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MDA
For Strength, Independence & Life

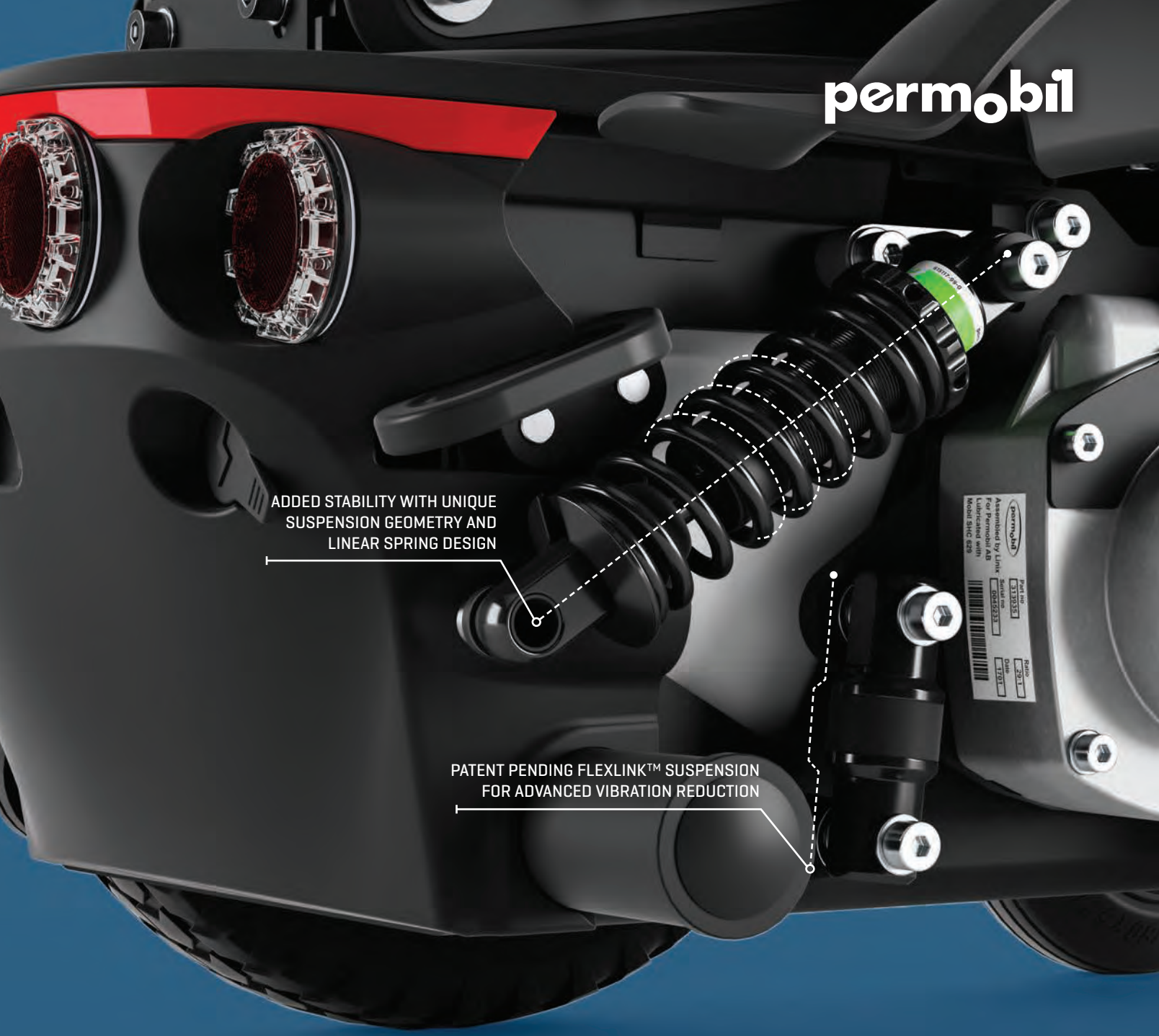
EMPOWERING FAMILIES WITH INFORMATION AND INSPIRATION

FILL THE BOOT
For many fire fighters, supporting MDA is a personal cause

ACCESSIBLE LIVING
Small changes at home make a big difference

finding breakthroughs across diseases

Following the approval of three drugs in six months, new MDA grants push for more progress



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MDA For Strength, Independence & Life

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

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

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Keeping Families at the Heart of MDA's Mission

This is a busy and productive time for MDA and the families we serve. The unprecedented approval of three new drugs in six months to treat diseases in our program — after many years of investments with no new drugs — is proof that this is a time of great momentum in neuromuscular research.

At MDA, we're building on this progress as we push to do more to help families like yours in hometowns across America. As proof of our commitment to keep you at the heart of everything we do, here are some updates on new initiatives we're developing for you and your family:

At MDA, we're building on recent research progress as we push to do more to help families like yours in hometowns across America.

Research grants: MDA recently awarded 29 new research and development grants with a total funding commitment of more than $7 million — thanks to all our fundraisers who worked so hard to raise these dollars — to support scientists who can unlock treatments and cures. Dozens of promising therapies are underway right now, and we will continue to help move these potential treatments toward the finish line. Read "Keeping Up the Momentum" on page 22 to learn more about MDA's commitment to finding breakthroughs across all neuromuscular diseases.

MDA Scientific Conference: In March, more than 500 medical and scientific leaders working to help drive scientific and therapeutic advances and discoveries gathered at MDA's biennial conference. Experts in the field shared their research and exchanged ideas to keep moving the needle for families living with neuromuscular diseases.

Clinical trial finder tool: We are making it easier for families to participate in clinical trials, which play a key role in the discovery and development of new treatments. MDA's new clinical trial finder tool at **mda.org/clinical-trials** is designed to make it easier for families to find clinical trials for which they may be eligible. We encourage you to visit the website to find trials near you and to explore additional resources.

National Community Advisory Committee: This month, our new 15-member advisory committee will meet for the first time in Washington, D.C. The committee comprises individuals with neuromuscular disease, siblings and parents, who will share their experiences to help inform and guide MDA's programs, offerings and direction.

MDA Public Policy & Advocacy Conference: On April 23-25, individuals and families will come together in Washington, D.C., at MDA's first public policy and advocacy conference, which will focus on advocating for policies and programs important to the neuromuscular disease community. Learn more about how you can participate and make your voice heard — even if you're unable to attend — on page 41.

MDA Summer Camp: MDA Summer Camp kicks off across the country next month, and our staff and volunteers are gearing up to help provide kids with the experience of a lifetime in a barrier-free environment where independence and personal development are valued and supported. MDA Summer Camp is a place where kids can truly live unlimited. Read "Summer Strong" on page 52 to learn about a young camper who formed lasting bonds with her camp counselors and how her camp family impacts her life.

Thank you for your continued partnership, volunteerism and financial support to make our shared progress possible.

Visit **mda.org** often to learn about all our programs and how you can get involved.

spring 2017

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pg 4
Melissa

MORE ONLINE

EDUCATION ESSENTIALS
Navigating the complexities of the educational system can be difficult. When your child has complex needs, it requires even more knowledge and advocacy skills. Learn the essential facts about IEPs and 504 plans and how they can help you prepare and advocate for the best education possible for your child in a *Quest* online exclusive article. **Visit mda.org/quest and search for "Education Essentials."**

MDA-018

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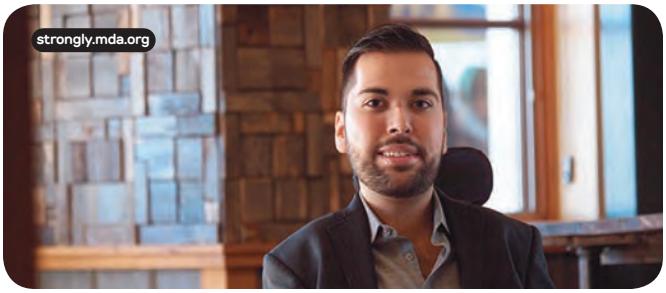
Speaking Up

Individuals with neuromuscular diseases are using social media to communicate about their everyday experiences. Here are some of the voices from MDA's young adult community on Twitter, Instagram and MDA's Strongly blog.



Joe Akmakjian, 26, has spinal muscular atrophy (SMA) and is serving his second year as MDA National Ambassador. Here's one thing he loves about the role:

"My travels in 2016 not only introduced me to new places; they introduced me to new faces. As a certified social butterfly, I enjoy spending time with all different types of people — especially new friends. It was so much fun meeting different members of the MDA family."

"When I was 5 years old, I was diagnosed with muscular dystrophy. Doctors told me that by age 13 I would be in a wheelchair. I am now 21, and I am completely mobile. In this image, I am climbing the stairs with my backpack on my back. A simple task, right? Wrong, not for me. ... Every day I must choose to climb that staircase. I choose to be the best I can be, to overcome all challenges." **#LiveUnlimited #MDA #LGMD Victoria Haire @victoriamaria_8**

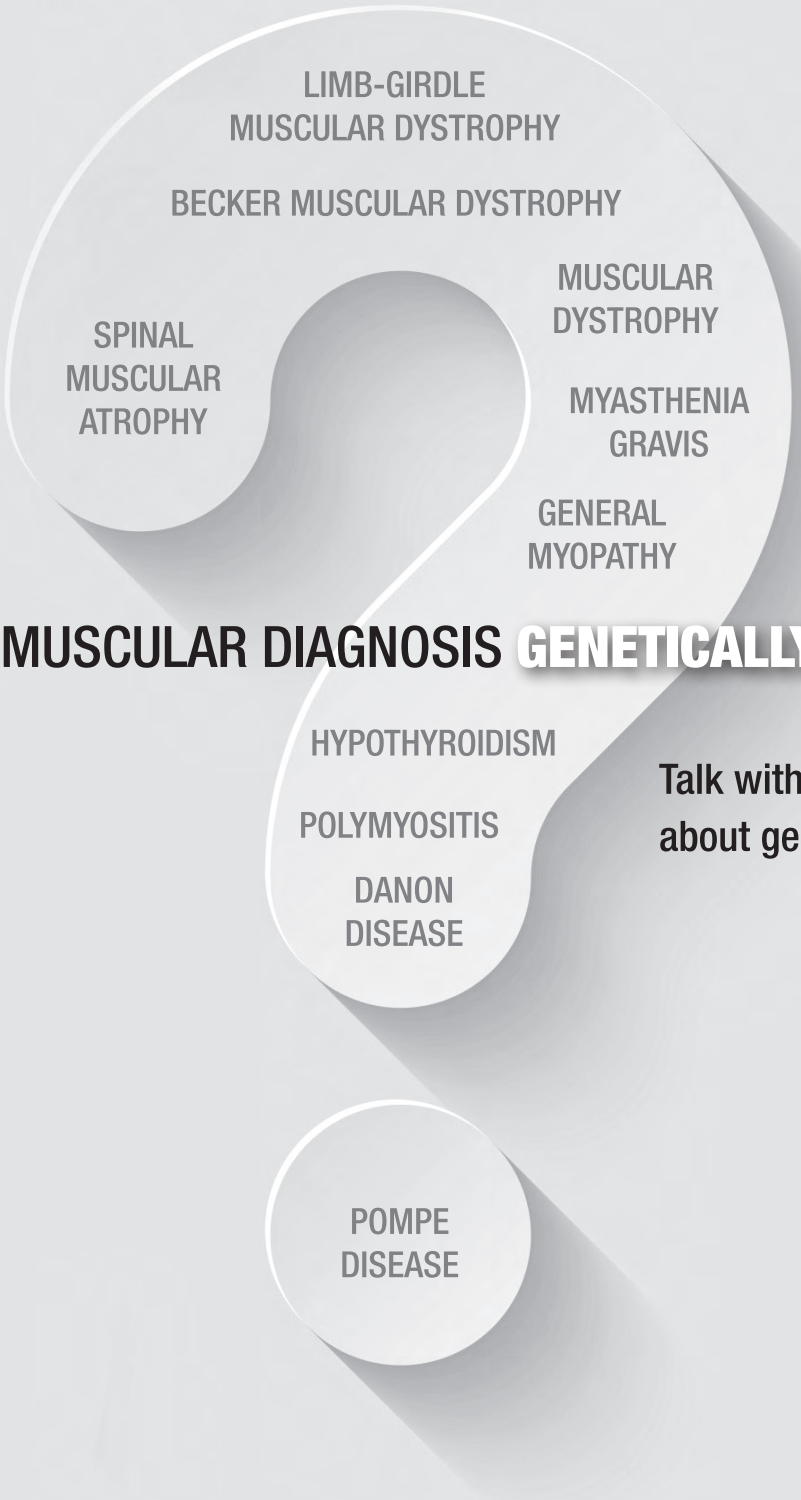
"Through and all we completed MDA's hike at El Yunque. THERE ARE NO LIMITS! Happy to have helped this queen @greshkalee cross that finish line." **#mdapr #liveunlimited anadeliafitness**

