in helping to advance NBS for neuromuscular diseases?

MDA has a critical role in NBS for Pompe disease (already on the RUSP), DMD and SMA, along with other neuromuscular diseases as they become eligible for addition to the RUSP.

MDA's engagement in the clinical care of infants identified in newborn screening is

paramount. Once these babies are diagnosed, our MDA Care Centers across the United States can play a key role in their confirmatory diagnoses, treatment and follow-up. The follow-up care in most cases will be lifelong, and in some situations (for example, late-onset Pompe disease) the clinical symptoms might be quite delayed. Only recently, with new treatments coming online and being made available, have MDA Care Centers been able to expand the vital role they play in the diagnosis and treatment of neuromuscular diseases.

MDA also plays an important role in educating and supporting providers and families about the NBS



program, and in working to influence public policy to ensure that disorders under MDA's umbrella are included on the RUSP.

MDA's efforts will continue to ensure the overall viability of the newborn screening program. Q

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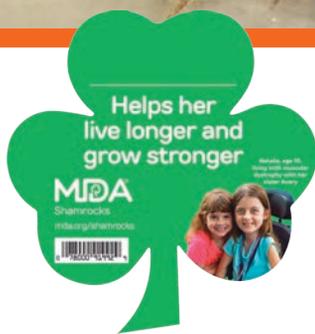


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access MDA

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



Shamrocks Spirit

This year marks the 35th anniversary of MDA's Shamrocks fundraising partnership with retail stores across the country. To get in the Shamrocks spirit, we'd like to introduce you to some of the kids featured in this year's campaign, which kicks off in February.

Meet Gabrielle, age 14

When Gabrielle was diagnosed at the age of 1, her parents were told she wouldn't see her third birthday. Now 14, Gabrielle is thriving and doesn't allow anyone or anything to stop her from doing what she loves. She attended MDA Summer Camp for the first time in 2016, and her favorite memory was being able to go horseback riding.

"I don't allow anyone to stop me from doing what I desire to do. I've traveled to Europe and the Caribbean and dream of traveling to other places, too."



Meet Natalie, age 10

Natalie enjoys playing with her little sister, Avery. After her diagnosis of Bethlem myopathy, her family didn't know what was in store for her future. Today, Natalie is getting the support and assistance she needs to live longer and grow stronger by attending MDA Summer Camp and receiving expert, multidisciplinary care at the local MDA Care Center. When she grows up, Natalie wants to

become an architect and build accessible homes and buildings.

"My favorite part of Shamrocks is going to all of the participating stores and taking pictures."



Meet Garin, age 13

Garin was diagnosed with Duchenne muscular dystrophy (DMD) at age 3, and even though daily life with this disease is challenging, that doesn't stop Garin from facing them head on with the most wonderful attitude. He loves animals – including his service dog, Ranger – and dreams of someday becoming an architect to design buildings that are more accessible.



“When facing each day with a fatal disease, you certainly learn to not sweat the small stuff and to appreciate each day. We know that if we enjoy the moments of today, we can handle the challenges of tomorrow when we get there.”

– Garin and Rebekah, Garin's parents

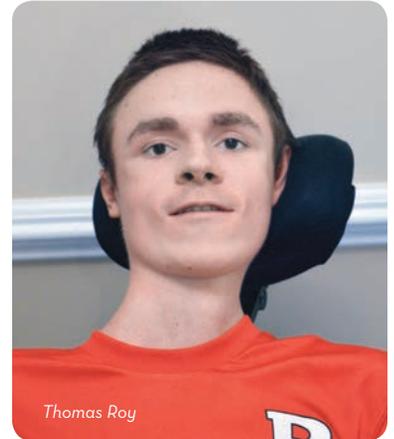
 Meeting our MDA families and volunteers makes a big difference in the spirit, commitment and effort to sell more shamrocks for participating retailers and their employees. When they meet the families they are helping, it makes their fundraising efforts so much more meaningful. There are so many ways you can get involved. Visit mda.org/shamrocks to find participating retailers near you and sign up to become an MDA Shamrocks volunteer, or contact your local MDA office.

College Connection

Online classes allow Thomas Roy to attend his dream college

Living with Duchenne muscular dystrophy (DMD) since the age of 2, Thomas Roy has seen the disease progress throughout his life. While he had always wanted to attend Rutgers University in New Brunswick, N.J., he assumed he would never be able to do so because of his living situation.