After years of being disappointed that her favorite dolls and stories didn't reflect her life, **Melissa Shang**, 13, wrote a book about a girl like her: a middle-schooler with Charcot-Marie-Tooth disease. Here, she describes the feeling that sparked the idea:

"For once, I don't want to be invisible or a side character."

Get Connected

Get and stay connected to MDA by joining us on **mda.org**. Connect with the MDA community on Facebook (**@MDANational**), Twitter (**@mdanews**) and Instagram (**mda_usa**).



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progress now

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

ALS (amyotrophic lateral sclerosis)

Participate in a Home-Based ALS Trial

Trial will assess whether disease progression can be reliably measured at home

Researchers are looking for 220 people with ALS and 30 people who do not have ALS from around the country to participate in the ALS AT HOME (ALS Testing through Home-Based Outcome Measures) study.

The aim of the study is to assess whether ALS patients can reliably measure disease progression from home, with the goal of changing the way clinical trials for ALS are performed. The hope is to be able to reduce the number of patients that have to be enrolled in a trial in order to reduce the length of the testing process. In addition, if the study shows that individuals with ALS are

If it can be shown that patients can reliably measure ALS disease progression from home, it would enable those who don't live near study centers to participate in clinical trials.

able to reliably measure their disease at home, future trials could be designed to include patients who do not live near a study center.

During the study, participants with ALS will actively track the progression of their disease from their homes (there are no clinic visits associated with the study) and will fill out intermittent surveys about their experience in the study and their ability to perform daily tasks.

With or without the help of a caregiver, participating subjects will perform measurements on themselves and record data every day for the first three months, and then twice a week for the next six months. Measurements include breathing function, hand strength, muscle quality and activity level. Equipment, as well as training in how to use it, will be provided.

To be eligible to participate, individuals must be ages 18 to 85, own a smart device (phone, tablet, etc.) with Bluetooth capability, have continuous internet access at home and meet additional eligibility criteria.

If you are interested in participating, contact study coordinator Kerisa Shelton at 602-406-6598, or visit ClinicalTrials.gov and enter NCT03016897 in the search box to learn more.

ALS (amyotrophic lateral sclerosis)

The C9ORF72 mutation is the most common genetic cause of ALS known to date. It is present in approximately 40 percent of ALS patients with a family history of ALS and 5 to 10 percent of ALS patients without a family history of the disease.

C9ORF72 ALS Study Seeks Participants

Improved understanding of the C9 form of ALS could lead to development of treatments

Researchers are looking for people to participate in a research study aimed at better understanding the specific form of ALS caused by a mutation in the gene for C9ORF72.

It's hoped that the in-depth study of patients with the C9ORF72 mutation will ultimately help in the development of treatments for this common form of ALS.

Trial participants will have as many as nine in-person visits and five telephone interviews over a period of three years. For those who live near the trial sites, each in-person visit may be tied to a regular clinic visit. For those who live out of town, one initial visit can be set up with all other visits performed via a telephone call and medical records review.

Investigators will assess clinical data to determine rates of disease progression and whether they correlate with the length of the DNA expansion in C9ORF72. Blood and optional cerebral spinal fluid samples that are collected also will be analyzed along with clinical measures.

Participants must be age 18 or older, have ALS with a confirmed C9ORF72 mutation and meet other eligibility requirements.

If you are interested in participating, call 314-362-6159 or email neuroclinicalstudies@neuro.wustl.edu. Visit ClinicalTrials.gov and enter NCT02686268 in the search box to learn more.

Encouraging Data Reported for Radicava

Experimental ALS drug is under FDA review

Osaka-based Mitsubishi Tanabe Pharma has reported encouraging 12-month efficacy and safety data for edaravone (brand name Radicava) for the treatment of ALS.

Radicava is thought to work by relieving the effects of oxidative stress (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.

Data showed that in the 48-week study, trial participants who were treated with Radicava in 10- to 14-day cycles for the full 48 weeks experienced significantly less functional loss when compared with those who received a placebo for the first 24 weeks, as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R).

Mitsubishi Tanabe submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in June 2016, seeking approval for Radicava to treat people with ALS. A decision on the NDA is expected by June 16, 2017.

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

If it receives FDA approval, Radicava would be the first drug approved to treat ALS in the U.S. in more than 20 years.

Congenital myasthenic syndromes (CMS)

CMS Study Seeks Participants

Trial will assess whether amifampridine phosphate may be an effective treatment

Researchers are looking for people with CMS to participate in a phase 3 study, being conducted by Catalyst Pharmaceuticals, to test the experimental drug amifampridine phosphate.

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

Study participants will receive an initial evaluation and several clinical exams and, after study completion, will be eligible for an expanded access program in which they will be able to continue to receive treatment with the drug.

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

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

MDA has supported the development of amifampridine phosphate for children with CMS.

For additional information, visit ClinicalTrials.gov and enter NCT02562066 in the search box. If you are interested in participating in the study, call 844-347-3277 or email EAP@catalystpharma.com.

Duchenne muscular dystrophy (DMD)

FDA Approves Emflaza

A corticosteroid, Emflaza could preserve strength in people with DMD

In February, the U.S. Food and Drug Administration (FDA) approved Marathon Pharmaceuticals' new drug applications for deflazacort (brand name Emflaza) to treat DMD in individuals age 5 or older, regardless of mutation.

Emflaza, a corticosteroid, works as an anti-inflammatory and immunosuppressant.

Treatment with Emflaza will not cure DMD, but in studies the drug has been shown to slow the loss of muscle strength and function, preserve cardiac and respiratory function, and reduce the incidence of scoliosis (curvature of the spine) in people with the disease. Importantly, the unwanted side effects often experienced with corticosteroids, such as weight gain, loss of bone mass, glucose intolerance (diabetes) and behavioral issues, may be less severe with Emflaza as compared to other steroids.

FDA approval of the two new drug applications, one for an immediate-release tablet form of the treatment and one for an oral-suspension (syrup) formulation, will now allow widespread access to Emflaza for kids and adults with DMD across the United States and make the drug among the first FDA-approved treatments for DMD.

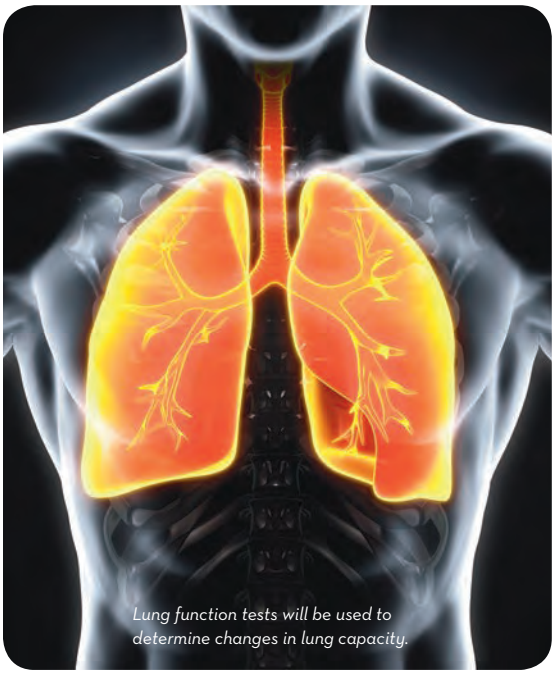
Both kids and adults with DMD may benefit from treatment with Emflaza.

MDA has a long history of supporting research and clinical study into the effects of corticosteroids, including Emflaza, on DMD, with studies to determine drug effects, mechanism of action, side effects and best dosing regimen.

At press time, Marathon announced it had reached an agreement for PTC Therapeutics to acquire and commercialize Emflaza. There was no word on how soon PTC — which is developing another drug, Translarna, for DMD — will make Emflaza available or what the drug will cost.

To find out more, visit emflaza.com, and read MDA's Strongly blog post at strongly.mda.org/deflazacort.

Duchenne muscular dystrophy (DMD)



Lung function tests will be used to determine changes in lung capacity.

Participants Sought for SIDEROS Trial

Study will assess the effects of Raxone on respiratory function

Researchers are looking for boys and men age 10 or older with DMD to participate in a phase 3 clinical trial.

The study, called SIDEROS, is designed to help researchers determine the safety and efficacy of idebenone (brand name Raxone), an experimental drug in development to treat DMD by Santhera Pharmaceuticals.

SIDEROS will assess whether Raxone, which scientists hypothesize may work by improving the way muscles utilize fuel to power movement, will slow decline in respiratory function.

Study participants will be assigned randomly to groups that will receive either Raxone or placebo. After completing the study, all participants will have the opportunity to enroll in an open-label extension study in which everyone will receive treatment with the drug.

The study will last 22 months, and during that time participants will be required to make nine clinic visits at three-month intervals. These visits will include a series of lung function tests to determine changes in lung capacity.

Trial sites are located in Alabama, Arizona, Arkansas, Florida, Indiana, Kansas, Maryland, Minnesota, Pennsylvania, Tennessee and Utah, with additional sites expected to open soon. Assistance is available for travel and hotel accommodations.

Visit greenphire.com for more information about travel and accommodations support. To inquire about participation, email SIDEROS@santhera.com. Learn more at santhera.com or visit ClinicalTrials.gov and enter NCT02814019 in the search box.

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Facioscapulohumeral muscular dystrophy **(FSHD)**

Participants Sought for FSHD Study

Study will evaluate the safety, tolerability and efficacy of ACE-083

Acceleron is developing ACE-083 for diseases like FSHD, in which improved muscle strength in a specific set of muscles may provide clinical benefit.

Researchers are looking for people with FSHD to participate in a phase 2 clinical trial, being conducted by Acceleron Pharma, to evaluate the safety, tolerability and efficacy of ACE-083.

Administered via intramuscular injection, ACE-083 is designed to increase muscle size and strength specifically in the muscles into which the drug is administered.

The study will take place in two parts, the first of which is a dose-escalation study to evaluate the safety and tolerability of ACE-083 in up to 36 FSHD patients. In the second part of the study, investigators will assess whether treatment with the drug increases muscle size, strength and function.

Travel expenses for mileage, tolls and parking will be covered. Depending on distance to the site, participants also may be covered for overnight hotel stays.

Participants must be age 18 or older, have genetically confirmed FSHD, or have a clinical diagnosis of FSHD and a first-degree relative with genetically confirmed FSHD, and meet additional criteria.

MDA is not involved with this trial but has supported foundational research in FSHD with an investment since inception of more than $22 million.

For additional information, including complete eligibility criteria, visit ClinicalTrials.gov and enter NCT02927080 in the search box. If you are interested in participating in the study, email clinicaltrials083@acceleronpharma.com.

Friedreich's ataxia **(FA)**

Disappointing Results in STEADFAST Trial

Horizon will continue to analyze trial data that could help inform future research efforts.

Treatment with Actimmune was not associated with any clinical benefit

Results from the STEADFAST phase 3 clinical trial in FA failed to show that the drug interferon gamma-1b (brand name Actimmune) was effective as assessed by any of the study's disease measurements.

Actimmune, which was under development by Horizon Pharma, is a biologically manufactured protein similar to one the body makes naturally to help prevent infection.

The study tested whether Actimmune would slow disease progression, as measured by a functional rating scale assessing capacities such as speech, ability to swallow, upper and lower limb coordination, gait and posture. However, results after 26 weeks did not demonstrate a statistically significant change from baseline between those who received the drug and those who did not. No new safety findings were identified.

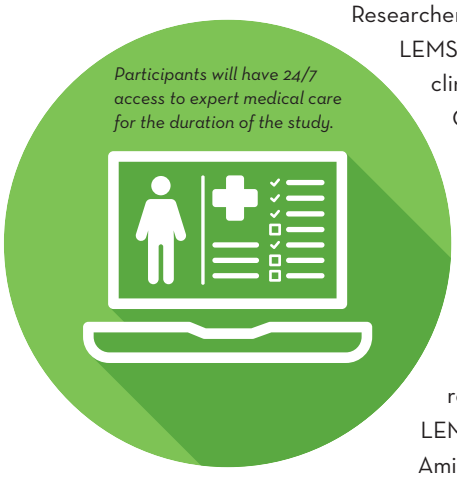
Based on the trial results, Horizon has decided to discontinue further development of Actimmune for FA.

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

Lambert-Eaton myasthenic syndrome (LEMS)

FirST-4-LEMS Study Seeks Participants

Trial will test effectiveness of amifampridine phosphate in controlling, reducing and/or eliminating symptoms of LEMS



Participants will have 24/7 access to expert medical care for the duration of the study.

Researchers are looking for people with LEMS to participate in a phase 3 clinical trial, being conducted by Catalyst Pharmaceuticals, to test the investigational drug amifampridine phosphate.

The Firdapse Strength Trial for LEMS, or FirST-4-LEMS, study is designed to evaluate the effectiveness of the drug in controlling, reducing and/or eliminating LEMS symptoms.

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

The study, which will last at least five and up to 12 days, will evaluate the effects of withdrawing amifampridine phosphate treatment from people with LEMS who currently are taking the drug.

Trial sites are located in Miami and Los Angeles. All treatment and participation costs (travel, hotel, meals, etc.) are covered.

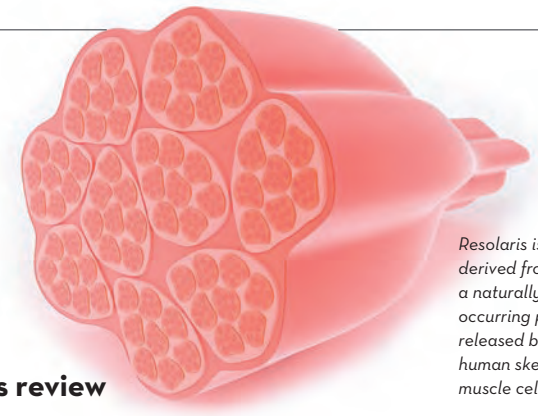
Participants must be age 18 or older, have a confirmed diagnosis of LEMS, currently be receiving treatment with amifampridine phosphate and meet additional criteria.

For additional information on this trial, including complete eligibility criteria, visit ClinicalTrials.gov and enter NCT02970162 in the search box. If you are interested in participating in the study, call 844-347-3277.

Limb-girdle muscular dystrophy (LGMD)

Resolaris Granted Fast Track Designation

Fast Track process facilitates development and expedites review



Resolaris is derived from a naturally occurring protein released by human skeletal muscle cells.

The U.S. Food and Drug Administration (FDA) has granted Fast Track designation to aTyr Pharma's experimental drug Resolaris for the treatment of LGMD2B.

Resolaris, which also is being developed to treat facioscapulohumeral muscular dystrophy, is derived from a naturally occurring protein released by human skeletal muscle cells. It potentially may provide therapeutic benefit to people affected by rare myopathies with excessive immune cell involvement — including LGMD2B patients, who experience progressive debilitating muscle weakness and atrophy as well as immune cell invasion in the skeletal muscle.

In early-stage tests, the drug has demonstrated a favorable safety profile as well as encouraging signs that it may have positive effects on muscle function.

Fast Track designation is granted by the FDA to facilitate development and accelerate the regulatory review process. It may be granted when a treatment would affect a serious condition where there is an unmet medical need, as with LGMD2B.

MDA has funded research on the role of dysferlin mutations in LGMD2B and other neuromuscular disorders since the late 1990s.

To learn more about Fast Track designation, visit https://fda.gov/ForPatients/Approvals/Fast/ucm405399.htm.

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Myasthenia gravis (MG)

Alexion Seeks FDA Approval of Soliris to Treat MG

Study results have demonstrated clinically meaningful improvements in patients treated with Soliris

Soliris is not currently approved in any country for the treatment of refractory MG, but it is approved in the United States, European Union, Japan and other countries for the treatment of other conditions.

Alexion Pharmaceuticals has submitted a marketing application to the U.S. Food and Drug Administration (FDA) for the investigational drug eculizumab (brand name Soliris) to treat people with refractory generalized MG.

A terminal complement inhibitor, Soliris is thought to work in MG by inhibiting the complement pathway to prevent destruction of the neuromuscular junction.

Refractory generalized MG occurs in a subset of MG patients. It causes muscle weakness in the head, neck, trunk, limb and respiratory muscles and does not respond to treatments that are typically helpful in other patients.

Alexion's marketing application is supported by comprehensive data from the phase 3 REGAIN study, which tested Soliris in patients with refractory generalized MG and demonstrated clinically meaningful improvements for several measures in patients treated with Soliris compared with those who received a placebo.

If approved, Soliris could address a significant unmet need for patients with refractory generalized MG who have largely exhausted conventional therapy.

Although MDA was not involved in the development and testing of Soliris, it has invested in previous research investigating complement inhibition as a therapeutic strategy for MG.

To learn more about Alexion Pharmaceuticals' development of Soliris to treat MG, visit alexion.com.

The Battery Matters

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Myotonic dystrophy (DM)

Nearly $1 Million in MDA Funding for DM Research

Award allows the Myotonic Dystrophy Clinical Research Network to continue its specialized work

Network researchers already have been responsible for critical clinical research in DM in the United States, and through combined efforts they have standardized equipment and procedures (for measuring myotonic and muscle strength, and for obtaining blood and biopsy samples) across all sites.

MDA announced the award of a clinical research network grant totaling $918,000 over three years to spur advances in DM research. This investment, which provides continued support for the Myotonic Dystrophy Clinical Research Network, will support five medical centers that specialize in DM research and clinical care.

The network is led by Charles Thornton, professor of neurology at the University of Rochester, who serves as its overall director. It was started, Thornton noted, to pave the way for testing new treatments in people.

Established in 2013 and supported by funding from MDA and other patient advocacy groups, the National Institutes of Health (NIH) and pharmaceutical company Biogen, the network's goals are to gain a more detailed understanding of the DM disease process and to collect data needed for clinical trials in order to inform what outcome measures, biomarkers and endpoints will be most appropriate.

All of the researchers in the network have free and unrestricted access to data generated at all of the sites. In addition, the network is committed to making access to study results broadly available to both academic and industry researchers in the United States and around the world.

To learn more, read strongly.mda.org/crng.

Myotonic dystrophy (DM)

IONIS-DMPK-2.5Rx Program Discontinued

Ionis and Biogen plan to move ahead with new 'LICA' technology

Ionis Pharmaceuticals reported that analysis from its completed phase 1-2 clinical trial of IONIS-DMPK-2.5Rx, an experimental compound for the treatment of DM1, showed mixed results.

In DM1, a mutation in the DMPK gene leads to the production of toxic RNA (genetic material made from DNA) that accumulates inside muscle and other cells. IONIS-DMPK-2.5Rx is an antisense oligonucleotide, designed to bind and reduce levels of the toxic RNA.

Although trial results showed encouraging trends in biomarker and splicing changes, the drug did not achieve the concentration levels in muscle needed for it to have an effect in treating the disease.

Following these results, Ionis decided not to advance its IONIS-DMPK-2.5Rx program. With partner Biogen, the company is now working on new technology called LICA (Ligand-Conjugated Antisense) in an effort to increase potency for future DM1 drugs. The LICA technology enhances distribution to skeletal and heart muscles.

Overall, the study provided a much better understanding of how future clinical trials and improved clinical endpoints may be used.

MDA has supported much of the foundational research that led to understanding the underlying molecular cause of DM1, paving the way toward the development of antisense-based and other therapeutic strategies.

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

Spinal muscular atrophy (SMA)

If it proves successful, the new approach could be combined with nusinersen (brand name Spinraza), a drug developed by Ionis Pharmaceuticals and Biogen and approved in December by the FDA to treat SMA, to boost the effectiveness of that therapy.

Antisense Approach Holds Promise

Study reveals potential new strategy to treat SMA

Encouraging results from an MDA-supported study, conducted in collaboration with Ionis Pharmaceuticals, have revealed a potential new strategy to treat SMA.

Study investigators identified a long non-coding RNA (lncRNA) present in human nerve cells, called motor neurons, that are affected in SMA. (RNA is the chemical step between DNA and protein manufacturing.) The naturally occurring lncRNA is a special type of RNA that does not encode for protein.