However, when Roy found out about the online degrees Rutgers offers – and that the university would accept transfer credits from the community college he attended – everything changed. >



Thomas Roy

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“I was happy to learn they would accept the credits I earned at [community college],” Roy says. “The experience of taking classes online has been very positive. It’s very convenient, as I can log on and study at any time of day, depending on how I feel.”

Roy is currently studying labor and employment in the School of Management and Labor Relations. He plans to continue his studies after he graduates by attending law school and specializing in discrimination law. Roy says his end goal is to be a discrimination law attorney.

“I feel that discrimination law is appropriate for me, and I know I would be a good advocate,” he says.

While Roy is focused on his studies, he still has time for fun.

“I’m a big sports fan,” he says. “I like college and pro football, basketball and baseball. Whatever the season, there is a sport or favorite team to hold my interest.”

 **MDA’s Young Adult Programs is committed to supporting young adults as they move through high school, higher education, employment and independent living. Check out a video about the program at youtu.be/PoFQsRrMvrQ or visit mda.org/young-adults to learn more.**

Joe Akmakjian Continues as MDA National Ambassador in 2017



MDA is pleased to announce our current National Ambassador, Joe Akmakjian, will continue to serve in this public role in 2017. As MDA’s most visible spokesman, Akmakjian has

“2016 was such a powerful year with so many wonderful memories. I met a lot of amazing individuals and families from all over who are so excited about the direction we are headed – just like me. I also met so many staff who have a great passion for this cause. From top to bottom, this organization never ceases to amaze me.”

– Joe Akmakjian

helped forge better connections between families, engage with current and new partners, deepen MDA’s reach among millennials, and help champion improved services for individuals living with neuromuscular diseases who are transitioning from childhood to adulthood.

In addition, he has enthusiastically supported our revitalized MDA brand, our Live Unlimited campaign and bold mission plan, and he will be fully engaged in our renewed efforts to discuss our progress with partners and

supporters across the country. In 2017, Joe will expand his online influence, help deepen our connection with state ambassadors, and advocate for the MDA community in new, impactful ways.

 **Be sure to check out Joe’s 2016 year-in-review blog post on Strongly at strongly.mda.org/joe-2016-year-in-review.**

Running Together

Brothers run the Chicago Marathon for MDA Team Momentum



Brothers Jake Donnowitz (left) and Bryant Donnowitz (right) meet MDA National Ambassador Joe Akmakjian.

When Bryant Donnowitz first got involved with MDA, he didn’t have a direct connection to neuromuscular diseases. He was interested in helping MDA’s cause, but he didn’t know where to start, so he contacted his friend, April Tunnicliff, who is now MDA Team Momentum’s senior national endurance manager.

“I reached out to April, and she told me about MDA and Team Momentum. She was very encouraging, so I got involved, and I’ve been doing it for four years now,” says Bryant, who serves as a member of the Team Momentum Volunteer Committee for the Chicago market.

While the 2016 Chicago Marathon was Bryant’s fourth time running for MDA Team Momentum, it was the first MDA Team Momentum race and first marathon for his younger brother, Jake.

“It all started with [Bryant] running it,” says Jake. “Every year after he finished, he said the exact same thing: ‘I’m done.’ But he just



The National ALS Registry: Get The Facts

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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



Do I need to update my information?

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



Will my information be private?

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



YOU JOINING



More information for research

A better understanding of ALS

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



The Donnowitz brothers flex their muscles for MDA families.

kept coming back, so I had this question in my mind: 'Why does he keep coming back?'"

After seeing Bryant cross the finish line in 2015 and

embrace the MDA Team Momentum group cheering him on, Jake started to get it. And when he crossed the finish line himself in October, he completely understood.

"Seeing the kids at the end of the race, that's really what brings you back," Bryant explains, "because they're so happy to see that you're helping the cause move forward in a positive direction. That's the main thing, but also every year family members, friends and co-workers start reaching out about the marathon, and so it helps bring more people into the organization. I feel like we're kind of the glue to bring people together."

This year, Bryant and Jake were joined by two of Bryant's friends, and they raised more than \$5,000 for MDA. While

the brothers weren't ready to commit to another marathon, it seems likely this won't be their last race for MDA.

"The only way I would run a 5K or another marathon or half marathon is if it is for MDA," says Jake. "I can't see myself going through that pain for another reason."