Investigators determined that the lncRNA represses production of the needed SMN protein (deficient in SMA) by inhibiting transcription of the SMN2 backup gene. Working with Ionis Pharmaceuticals, they developed a strand of modified DNA, called an antisense oligonucleotide, that binds to and blocks the lncRNA, and showed it could increase SMN protein production.

The work, funded by an MDA development grant and co-funded by the American Association of Neuromuscular & Electrodiagnostic Medicine Foundation for Research and Education, may lead to the development of a new treatment that could be useful by itself or in combination with other therapies in development for SMA.

To read more, see strongly.mda.org/smatreatment.

Riding High in the Saddle

Being on horseback builds strength and confidence for individuals with neuromuscular diseases

BY BARBARA & JIM TWARDOWSKI, RN

Kierra, MDA Ambassador for North Carolina, has been enjoying adaptive riding for six years.

More Online
For an extended version of this article, go to **mda.org/quest**.

Kierra, a typical 12-year-old, enjoys reading *The Hunger Games*, making silly faces to annoy her parents and riding horses. She rode for the first time at age 6. "I was afraid the horse would fall over. It's kind of scary when you get on top of something that's five feet taller than you are," says Kierra, who has Ullrich myopathy (a form of congenital muscular dystrophy) and uses a power wheelchair.

Kierra's fears quickly disappeared, and she's been enjoying adaptive riding for the past six years. "When you go fast on the horse you don't feel like you're sitting down; you feel like you're running," she says.

The steady clip clop of a horse's gait is remarkably similar to a human walking pattern: rhythmic, consistent and predictable. The repetitive nature of this movement allows riders to practice and refine their balance responses. This can lead to improved core stability and postural control.

TWO WAYS TO RIDE

No piece of equipment can duplicate the movement of a horse. Individuals with neuromuscular diseases can get the benefits of being on horseback from hippotherapy or adaptive riding (also known as therapeutic riding). Both involve riding horses, but they have significant differences:

Hippotherapy is a medical treatment strategy performed one on one by a licensed physical therapist, occupational therapist or speech therapist. The service is billable through medical insurance but not covered by all provider plans. Like all therapy sessions, there is an individualized care plan and periodic progress evaluation to determine the need for ongoing service. The American Hippotherapy Association (AHA) facilitates research on hippotherapy and provides training for therapists.

Adaptive riding is a recreational activity that teaches horseback riding skills for people with complex physical needs. A certified adaptive riding instructor works with individuals or groups. Payment for adaptive riding is an individual's responsibility, although many nonprofit facilities offer scholarships. The Professional Association of Therapeutic Horsemanship International (PATH International) recognizes 866 member centers.

Equine-centered activities are beneficial for a range of disabilities, including neuromuscular disorders, developmental delays, sensory processing disorders, genetic syndromes and Autism spectrum disorder.

Typically, the first hippotherapy or adaptive riding session involves assessing an individual's balance, coordination and physical limitations. The staff will then choose what equipment and horse is required. Helmets should always be worn, and some riders use specially adapted saddles.

GAINING STRENGTH AND CONFIDENCE

Twelve-year-old Gregory, who has Duchenne muscular dystrophy (DMD), has participated in adaptive riding for four years. He rides a pony that is 14 hands high (4 feet, 9 inches). A Velcro belt is wrapped around his waist to secure him to the saddle. A volunteer leads his horse, while a sidewalker follows on his left and an instructor on his right. Gregory loves being out of his wheelchair

and up high. His lessons are conducted along trails, and he often spots deer, foxes and red-tailed hawks.

"Gregory has limited trunk control and is very capable of steering his horse on his own," says Laurie Kelley, who is a PATH International-certified instructor. "Sitting on the horse helps Gregory work his core and upper body because he has to maintain his balance. Riding is not just physically beneficial; it's empowering for Gregory to manage a 950-pound animal."

Kierra also has felt that empowerment and freedom. During her six years of riding, she has built positive, self-affirming relationships with the staff and animals she sees every week. She credits riding with giving her the confidence she needs to live beyond her physical limitations and reach out to others as MDA Ambassador for North Carolina.

MAXIMIZING MOBILITY

When Grayson, who has DMD, began hippotherapy at the age of 4, he was unable to hold himself upright. He progressed to sitting and doing standing exercises on a horse. His occupational therapist, Linda Frease, explains the goal of hippotherapy was to keep him "as mobile as possible for as long as possible." Grayson, now 14, only recently began using a power wheelchair, and he credits hippotherapy with helping him remain ambulatory much longer than is typical for his progressive disease.

Grayson, pictured doing his favorite exercise at age 4, considers hippotherapy more play than work.

Frease explains that Grayson's therapy involved exercises focused on improving his postural stability, core strength and endurance. But to Grayson, it felt more like play than exercise. This is a common feeling among kids who participate in hippotherapy or adaptive riding. Parents and therapists report that the time the kids spend on horseback is time when they get to feel just like any other kid.

However, equine activities are not just for kids. Grown-ups can reap the benefits, too. Frease works with adults who have mobility limitations due to a number of conditions. Her oldest client is 92 years old. Q

Barbara Twardowski has Charcot-Marie-Tooth disease (CMT) 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.

THREE STEPS TO GETTING ON A HORSE

1. Consult with your MDA Care Center physician and medical team to determine if hippotherapy or adaptive riding is appropriate.

2. Find a facility within a reasonable distance of your home. Visit the American Hippotherapy Association (**americanhippotherapyassociation.org**) and the Professional Association of Therapeutic Horsemanship International (**pathintl.org**) to locate therapists, instructors and facilities in your state.

3. Meet with a therapist or instructor to discuss your goals. Ask questions such as:
   - What certifications has the individual or facility earned?
   - Is the service being provided considered hippotherapy or adaptive riding?
   - What type of documents are required to begin?
   - How long is a typical session?
   - Are sessions conducted indoors or out? What happens when weather conditions are poor?
   - What safety precautions are in place?
   - What equipment is required?

A Rider's Experience

A woman with Friedreich's ataxia (FA) thought her riding days were over. Read "Not Just Horsing Around" at **mda.org/quest** to learn about her experience with therapeutic riding.

KEEPING UP THE momentum

Following the approval of three drugs in six months, new MDA grants push for more progress

What are the effects of steroids on muscle repair in patients with Duchenne muscular dystrophy (DMD)? How can gene therapy impact people with Charcot-Marie-Tooth disease (CMT)? Will identifying biomarkers cut down on the need for muscle biopsies for those with myotonic dystrophy (DM)?

These are some of the pressing questions researchers in the neuromuscular disease field are asking — and MDA is providing support to help them find answers. Our latest efforts include the award of 29 new research and development grants to support scientists working to bring treatments and cures to MDA families.

MDA's research program has scored some big wins recently, with three drugs that can trace their origins to MDA research grants having received FDA approval. In September, the U.S. Food and Drug Administration (FDA) approved Exondys 51 for the treatment of some forms of DMD. In December, Spinraza was approved to treat kids and adults with spinal muscular atrophy (SMA). And in February, the FDA approved Emflaza to treat DMD, regardless of the genetic mutation underlying the disease.

That's why we are so optimistic about this new round of funding — because we know that any project could lead to the development of a drug that will go on, like the three we've seen in recent months, to become available treatment options for families.

TOWARD TREATMENTS AND CURES

Making up MDA's Winter 2017 grant cycle, the new awards are part of MDA's commitment to double research spending on drug development and clinical trials by 2020 as we work to make treatment options available for all the diseases in our program.

Some of the new awards are co-funded by MDA and other organizations, and reflect our efforts to strengthen partnerships and work together on shared goals.

For this latest round of awards, MDA reviewed 187 applications and had sufficient funds to approve funding for approximately 15 percent of them — 29 grants with a total funding commitment of more than $7 million. Here are some highlights:

- **Mattia Quattrocelli**, at the Center for Genetic Medicine, Northwestern University – Chicago, was awarded an MDA development grant totaling $180,000 over three years to study the effects of glucocorticoids on muscle repair and regeneration in DMD. The work, co-funded by the American Association of Neuromuscular & Electrodiagnostic Medicine Foundation for Research and Education, is expected to help improve glucocorticoid-based treatment strategies in DMD.

- **James Novak**, postdoctoral associate at Children's Research Institute, Children's National Health System in Washington, D.C., was awarded an MDA development grant totaling $180,000 over three years to examine why exon skipping drugs are most effective at getting to muscles that are actively undergoing repair. The work, co-funded by the Hearst Foundation, is expected to help optimize the effectiveness of exon skipping therapies in DMD.

- **Kleopas Kleopa**, professor and senior consulting neurologist at the Cyprus Institute of Neurology and Genetics, Cyprus School of Molecular Medicine, in Nicosia, Cyprus, was awarded an MDA research grant totaling $119,999 over a period of two years to test whether gene therapy treatment after disease onset could lead to functional improvements in CMT1X, the second most common form of CMT. The grant is co-funded by the CMT Association. (See "Running for Research" to learn more about this study.)

- **Henry Kaminski**, Meta A. Neumann Professor and Chair of the department of neurology at George Washington University in Washington, D.C., was awarded an MDA research grant totaling $367,187 over three years to test a therapeutic strategy in cell and rat models of myasthenia gravis (MG), with the

Follow the Money

Do you wonder how MDA decides how to award grants and which research to fund? Find answers to these and other questions about our grants program by searching for "MDA Grants Work to Find Breakthroughs Across Diseases" at **strongly.mda.org**.

Learn More

For more information on all the new grants, check out MDA's Grants at a Glance at **mda.org/gaag**.

intent to demonstrate the feasibility of the approach and then move to human clinical trials.

- **Thurman Wheeler**, at Massachusetts General Hospital in Boston, was awarded an MDA research grant totaling $330,000 over three years to develop biomarkers in blood or urine that will reduce the need for muscle biopsies to measure disease progression and drug effectiveness in myotonic dystrophy and other neuromuscular disorders.

At MDA, we know that successes like the approvals of Exondys 51, Spinraza and Emflaza are the result of a number of different contributions that come together at just the right time and in just the right way.

All of the new grants have the potential to lead to breakthroughs that will improve the lives of the individuals and families we serve.

MDA's current research commitment totals more than 150 research projects around the world, each of these a step forward toward treatments and cures.

SUPPORTING GROUNDBREAKING SCIENCE

As an umbrella organization, MDA is harnessing the power of a big-picture approach in our search to find the breakthroughs that will lead to treatments and cures.

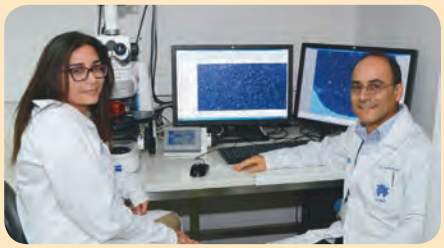
We support the world's best scientists who are working on the best science — research that has the potential to make an impact across the boundaries of all the diseases in our program.

With research investments totaling more than $1 billion over the last six decades, MDA's fingerprints are on nearly every major advance in neuromuscular disease research, and with these new awards we are continuing to fund the science that will change the neuromuscular disease landscape, usher in progress and make a difference in the lives of our families and loved ones. Q

RUNNING FOR RESEARCH

MDA supporters help fund research, such as a new study examining gene therapy in CMT

When Colin Batty takes his mark at the Boston Marathon this month, he'll be running toward real progress for his 40-plus family members who live with Charcot-Marie-Tooth disease (CMT). The funds he is raising as a member of MDA's Team Momentum help make grants like the ones we announced in March possible.



Kleopas Kleopa (right) and researcher

Batty's miles will directly support researchers like Kleopas Kleopa, professor and senior consulting neurologist at the Cyprus Institute of Neurology and Genetics, Cyprus School of Molecular Medicine, in Nicosia, Cyprus, who is investigating a gene therapy approach in CMT1X, the second most common form of CMT.

Co-funded with the Charcot-Marie-Tooth Association, the grant is the first under a partnership formed in 2016 that aims to advance CMT research, therapy development and clinical care, and increase understanding about the disease by improving education for kids and adults affected by CMT, medical professionals and the public.

With previous MDA support, Kleopa and colleagues pioneered a gene therapy approach to treat CMT1X, showing that a single lumbar injection of the gene that is mutated in the disease was associated with production of normal protein in nerves and improvement of peripheral nerve health and motor performance.

Kleopa's new work will advance and expand on this approach as his team examines whether repeated injections can lead to increased protein levels and tests whether treatment at later stages of the disease can lead to improvement similar to that seen for treatment in the early stages.

The new study illustrates MDA's big-picture approach to neuromuscular disease research, in which breakthroughs in one disease area are expected to inform advances in others.

"We hope that this study will facilitate further investigation into the potential of gene therapy for inherited neuropathies and other neuromuscular diseases," Kleopa said.

The new grant was approved by MDA's Board of Directors following careful deliberations and analysis by MDA's Research Advisory Committee, through which leading clinicians and scientists in volunteer roles oversee the peer-review process.

Research like Kleopa's wouldn't be possible without the support of MDA families and event participants, as well as partners and supporters who participate in, organize and donate to community fundraising programs such as MDA Muscle Walk, Fill the Boot and MDA Team Momentum.

MDA has funded more than $36 million in CMT research since 1950 and, including this most recent award, currently is funding 16 CMT grants with a total funding commitment of more than $4.3 million.

MDA Shamrocks: Symbols of Strength, Independence and Life

Thank you to our top 2017 MDA Shamrocks national partners! We are so grateful to these dedicated corporate partners and their caring customers and employees, as well as so many others across the country. Their support helps raise funds for muscular dystrophy research, care and support through the nation's largest St. Patrick's Day fundraiser.

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FILL THE BOOT gets personal

BY SUSAN JOHNSTON TAYLOR

Fire fighters are among MDA's most dedicated supporters. These men and women have a special connection to the cause

Matt Onyshko had been a proud member of the Pittsburgh Fire Fighters IAFF Local No. 1 for a little more than a year when he was diagnosed with ALS (amyotrophic lateral sclerosis) at the age of 27. The former college football player and father of two continued working as a fire fighter for a few more years but stopped in 2012 due to the disease's progression. He now uses a wheelchair, feeding tube and an eyegaze communication device.

Thanks to a "leave bank," Onyshko's fellow fire fighters have worked his shifts for the past five years so he can continue collecting a paycheck and receive benefits. He's eligible to receive his disability pension later this year, after 10 years on the job.

"The fire fighters have been remarkable; it's an amazing brotherhood," says Matt's wife, Jessica. "The fact that the city allows them to have this buddy system is amazing."

FROM BOOT TO BENEFICIARY

That willingness to give their time to help others extends beyond fellow fire fighters to all individuals and families living with muscular dystrophy, ALS and related life-threatening diseases. For more than 60 years, fire fighters have been among MDA's strongest supporters through the Fill the Boot fundraising campaign. In some cases, like the fire fighters at Pittsburgh Local No. 1, the cause directly affects one of their own.

Fill the Boot supports MDA research and services, including a nationwide network of more than 150 MDA Care Centers and nearly 50 centers with the MDA ALS Care Center designation. Jessica says her husband visits the MDA ALS Care Center at the University of Pittsburgh Medical Center every six months. "[The MDA Care Center] puts together the respiratory therapist and the neurologists, so we get to see all their doctors and specialists in one day without having to make multiple trips," she says. "Getting out of the house can take hours, so it's a real blessing." Fortunately, the Onyshkos don't have to travel far to their Care Center. Jessica says some patients travel from several hours away to receive the specialized care available there.

The Pittsburgh fire fighters also hold frequent fundraisers for the Onyshko family, such as charity hockey games, golf tournaments and CrossFit workouts, to help cover some of their other costs. The couple even appeared on the "Ellen DeGeneres Show" and received a home makeover and a surprise check for $50,000. "That was so much fun, and [we made] so many awesome memories," Jessica says of the experience. "Everything in my house was so plain. We pretty much lived in a bachelor pad, and they made it a home."

> *"The fire fighters have been remarkable; it's an amazing brotherhood."*
>
> *– Jessica Onyshko, Matt's wife*

PHOTO COURTESY OF POST GAZETTE

Matt Onyshko, a fire fighter with ALS, and his wife Jessica appeared on "The Ellen DeGeneres Show," along with some of the Pittsburgh fire fighters who have helped support them in their journey with ALS.

Jason Mundell

> *"Knowing that they have a fellow fire fighter with children who have a neuromuscular disease, I think it's really helped raise their awareness of it."*
>
> — Jason Mundell

LOOKING OUT FOR FAMILIES

Jason Mundell is training and safety lieutenant for Mulvane Fire Rescue, operations lieutenant for Wichita Fire Department and a member of IAFF Local No. 135 in Kansas. Mundell has been with Mulvane Fire Rescue for 24 years, but he first got involved with Fill the Boot at age 14 as a junior fire fighter because a childhood friend had Duchenne muscular dystrophy (DMD).

Mundell has been an MDA coordinator, organizing local Fill the Boot events, for more than 10 years now. He also adopted a son and daughter who both have Charcot-Marie-Tooth disease (CMT) and attend the MDA Care Center at Via Christi Medical Center in Wichita.

In addition to supporting multidisciplinary care at MDA Care Centers nationwide, Fill the Boot helps fund MDA Summer Camps across the country, and Mundell's children, both 10, love going to MDA Summer Camp. "That's something they really look forward to," Mundell says. "They talk about it all year-round: the friends they meet and their counselors." The kids love zip-lining, archery and, especially, dress-up days at camp.

The fire fighters in Mundell's department know his kids, and he thinks that helps motivate them to raise money for Fill the Boot. "Knowing that they have a fellow fire fighter with children who have a neuromuscular disease, I think it's really helped raise their awareness of it," he adds.

Roger Lopez, assistant to the general president and IAFF-MDA national coordinator, has volunteered at MDA Summer Camps for 14 years and seen the impact of Fill the Boot firsthand. "By the end of the week, [the kids] are having such a great time, they don't want to go home," he says. "It's such an amazing experience. For us as fire fighters, we've really taken this mission to heart."

GETTING INVOLVED

While fire fighters all around the country are enthusiastic about hitting the streets with boots in hand for MDA, they especially enjoy getting support from their communities. Gina Rader's son Grant was diagnosed with mitochondrial complex 1 deficiency in 2007. Since then, they've been delivering bottled water to fire fighters in Wichita during Fill the Boot events. "We've developed a special bond with the fire fighters — they almost start to become part of your family," Rader says.

Her son Grant, now 13, has been attending MDA

> *"[The fire fighters] know me, and they have been a big part of the community."*
>
> — Becky Fulcher


Becky Fulcher

GIVE MUSCULAR DYSTROPHY THE BOOT

How Fill the Boot donations help fund research, care and support for families

Since 1954, the International Association of Fire Fighters (IAFF) has raised more than $607 million to help kids and adults with muscular dystrophy, ALS and related neuromuscular diseases. To support MDA's Fill the Boot campaign, thousands of fire fighters hit the streets or storefronts seeking donations from motorists, pedestrians and others. In 2016, more than 100,000 fire fighters participated in more than 1,600 Fill the Boot events across the country, collectively raising more than $24 million.

Fill the Boot donations help fund research across diseases in MDA's program to help accelerate treatments and cures, and they help provide highly specialized multidisciplinary care for kids and adults from day one at MDA Care Centers. Contributions also help MDA provide additional services and support to families, including MDA's equipment assistance program and the MDA Resource Center. IAFF contributions also help send thousands of kids to MDA Summer Camp each year where they experience a week of freedom and independence and where they can truly live unlimited — all at no cost to their families.

To learn how you can support Fill the Boot, visit **mda.org/get-involved/fill-the-boot**.

Grant (left) delivers water to a Wichita fire fighter during a Fill the Boot event.

> *"We've developed a special bond with the fire fighters — they almost start to become part of your family."*
>
> *— Gina Rader, Grant's mother*

Summer Camp every year since his diagnosis and loves archery, crafts, swimming and making new friends. "Even a single dollar makes a difference in sending these kids to camp," she adds.

Similarly, Becky Fulcher, who was diagnosed with Friedreich's ataxia (FA) at age 8, attended MDA Summer Camp every year until she was 17. She's also attended 11 Fill the Boot events in her hometown of McPherson, Kansas. "My favorite memories are when my family would come out and hang out for a while," Fulcher says. "My aunt always brought my cousins and would give them money to drop in my boot."

Now 21, Fulcher is studying communications at Wichita State University. "It's a pretty small town," Fulcher says. "We don't have very many people affected by neuromuscular diseases." Knowing that the fire fighters of the McPherson Fire Department are working hard for her and others like her has made such an impact on Fulcher that she drives back to her hometown each year to support the department during their Fill the Boot events. "They know me, and they have been a big part of the community," she says. Q

Susan Johnston Taylor is a freelance writer based in Austin, Texas.

A Lifesaving Tradition

IAFF has been a strong MDA supporter and partner for more than 60 years. Read about how Fill the Boot started and the impact it has had over the decades at **mda.org/quest/article/giving-muscular-dystrophy-boot**.

Even small changes can make a big difference in accessibility

BY ELIZABETH MILLARD

THE COMFORTS of home

When Brenda Allen's son, Tyler, was diagnosed with Duchenne muscular dystrophy (DMD), the family had just closed on a home. Although Tyler was only 3, they immediately knew that the house wouldn't work. It lacked many accessible features, like a bedroom on the first floor and a large bathroom to accommodate a wheelchair.

Tyler

"We didn't want to keep moving as he got older, so we decided to build a new one that had features he would need later on," Allen says. "But even then, we still had to make some modifications along the way."

The new home had wider doors, a sloped walkway and a roll-in shower, but as Tyler got older, they had to tweak some aspects of the rooms to make them more accessible. For example, the builder had put cabinets under the bathroom sink, which didn't allow Tyler full access to the sink in his wheelchair, so they removed them. They also removed a shower bench, because the space became too tight when a shower chair was added.