Join MDA Team Momentum to cross the most meaningful finish line of your life. Upcoming events include the Chicago Spring Half Marathon & 10K (May 21), Colfax Marathon in Denver (May 21), Boston's Run to Remember (May 28) and Rock 'n' Roll San Diego (June 4). Learn how you can get involved with MDA Team Momentum at mdateam.org.



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Learning by Heart

Teacher Keiloni Johnson helps students in the classroom and beyond



Keiloni Johnson

Keiloni Johnson, a 39-year-old teacher from Virginia, who has spinal muscular atrophy (SMA), always wanted to work with children. When she got to college, she thought she wanted to go on to study

the law and become a family lawyer. However, that changed when Johnson took on an internship at a campus day care center as part of her studies.

"I enjoyed that a lot, and I was still working with kids," Johnson says. "I ended up changing my major from political science to family and consumer sciences. I still wanted to focus on kids, but I just didn't see myself in the courtroom."

Johnson followed up her undergraduate degree at Virginia State University with a master's degree in vocational education. From there, she started looking for teaching jobs, and she found the perfect opportunity

"I ended up changing my major from political science to family and consumer sciences. I still wanted to focus on kids, but I just didn't see myself in the courtroom."

— Keiloni Johnson

when a spot for a life planning and wellness teacher opened up at J.R. Tucker High School in Hernico, Va. Her love for the job and her students has kept her there for the past 14 years. ▶



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MDA Summer Camp has helped Keilondi Johnson and thousands of other campers develop independence and a taste for adventure.

“The school is really diverse, so every day you learn something new,” she says. “The kids are great, and they don’t look at me as necessarily having a disability. They just look at me as their teacher.”

Johnson says that just in case kids are uncomfortable or don’t know how to deal with

her neuromuscular disease, she does a PowerPoint presentation at the beginning of the school year to introduce herself to them.

“It tells a little about me and about [neuromuscular disease] and the type that I have,” she says. “It tells them about some of my limitations. In case they have questions and they’re afraid to ask, I just introduce it.”

Johnson has formed bonds with many of her students, and she points to the moments when she sees her students flourishing after they’ve taken her class as the highlight of her job.

“I have those students who come back and say they used the job portfolio [we created in my class] to get a job, and

those kinds of things make it all worthwhile,” she says.

And while teaching is her passion, Johnson also has an adventurous streak. One of her favorite activities was attending MDA Summer Camp. Now, she looks for ways to do exciting activities from her wheelchair.

“I’m always doing research on different things and their accessibility to people in wheelchairs,” she says. “Like hot air balloons – they have one for wheelchairs – or scuba diving. I haven’t done those yet, but I’m looking into it.”

 **Find more stories of people living beyond limits, as well as research news and updates about MDA resources at strongly.mda.org.**

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Passion Projects

An interview with author and filmmaker Crystal Emery

PHOTO COURTESY OF NATALIA RAMIREZ



Crystal Emery with (from left) director Bill Duke, actor Danny Glover, and Dr. Robert Ross, CEO of The California Endowment.

Author, producer and filmmaker Crystal Emery, who has Charcot-Marie-Tooth disease (CMT), wrote and directed her first play in the fifth grade, and she hasn't stopped telling stories since then. She's currently touring the country with her documentary film, "Black Women in Medicine," and her foundation, URU, The Right To Be Inc. Quest spoke with Emery over the phone about the film, her passion for the arts and more. ▶

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“I’m a storyteller, and they had extraordinary stories. My heart was moved – it wasn’t a strategic-type thing – I didn’t research them at all. It was really just a heart vibration.”
 – Crystal Emery

You’ve been writing and directing since fifth grade. How did you end up where you are now?

I was born this way. When I was in fourth or fifth grade, I would direct episodes of Green Hornet and Kato versus Batman and Robin. In kindergarten, I did my first acting in a play. It wasn’t a conscious choice; I literally was born an artist.

I was an actress, but [due to my CMT] in high school I started walking with a limp. By the time I got to college, nobody was casting or hiring an actress walking with a limp, and I had always loved directing, so I just morphed from acting to directing.

What drew you to “Black Women in Medicine” as a subject?

I started out directing feature films, and the documentary thing was something somebody challenged me on. The universe has a way of directing you where you should be. While I was in the process of trying to get a job with a well-known person in the arts, I was asked by a group of doctors to interview another doctor and through that I met these wonderful women. I’m a storyteller, and they had extraordinary stories. My heart was moved – it wasn’t a strategic-type thing – I didn’t research them at all. It was really just a heart vibration.