"You learn along the way what works and what doesn't," says Allen.

FRESH IDEAS

For homeowners looking to boost accessibility, new construction isn't always an option. Fortunately, there are many DIY measures and modest renovations that can have a major impact. Here are some to consider:

- **Widen doorways by a few inches** by taking the door and surrounding molding off, suggests Amber Ward, occupational therapy coordinator at the MDA ALS Care Center at Carolinas Medical Center. A curtain on a tension rod can provide privacy, if needed. Another tactic is to install "double hinges" that allow the door to swing completely open, also increasing the width.
- **Swap out regular doorknobs with lever-style handles.** Ward says this can help people with hand weakness and limited mobility, because a door can be opened with an arm or elbow instead of the standard grip-and-turn.
- **Lower beds by using a half-height box spring**,

or even no box spring. Use wooden slats under the mattress for support.

- **Implement smart home controls** that can turn lights on and off, set thermostat temperatures, lock doors and perform other functions from a smartphone. "This can be so helpful for lighting someone's way, and they can turn off the lights once they're seated or in bed," Ward says.
- **Install a SuperPole** in areas where railings would be awkward to use, Ward suggests. This type of pole runs from floor to ceiling and assists someone rising from a seated or lying position.
- **Consider accessibility products** before making major renovations, suggests occupational therapist Susan Bachner, a fellow with the American Occupational Therapy Association. "There may be the perfect product that does just what you need, and it's often less expensive than bringing in a contractor," she says. For example, rather than ripping out a tub to put in a wheelchair-accessible shower, consider purchasing a battery-powered tub lift that can raise and lower someone into the bathtub. (See "Home Accessibility Products" for more suggestions.)

There are definitely times when hiring a contractor makes sense for a major renovation. For instance, someone may need a wheelchair-accessible ramp to the front door.

But even in those circumstances, focusing on just one or two areas of the home can keep costs down while increasing accessibility. For example, the Allens found that having a separate bathroom for Tyler with ample space for him to move around gave him a sense of independence. Focusing funds and efforts on a project like that could yield nearly as many benefits as redoing an entire house.

GETTING STARTED

Whether taking on a series of small changes or a larger renovation, like expanding a bathroom or bedroom, the first step is to understand exactly what's needed. Your MDA Care Center team members, including the physical and occupational therapists, are great resources for helping you assess your needs and offering tips on modifications that will give you the most bang for your buck.

"We often see rails in the hallway that tend to be more in the way than helpful," says Ward. "Or they're installed on the side where someone has more weakness."

Rather than implementing a wealth of modifications all at once, Bachner suggests a progressive approach, based on what's needed now and in the near future. "Observe movement, any struggles or challenges, and make note of that, so you can modify from there," she says. "Making changes based on what you think someone needs rather than what they actually need could lead to modifications that aren't used and expenses that could have been avoided."

Securing funding for home modifications may be a necessary step to get started, and resources can vary significantly by state or county. Allen suggests connecting with your local MDA office or the MDA Resource Center for advice, resources and support. "They've helped us all along the way," she says.

HOME ACCESSIBILITY PRODUCTS

If your home has some accessibility challenges, consider accessibility products. These companies offer products that can make everyday tasks easier and may prevent the need for major home renovations.

AmRamp
amramp.com
This company offers wheelchair ramps, stairlifts and other accessibility products, which can be rented or purchased. Also available are portable ramps, portable showers, overhead patient lifts, pool lifts and grab bars.

Barrier Free Lifts
barrierfreelifts.com
Their lifts, slings and bathing aids make transfers and daily tasks easier.

Mobility Lifter
mobilitylifter.com
These portable wheelchair stair climbers are battery powered and designed to aid transportation up and down stairs indoors or outside.

Open Sesame
opensesamedoor.com
This company specializes in residential automatic door openers, which make getting in and out easier for individuals who use wheelchairs or scooters.

Power Access
power-access.com
This company also makes easy-to-install automatic door openers for homes.

NuProdx
nuprodx.com
Choose from shower chairs that are stationary or wheeled, as well as tub and slider systems, in pediatric and adult sizes.

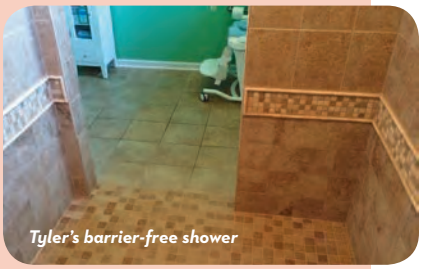
ShowerBuddy
myshowerbuddy.com
These chairs are geared for showers or baths. There are six different models ranging from pediatric to adult.

SuperPole
healthcraftproducts.com
This floor-to-ceiling pole can be installed anywhere and is especially helpful for extra support when rising from a sitting or lying position.

SureHands
surehands.com
This company offers lifting systems, body support systems, shower-bathing trolleys, safety belts, sliding sheets and other mobility products.

COMMON HOME MODIFICATIONS

Bathrooms: Often the most labor- and cost-intensive room for renovations, a bathroom can be a mix of DIY and contractor-led changes. Typical changes include installing grab bars and roll-in showers, lowering or raising toilet seats and sinks, and bringing in shower or bath chairs and benches.



Tyler's barrier-free shower

Kitchens: Many times, changing a kitchen can be done affordably, based on relocating often-used items and making cupboards or drawers easier to access. Typical changes include bringing in a rolling cart, putting dishes and glasses in low drawers, and installing pull-out shelving in cupboards.

Closets: Built-in shelving often gets in the way of wheelchairs and walkers, so it's usually better to use free-standing shelving units instead. Also, lowering or eliminating closet rods can be helpful.

Doorways: To accommodate wheelchairs and scooters, doorways often have to be widened. Some can be increased by installing offset hinges or removing molding, while others require a contractor to cut into the surrounding wall.

Fixtures: Low-hanging lights and fans may need to be repositioned if they're in the way. New smart home fixtures also can be useful, since someone can turn lights, fans and other home controls on and off from a smartphone.

A fully accessible shower helped increase Tyler's independence at home.

For the Allens, creating a more accessible space for Tyler made a huge difference in his everyday life and gave him greater independence. Now that he's 13, the family moved to a town with a better school system. Living in a rental unit while a new house is under construction, they're realizing just how important accessibility can be.

"We're seeing that accessibility features allow Tyler to feel safe, not only physically, but emotionally," says Allen. "They really do make such a big difference." Q

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

The National ALS Registry: Get The Facts

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

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

Who can sign-up?

Anyone with ALS

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

What do I need?

- A computer with an internet connection
- An email address

What if I need help?

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

What kind of information is collected?

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

Will my information be private?

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

Do I need to update my information?

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

YOU JOINING

- More information for research
- A better understanding of ALS
- The chance to help create a better future for persons with ALS

LEASE on life

Use resources and your advocacy skills to find accessible rental housing

BY CHERYL ALKON

A few years ago, Michele Boardman was ready to move into her first apartment. But Boardman, 30, who lives with limb-girdle muscular dystrophy (LGMD), had several factors to consider besides rent and location.

She had to find a place that would accommodate the modifications she needed: a front door with a number-pad lock, doors removed from her bedroom and bathroom for easy wheelchair access, under-sink cabinet doors removed for knee space and a barrier-free shower.

She found what she needed in a ground-floor apartment outside of Philadelphia. It was just 20 minutes from her parents' home as well as from the office where she is a community work incentives coordinator for AHEDD, a nonprofit human resources organization.

A PLACE TO CALL YOUR OWN

For individuals with neuromuscular diseases, finding rental housing that is, or can be made, accessible can be challenging. In many cases, organizations dedicated to fair housing or independent living may be able to help you in your search.

Brian Peters, the housing subcommittee co-chair of the National Council for Independent Living and a community access and policy specialist for Independence First in Milwaukee, notes that you should consider not just what is available in the community you want to live in, but what the housing is like. Older apartment buildings with narrower hallways and tighter bathrooms, for example, are less likely to have the space for power wheelchairs without significant modifications. Newer buildings, particularly those built after 1991, when the Fair Housing Act's rules about new construction went

KNOW YOUR RIGHTS

The Fair Housing Act protects individuals from discrimination because of race, color, national origin, religion, sex, familial status and disability when they are renting, buying or securing financing for any housing.

Here are key points of the Fair Housing Act:

- Landlords may not refuse to rent based on disability.
- It is unlawful for landlords to set different terms or conditions for individuals with disabilities.
- Landlords must allow individuals with disabilities to make reasonable accommodations to a dwelling or common areas, at the renter's expense.
- Landlords must also make reasonable accommodations in rules (for example, waiving a no-pets policy for a service dog).
- Buildings with four or more units ready for first occupancy in 1991 or later must meet certain accessibility criteria, including:
  - Accessible public and common areas
  - Doors and hallways wide enough to accommodate wheelchairs
  - Accessible light switches, electrical outlets and environmental controls
  - Reinforced bathroom walls to allow installation of grab bars
  - Kitchens and bathrooms that can be used by people in wheelchairs
  - These features are required in ground-floor units in buildings without elevators and in all units in buildings with elevators

In some places, state or local law may have more stringent standards.

If you feel your rights have been violated, contact your state fair housing agency, or file a complaint at **hud.gov** or by calling **800-669-9777**.

into effect, are more likely to have wide doorways, ramp access and other accessible features.

Once you've narrowed down your options, approach potential landlords with an open mind. "Think of it as a negotiating process, not a yes or no question," says Peters. "While landlords are obligated to make or allow reasonable accommodations or modifications, what's considered 'reasonable' can vary. Think about what works best for you. If you can't get that, what can you live with? What can the landlord live with?"

Boardman asserts this is no time to be shy. "Having an open but assertive approach is helpful," she says. "Test out your negotiating skills and know your rights."

RIGHT AT HOME

It's a good idea to know the fair housing laws confirming that individuals with disabilities are a protected class. "If you're having difficulty with a landlord, a good resource is your local fair housing organization," says Peters. "They should be knowledgeable about both the federal law and any local ordinances or state laws. They can educate the landlord about what is required."

Peters notes that any modifications a landlord agrees to are supposed to be paid for by the renter and then restored, at the renter's expense, upon moving out. But there are some exceptions.

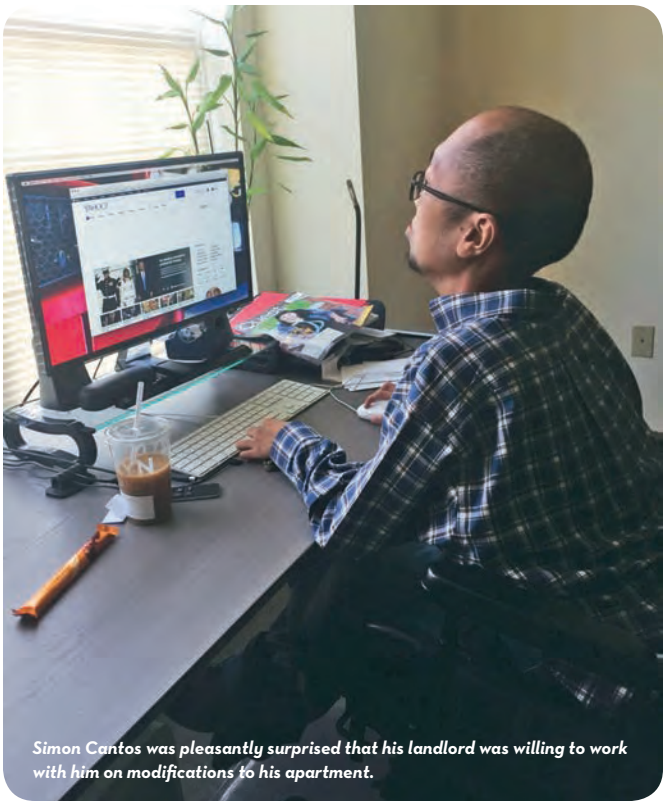
"If the property is federally funded under the Department of Housing and Urban Development or Department of Agriculture, the property owner may be required to cover the cost of reasonable modifications, like adding grab bars or creating knee space under the sink," Peters says.

And sometimes, renters are pleasantly surprised by what a landlord will do. "Some landlords are willing to negotiate or even fund some improvements if they think the person will be a good long-term tenant and they'll make that money back," Peters says.

"I didn't have to pay for any of the changes I made when I moved into my apartment," says Simon Cantos, 34, who has Ullrich congenital muscular dystrophy.

"I wanted to be able to control the thermostat through my phone, and they granted that request. All I needed to do was buy [the thermostat], and they installed it." His apartment also has low light switches that are easy to reach from a wheelchair. "The whole experience went well," he says. "I was very much blown away." Q

Cheryl Alkon is a freelance writer based in Massachusetts.



Simon Cantos was pleasantly surprised that his landlord was willing to work with him on modifications to his apartment.

TIPS FOR FINDING ACCESSIBLE HOUSING

Most states have a fair housing agency. It may be a dedicated fair housing commission or part of the state attorney general's office or a civil rights agency. In states without a centralized fair housing agency, look for the U.S. Department of Housing and Urban Development (HUD) office for your region, or find a local agency dedicated to fair housing, independent living or disability rights using the resources below.

- Centers for Independent Living: **713-520-0232, ilru.org**
- HUD Office of Fair Housing and Equal Opportunity: **800-669-9777, hud.gov**
- National Council for Independent Living: **877-525-3400, ncil.org**
- National Fair Housing Alliance: **202-898-1661, nationalfairhousing.org**

Find Resources Where You Live

Contact the MDA National Resource Center at **ResourceCenter@mdausa.org** or **800-572-1717**. MDA's trained information specialists can provide one-on-one assistance with locating resources in your community. Specialists are available Monday through Friday, 8 a.m.–5:30 p.m. Central time, and are typically able to answer questions within 24 hours of a request or the next business day.

Harley-Davidson sidecar rides are a beloved part of MDA Ride for Life.

SMILES FOR miles

Harley-Davidson riders raise money and make connections at MDA Ride for Life

BY ANDREW CONNER

When Jon Burcaw attended his first MDA Ride for Life in 1999 with his son Shane, who has spinal muscular atrophy (SMA), they were simply there to support the Harley-Davidson riders raising money and awareness for MDA. Little did he know that by 2007 he would be known as "Smilin' Jon" and have his own Harley with a special sidecar that makes entering and exiting easier for individuals with disabilities. "[My first Ride for Life] made me smile, and I've been pretty much stuck that way ever since," Jon says.

Along for the Ride

The 30th annual MDA Ride for Life kicks off May 6 from the Reading Fairgrounds in Leesport, Pa. To learn more, visit **mdarideforlife.org**.

SHARING HISTORY

MDA and Harley-Davidson Motor Co. have been affiliated since 1980, and more than 100 MDA-Harley Davidson rides are held each year across the country to benefit MDA families. MDA Ride for Life began in 1988, after members of the Eastern Harley-Davidson Dealers Association decided to combine their separate fundraisers supporting MDA into one large event. The event, which takes place over two days in different venues throughout Pennsylvania, features its namesake motorcycle ride, a concert and a parade.

"As the event has evolved, we've seen a group of fundraisers who get involved and really take the cause to heart," says Carol Schaeffer, owner of Schaeffer's Harley-Davidson in Orwigsburg, Pa., and one of the dealers who participated in the first MDA Ride for Life. "Through the years, they've been raising more and more money. In 1988, we raised something like $10,900, and last year we raised about $253,000."

In total, Ride for Life has raised more than $20 million to benefit MDA families. Jon has raised more than $210,000 in his years as a rider, but he says the connections he has made as a Ride for Life participant are just as important. In 2011, he published a novel, *The Sidecar Kings*, based on his experiences at Ride for Life and MDA Summer Camp.

"*The Sidecar Kings* was written to pay tribute to the Schaeffers, my fellow riders and MDA campers," says Jon. "Without these folks there would have been no book and I would not have been a witness to the amazing goodness that comes when you put an MDA camper into a Harley sidecar. It is a thing of magic."

MAKING MAGIC

Jon has seen the magic of helping a child with a neuromuscular disease to live unlimited through his son Shane, who was diagnosed with SMA as an infant, and has attended MDA Ride for Life and other events at Schaeffer's Harley-Davidson since he was a child. Shane is now a 24-year-old author, entrepreneur and motivational speaker.

"It's always great to see the Harley-Davidson riders who do so much to fundraise for the

LIKE FATHER, LIKE SON

Shane Burcaw (center) surrounded by (from left) his parents, Susan and Jon, his girlfriend, Hannah, and his brother, Andrew, with his girlfriend, Laura.

While Shane Burcaw still attends MDA Ride for Life with his father, Jon, he is also busy traveling the country promoting his book and charitable organization, both named for a blog Shane started as a college student, called Laughing at My Nightmare.

"I fell in love with writing in high school," he says. "With writing and humor, I was able to begin breaking down some of the social barriers that society creates for people with disabilities. ... The blog was an extension of that."

As his blog gained traction online, Shane began to receive hundreds of messages every week from people thanking him for helping them overcome their personal struggles with his unique and funny stories. Shane saw this popularity as an opportunity and created the Laughing at My Nightmare organization to help support individuals with neuromuscular diseases.

"Creating an effective, sustainable organization is no small task, which we quickly learned," Shane says of the entrepreneurial experience. "Luckily, we've been surrounded by amazingly passionate individuals who have assisted and guided us along the way. I'm so proud of how far we've come."

Shane also travels the country with his cousin, Sarah, giving motivational speeches. In the past two years, they've participated in more than 80 speaking engagements.

"Our talk shares the idea that positivity is an effective way to overcome adversity," he says. "We also speak about embracing our differences, inclusion and promoting a better understanding of disability. I use funny stories from my experience with disability to illustrate these concepts."

In addition to sharing his message, Shane enjoys the change of pace and scenery that traveling provides.

"One of my favorite experiences was flying across the country for a few speaking engagements in San Francisco," he says. "I had never been to the West Coast, so it was not only awesome to explore the beautiful city, but our speeches also went really well. Plus, I survived air travel with my wheelchair, so it was an all-around success."

Funny Pages

Learn more about Shane and read his blog at **laughingatmynightmare.com**.

Shane and his cousin Sarah

> *"We couldn't do this without our riders. And the thing that really spurs them to come out is how attached they've become to MDA families."* — Carol Schaeffer

cause," says Shane. "I remember when I was little, my favorite part was the exorbitant amount of candy I would come home with each year."

The Schaeffers and the Burcaws both look forward to this year's event, which will mark the 30th time Harley-Davidson riders and the MDA community have come together for Ride for Life. They'll be joined by hundreds of other supporters and riders, some of whom are personally connected to MDA and others who have developed bonds with MDA families through the ride.

"We couldn't do this without our riders," says Schaeffer. "And the thing that really spurs them to come out is how attached they've become to MDA families like the Burcaws. In some cases, we've watched boys with Duchenne muscular dystrophy come at 6 years old and be walking, and years later they end up in a wheelchair. And that speaks to what we want to do [with Ride for Life]: raise money for a cure to get them out of the wheelchairs." Q

Andrew Conner is a writer and editor for Quest.

A chance to advance DMD therapy

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

The *SIDEROS* study

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

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

The therapy

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

Who can participate?

- Males with DMD, any mutation
- Age 10 or older
- Ambulatory or non-ambulatory
- On corticosteroids for at least 12 months
- Forced Vital Capacity between 30% and 80%

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

santhera Pharmaceuticals

access MDA

Your guide to the MDA community

Journeys of a Lifetime

Carden Wyckoff and her brother seek adventure and raise FSHD awareness

Carden Wyckoff, a 24-year-old professional with facioscapulohumeral muscular dystrophy (FSHD), went on her first piggyback adventure in 2015 when her brother Spencer carried her on his back through the Reebok Spartan Sprint, a tough three-mile obstacle course. After their second Spartan race in 2016 — and after Spencer appeared on national TV on "American Ninja Warrior" — Carden and Spencer were approached by a friend who had co-founded the travel company Vestigo.

Carden and her brother Spencer

"He said, 'I want to partner with you guys and see you tackle the Appalachian Trail,'" says Carden, who works for a large tech company in Atlanta.

Their goal was to do the 21-mile section of the trail in Georgia, which is seven times longer than the Spartan Sprint. Although they had never done anything that demanding, Carden jumped at the opportunity and began planning the hike. In October 2016, they set off.

"It was the most remarkable experience," Carden says. "The camaraderie on the trail and the teamwork were amazing. We met a lot of people on the way. Our story got out when we were on the trail, and people would hike to find us. People found us and said they wanted to be a part of our journey. That was really cool."

With the exposure on social media and in local news, Carden felt she had an opportunity to tell more people about FSHD and created **piggybackadventures.com**.

"It's a way to raise awareness and bring in individuals to help out and be a part of that mission," she says. "And so now there's this expectation [that we'll continue doing adventures]."

Carden doesn't plan to let anyone down. She and her

brother are planning to take on Mount Kilimanjaro in Tanzania this October with other friends and family.

"It's been a dream of mine to go to Africa, and I really enjoy wildlife," Carden says. "What better way to experience it than to hike the tallest mountain on the continent with a bunch of my friends and family?"

Climbing Mount Kilimanjaro offers some significant challenges compared to the Appalachian Trail, but Carden and her crew are already preparing. Carden sees a connection between her love of planning for trips like these and her love of advocating for people with disabilities.

"Fun for me involves things like Piggyback Adventures," she says. "I love planning and traveling and breaking down those barriers and redefining what disability means."

While studying at the University of Georgia in Athens, Carden, along with some of her peers, fought to make the university's famous Arch, which many students stand underneath for photos after graduation, accessible to everyone. She continues this advocacy in her daily life and documents it on her Instagram page, where she often posts photos and videos of curb cuts in Atlanta that provide better accessibility under the hashtag **#EqualAccess**.

Learn more about Carden's upcoming adventures at piggyback adventures.com. Check out her Instagram page where she advocates for better accessibility @cardenofmilk.