Why should readers check out “Black Women in Medicine”?

It’s about the trial of the human spirit. Working against all odds. People tell us in the disability community that you can’t. Or they

don’t help you or they make it more difficult because they can’t see through their limited viewpoints. “Black Women in Medicine,” and all of the doctors featured, show that it doesn’t matter where you come from or what your circumstances are – you can still overcome that.

It’s about faith and determination. I just spoke at a TEDx talk, and the talk was about the intersection of race, gender and disability, and how it’s like a car crash, yet you can ride out of those ashes. Is it easy? Hell no, it is not. My film is showing in New York now, and I can’t even go to the theater because it’s not wheelchair accessible, but did that keep me from making the film?



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What are some of the highlights of traveling the country with your film?

Having Danny Glover host the screening of your film makes it worth going to California. But it really is the response from young people who say I always dreamed of becoming something and when I heard you speak and I saw the movie, I realized I could do it.

What advice do you have for readers, especially younger readers, who want to get involved in the arts?

I would give anybody the same advice: Follow your heart. I live off my creativity, and I wake up every day loving what I do, so on the days when the wheelchair breaks, or when we rented [an accessible] van and it broke, I remember that I love what I do. If you love what you're doing, nothing can stop you.

What do you like to do for fun?

I create all the time. When I'm blowing off steam, I'm creating recipes, sitting in the kitchen telling the home health aide how to cook.

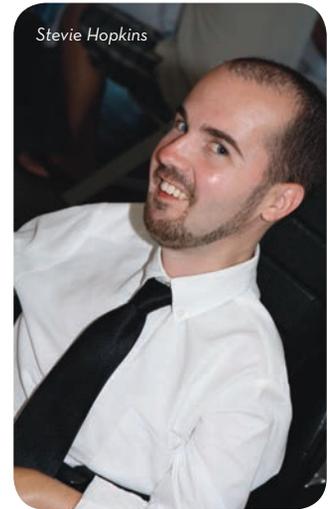
 **Learn more about "Black Women in Medicine" and Crystal Emery's foundation, URU, The Right to Be, at changingthefaceofmedicine.org. "Black Women in Medicine" also can be seen on PBS.**

Spreading Love

Entrepreneur Stevie Hopkins builds a business that is fighting for people with disabilities

In 2004, when Stevie Hopkins, a 33-year-old entrepreneur who has spinal muscular atrophy (SMA) and his sister, Annie, who also had SMA, put a wheelchair heart design on a pub crawl T-shirt, he never imagined it would eventually turn into a business that employs himself and more than 20 other employees, including a half-dozen employees with disabilities.

After the pub crawl, Annie, who had created the design, got the symbol tattooed on her shoulder, and when people saw it, they always wanted to know more about it. ▶



Stevie Hopkins



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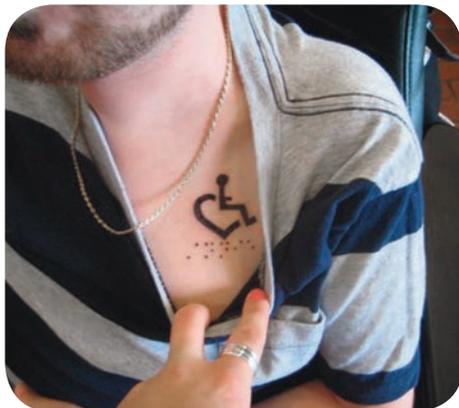


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Left: Stevie Hopkins shows off a tattoo of his sister's design. Right: Stevie and Annie.



“She showed the tattoo to people during one of her final years of MDA Summer Camp,” Stevie says, “and people were just flipping out. At the end of the camp week, I think more than 20 people went in three vehicles to a tattoo parlor to all get the same tattoo together.”

From there, Stevie and Annie copyrighted and trademarked the logo and started a company called 3E Love, which stands for embrace, educate and empower. They incorporated in 2007 and began selling T-shirts with the logo.

While that was happening, Stevie worked as a financial planner, owned a small record label and managed bands in his free time.

“My friend and I promoted concerts, probably 100 a year, and we were doing really well,” he says. “We were having the time of our lives.”

However, in 2009, a combination of events led Stevie to take some time away from the music business and financial planning and focus on 3E Love full time. In January of that year, Annie passed away, devastating Stevie and his family. The economic downturn also hurt his financial planning and music promotion businesses.

“I was burned out, so I jumped in full speed with 3E Love for the next two or three years,” he says.

In those years, Stevie grew 3E Love from a niche T-shirt business to a company that not only designs shirts and licenses out the wheelchair heart logo to other companies, but also does all of its own T-shirt printing. The fact that they design, print and merchandise in-house caught the attention of local bands and businesses, and now a significant portion of 3E Love’s income comes from sales not related to the wheelchair heart logo.

In a funny way, Stevie’s exit from the music business has come full circle, as now he is back working with local bands that he was promoting years ago.

“A local band needed shirts and they liked that I spoke their lingo, so they kept printing with us, and they referred me to a bigger band, and now I’d say 80 percent of our revenue comes from the music business,” he says.

But just because 3E Love is expanding beyond the wheelchair heart logo doesn’t mean Stevie has forgotten the company’s roots. He has launched multiple advocacy campaigns for people with disabilities, and he plans to continue to do more as his business grows.

“This year, I launched Vote With Heart, which is a lobbyist group for people with disabilities,” he says. “3E Love funds all of it. I’ve invested a lot in it, and I’m just doing what I feel is right now.”

Ultimately, Stevie would like 3E Love to have regional representatives who will go to events, speak, advocate for local policymaking and sell 3E Love merchandise. His goal is to have at least 50 percent of the 3E Love team made up of individuals with disabilities.

 **Learn more about 3E Love at 3elove.com.**
Check out Vote With Heart at votewithheart.com.

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Sign up to become an MDA advocate and make a difference

MDA is dedicated to doing everything in our power to advocate for policies and programs that help save and improve the lives of kids and adults living with neuromuscular diseases. Together, we ensure that our collective voice is heard.

MDA advocates are actively engaged in every state across the nation, and in almost every U.S. congressional district. With your help, we will continue to make a positive impact. MDA and our advocates fight for life-changing public policies, and we are proud



to collaborate with other organizations and stakeholders in the disability community to ensure the passage and implementation of policies and programs supporting the families MDA serves.

 **We can't do it without you! Sign up to become an MDA advocate and receive action alerts and policy updates. Go to mda.org/advocate to get started.**

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 **MDA Summer Camp is truly a place where kids can live unlimited. To learn more, visit mda.org/summer-camp.**

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Achieving Through Adversity

A disability is not always a disadvantage

BY ANN MOTL



I have read many articles about people succeeding *despite* their disabilities, but I believe people can succeed *because* of their disabilities.

The adversity I faced navigating academic and professional challenges as a person with a disability paved

the way for the satisfying life I enjoy now. Here I sit, a law clerk for a federal judge preparing to begin private practice at a patent litigation firm, living with a cat in my own apartment and supported by friends and family. The bumps in the road made me a tougher, smarter person and helped me sharpen the skills I use every day as an attorney.

I was diagnosed with Charcot-Marie-Tooth disease (CMT) when I began having difficulty walking at age 5. My diagnosis was challenging for my entire family. My mom had to learn how to be my advocate, fighting to ensure I had equal access to academic opportunities throughout my education. I was unaware of how much effort this took until I went to college and had to advocate for myself.

I moved three hours from home to attend the University of St. Thomas in St. Paul, Minn. I was living on my own for the first time and was in charge of my education, disability accommodations, managing my personal care attendants and doing my own laundry. Though overwhelming at times, each challenge led to an important self-development.

I majored in mechanical engineering. Sometimes my

physical limitations prevented me from performing aspects of my engineering coursework, such as assembling machines. I worked together with professors and my university's disability services office to ensure I had the necessary accommodations to participate in the full engineering coursework and college experience. Sometimes this meant I contributed to my team by acting in leadership or communicator roles. I wrote lab reports and presentations, fostering my passion for communication.

Though college provided an opportunity to discover my passions and sharpen my skills, I encountered challenges from faculty who had seemingly never met a student in a wheelchair before. The grossest example of this was a lab instructor who demanded a meeting with disability services and me when he found out I would be in his class. He railed against my inclusion, and I will never forget when he stated, "No one told me the girl in the wheelchair would be in my class!"

My mother told me that she came up against similar complaints from my high school teachers. Now it was my turn to put my advocacy skills to use. Spoiler alert: I

took his lab with my usual accommodations, and in the end I passed. Though I can't say he ever appreciated my value as a student, I eventually graduated college magna cum laude, with my engineering degree.

I'm loathe to give this instructor who didn't want me in his class any credit, but he certainly helped me sharpen my skills as an advocate, which helped me make the change from engineering to law. Indeed, I enjoyed the advocacy and communications skills I developed in college so much that I decided to seek out a career as a patent attorney, so I could combine my love for words with my technical expertise. After graduating from college, I pursued a law degree at the University of Minnesota.

Law school presented new challenges, but by this time, I embraced challenges. The lectures were unlike anything I had experienced. Students must take extensive notes while professors lecture for entire class periods. Handwriting and typing are difficult for me. I normally use voice recognition software, but this tool wasn't available during law school lectures. Fortunately, over the course of my life and especially during law school, I developed listening skills such that I can often recall a conversation word for word. These listening skills were crucial to ensuring my success in school and work.

Though I can't predict the future, I know I will continue

CONNECT WITH MDA'S YOUNG ADULT COMMUNITY

MDA is committed to supporting young adults through resources, community-building and programming as they move from high school to higher education and employment, and as they pursue independent living goals. MDA's peer-led initiative seeks to create solutions with young adults to exceed limits and unleash their fullest potential. Visit mda.org/young-adults to join the MDA young adult community and learn more about important updates and upcoming opportunities.

to face adversity, whether it be lack of affordable housing, lack of physical access, transportation difficulties or health insurance issues.

Of course, my disability is not the only challenge I face. Everyone experiences challenges and disappointments

women continue to experience discrimination in the workplace, but in the meantime, we can try to use these situations to our advantage.

We should continue to work at a societal level to implement changes that will create a more equal world.

“Sometimes this meant I contributed to my team by acting in leadership or communicator roles. I wrote lab reports and presentations, fostering my passion for communication.” – Ann Motl

throughout life. And I have learned much about overcoming adversity by listening to the struggles of people from many different backgrounds.

For example, many female attorneys face insidious discrimination at the beginning of their careers. Some attorneys have reported going to court and being mistaken for the court reporter or a non-attorney. Before opposing counsel realized they were the attorneys, these women were able to see their opponent with his guard down and gain an advantage. It's certainly unfair that

Meanwhile, I believe that an important part of meeting today's challenges is seeing the positives in negative situations.

So please don't say that I've accomplished these things despite my disability. It is because of my disability and the adversity I have experienced that I am where I sit today. [Q](#)

Ann Motl, 26, has CMT and is currently a judicial law clerk. She will practice as a patent litigation attorney beginning in fall 2017. She lives with her cat, Pixie, in Minnesota.

Mary's Marathon

MDA Muscle Walk team boasts more than 100 members



Among Mary Grace's many supporters for the MDA Muscle Walk of Cincinnati (bottom right) were her parents and three sisters (above) and four cousins (top right).

It took a while for Colleen Stratton and her husband, Charlie, to process the news that their daughter, Mary Grace, who is now 5, was diagnosed with limb-girdle muscular dystrophy (LGMD) in June 2014.

"The shock was settling in, and I was almost in denial," Colleen says.

But in the year that followed, the Strattons found support by engaging with their local MDA community. They made their MDA Muscle Walk debut with a bang at the 2016 Muscle Walk of Cincinnati last April, where their team, Mary's Marathon, was more than 100 strong.

"I'm one of 12 children, so I expected a big turnout," Colleen says, "but I was blown away. People came who didn't even know us personally — they just knew us through a friend. And it was raining, but they all showed up and then some. I had to step back, and I started crying at all of the support Mary Grace was getting."

"The name of our team is Mary's Marathon because, in our mind, slow and steady wins the race," Colleen says. "We're here to help her cross the finish line every time she can."

The team raised almost \$4,800 for MDA. And while Colleen is proud of their fundraising, the outpouring of support for her daughter is what made the event so memorable.

"We had [everyone] gathering around a backdrop for a photo," she says. "That moment stood out more than anything because so many of these people I had never even met before were there for Mary Grace. It made me feel good that we still have so many good people in this world." **Q**

Get Involved

Help bring strength to life at your local MDA Muscle Walk by starting a team like Mary's Marathon. Average Muscle Walk teams have 10 participants, but shoot for the stars. Visit mdamusclewalk.org to find your local walk, register and begin recruiting your family and friends.

Working Together

Marathon Pharmaceuticals is committed to working with patients, caregivers and advocacy groups in the effort to fight **Duchenne muscular dystrophy**.



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