MDA Hosts First Public Policy & Advocacy Conference

MDA families to advocate for policies and programs important to the neuromuscular disease community

MDA is hosting its first national Public Policy & Advocacy Conference for individuals and families living with neuromuscular diseases April 23–25 in Washington, D.C. The event will focus on policies that accelerate the development of treatments and cures, ensure access to health care and help families live unlimited. Nearly 100 individuals are expected to attend.

The conference aims to empower families to advocate for legislation, policies and programs that support the neuromuscular disease community. On April 25, attendees will meet with congressional members throughout the day to help deliver a coordinated message to policymakers on Capitol Hill.

Even if you are unable to attend the conference, we still need your help to make sure our voices are heard. Sign up today to become an MDA advocate, and you will receive email alerts on how you can take action alongside the families meeting with congressional members in Washington, D.C., on April 25, as well as emails with policy updates and ways to stay involved beyond the conference. Go to mda.org/advocate to get started.

Always Up for a Challenge

Nine-year-old takes on obstacle course to raise awareness about CMT

Nine-year-old Lily Sander doesn't remember life without Charcot-Marie-Tooth disease (CMT), but she has never let it hold her back. After undergoing major foot surgery three and a half years ago to correct the extreme curving of both feet caused by CMT, Lily had to learn how to walk again. She now walks with the help of leg braces and has been thriving.

"'I have CMT, but it doesn't have me,' is her motto," says Lily's mom, Julie Sander.

Last year, Lily participated in the Spartan Kids Race, an obstacle course for children ages 4 to 14, in conjunction with the Charcot-Marie-Tooth Association (CMTA). Lily, along with a group of her friends, conquered the half-mile course even though it was cold, windy and very muddy.

"The best part was that she experienced something she hadn't done before," says Sander. "She's big on pushing herself, so the thrill of achieving this goal was the highlight for her."

Lily will challenge herself again this year at the Spartan Kids Race on Memorial Day in Fort Mill, S.C. Her father and two brothers will join her, and she hopes even more friends will race with her this time, since it's closer to their home. "We're hoping to make it a community event," says Sander.

The 2K course (the shortest distance offered) is triple the distance of last year's race, but Lily is stronger and in better condition. "We believe staying active has been instrumental in helping to keep her disease from progressing," says Sander.

Lily has participated in gymnastics for two years, and she joined a competition team in January. She goes to training one day a week, but she plans to increase her training days

Lily surmounts an obstacle in the Spartan Kids Race.

as her body adapts. There are some things she can't do, her mom says, but Lily focuses on her strengths and dreams of becoming an Olympic gymnast. She has the drive to do it, and she doesn't like to be told she can't do something. "So much of what Lily teaches people is that the majority of limitations we have exist only in our heads," says Sander.

When Lily was first diagnosed with CMT, it was difficult for her and her family to find support. "It was like being on an island," says Sander. CMT affects about one in 2,500 people in the U.S., but there are many different types of the disease so it's not as well-known. The Sander family found support and a community through MDA by attending their local MDA Care Center and signing Lily up for this year's MDA Summer Camp.

One of the Sanders' goals in participating in events like the Spartan Kids Race is to bring attention to CMT and help others living with the disease. "We're trying to bring awareness because awareness drives funding, funding drives research, and research and drug development can dramatically impact the lives of people with CMT," Sander explains. "We want CMT to become a household name."

Cross the finish line with MDA Team Momentum. Run or walk for kids and adults with muscular dystrophy, ALS and related diseases that take away strength, independence and life. Sign up today at mdateam.org.

Impacted by Friedreich's Ataxia? Share Your Voice!

How to get involved in the Friedreich's Ataxia Patient-Focused Drug Development Meeting

An upcoming Friedreich's ataxia (FA) Patient-Focused Drug Development (PFDD) meeting with the U.S. Food and Drug Administration (FDA) is your opportunity to tell the FDA and drug developers about challenges and burdens you have experienced with FA, and share your thoughts about what is most important to you in evaluating potential new treatments for the disease.

The meeting, co-organized by the Friedreich's Ataxia Research Alliance, Muscular Dystrophy Association and National Ataxia Foundation, marks the first time patients and families affected by FA will be able to speak directly to the FDA and share their experiences in their own words. >

Information captured at the meeting, summarizing input about the patient experience from people with FA across the country, will be published in a "Voice of the Patient" report and submitted to the FDA for inclusion in the framework used to evaluate future FA therapies.

There are several ways you can get involved:

- Attend the meeting in Bethesda, Md., on June 2, from 8 a.m.–12:30 p.m., at the College Park Marriott and Conference Center.
- If you cannot attend, join online via streaming webcast and share your input on the specific panel and demographic questions.
- Keep an eye out for future communications and surveys through which you may be able to contribute your thoughts.

No one can make the voice of the FA community heard more than those impacted by the disease. Your participation is critical to making sure our collective voice makes an impact. This is your chance to make sure your input helps guide the development of successful, effective and meaningful treatments for FA.

To learn more about the meeting and how you can contribute, visit cqrcengage.com/mda/pfdd.

Clinic Connection

Dwight Koeberl fights neuromuscular disease through his work and as an MDA Jailbird

Dwight Koeberl, M.D., Ph.D

Dwight Koeberl, M.D., Ph.D., a pediatric medical genetics specialist at Duke University Medical Center in Durham, N.C., attended his first MDA Lock-Up six years ago as a guest.

"I saw a patient at the clinic [whose family] was doing the MDA Lock-Up," he says. "After I attended that with them, I have a sneaking suspicion they mentioned my name to MDA [as a potential Lock-Up participant], and that was how I got involved."

Koeberl's connection to MDA began through his work as a doctor and medical researcher studying genetics and gene therapy. He didn't necessarily expect to be working on neuromuscular disease when he first arrived at Duke University, but the timing was right, as a colleague had just developed the enzyme replacement therapy treatment for Pompe disease. Since joining Duke, Koeberl has worked on multiple grants funded by MDA. >

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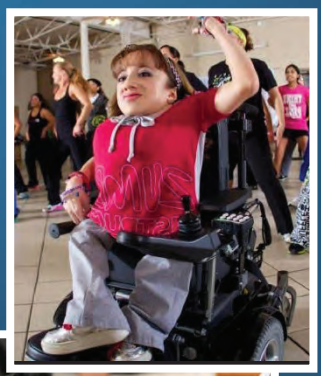

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"We've been working on gene therapy for Pompe disease since before I got my first grant," he says, "but it has evolved over time. Currently, we actually have an FDA go-ahead to do a clinical trial of gene therapy in Pompe disease."

In addition to this clinical trial, Koeberl is currently researching what he calls the "next generation" of gene therapy — a gene-editing method known as CRISPR — for which he is hoping to receive grants so he can further study the cutting-edge technology.

"The first generation of gene therapy is replacing the gene to correct the patient's muscle, and the second is to actually correct the patient's mutation in their muscle so their cells will be permanently corrected," he says.

Even though he's busy with this research, Koeberl finds time every year to raise money for MDA. He estimates he's attended around six MDA Lock-Up events and has raised around $1,000 each time. He attributes his ability to raise the funds year after year to his colleagues' connection to the cause through their research.

"I think part of the reason I'm able to fundraise is my colleagues here all understand the importance of MDA and how important [MDA Summer Camp] is for the kids, some of whom are our patients," Koeberl says.

MDA Lock-Up is a fun and inspiring community event that raises funds and awareness to help kids and adults with neuromuscular diseases. Visit mda.org/lockup, and get involved today.

Get Involved

Find your passion, and get involved in the MDA community in a way that's meaningful for you. Learn more at **mda.org/get-involved**.

Advancing Scientific Breakthroughs to Save and Improve Lives

MDA's Scientific Conference brings together experts working toward scientific and therapeutic advancements

More than 500 leaders in the academic, government, industrial and clinical arenas who work in various ways to drive scientific and therapeutic advancements and discoveries participated in the MDA Scientific Conference, held March 19–22 in Arlington, Va. Attendees gathered to share critical updates on a wide range of scientific topics.

At MDA, we work to advance scientific breakthroughs to save and improve the lives of individuals living with neuromuscular diseases. This conference marked a special time in history, as we've recently seen three drugs — all developed and tested with critical support from MDA — be granted U.S. Food and Drug Administration (FDA) approval to treat diseases in MDA's program.

To help keep the momentum going, the 2017 conference focused on better understanding disease causes, identifying new therapeutic targets and innovative technologies, and discussing new advances in preclinical and clinical research — all aimed at accelerating drug development and targeted treatments for neuromuscular diseases.

Conference presentations and discussions focused on the following areas:

- Genetics, epigenetics and gene discovery
- Approaches to precision medicine
- Understanding nuclear membranes and protein misfolding/turnover
- Gaining insights into channelopathies, dystrophin glycocomplex diseases, mitochondrial myopathies and repeat expansion diseases
- Exploring new findings in motor and peripheral neuron diseases
- Clinical trials
- Strategies and resources for academics working to move forward with a drug idea
- A career workshop for trainees
- Public policy and advocacy updates

Go to strongly.mda.org to read more about all the different ways MDA is working to uncover breakthroughs that accelerate treatments and cures.

Peak Performance

Nicki Hudson, who was diagnosed with Charcot-Marie-Tooth disease (CMT) at age 12, summited Mt. Kilimanjaro in Tanzania in 2016. Here, she's pictured at a village school, where her climbing group stopped to give out supplies. Hudson made the trek partly to show her 7-year-old daughter, who also has CMT, that nothing can stop her from reaching her goals. Read the full story on MDA's Strongly blog at **strongly.mda.org** by searching for "No Mountain Too High."

"Don't just write yourself off because you have muscular dystrophy. Let it be a motivator." — Nicki Hudson

Interdependence Day

Recovering from an injury helps redefine what it means to be independent

BY MATT CURCIO

My understanding of the concept of independence recently blew up in my face. Part of independence, for me, has been my ability to live on my own. In fact, I live 1,200 miles away from any relatives. Sure, I cannot walk very far, but a massive part of embracing my freedom has been my ability to drive, to travel and to go where I want when I want.

Well, on June 16, 2016, I stood up from my scooter, like any other day, but my body had a plan of its own. My ankle rolled, fracturing on both sides. I was devastated, and fear smacked me in the face harder than my knees hit the pavement.

I have to admit upfront that breaking any of my bones has been a deep fear since I was old enough to realize what simple injuries and illnesses can do to someone like me. I have been terrified for years about how something like a broken ankle could strip me completely of the independence I have worked so hard to attain.

I am writing this article to share with you that months and months after my accident, I am still not 100 percent healed. The injury took a lot from me: time, energy, financial stability. But it did not strip me of my independence. No, it just redefined independence for me.

Did my family have to drive cross-country to pick me up from a hospital and drive me back to their home so I could recover safely? Yes.

Did I have to take a leave of absence from a job that I love? Yes.

Was I unable to drive for more than two months and walk for more than three months? Yes.

But did that steal my independence? No.

I would be lying if I did not admit that on many days I felt like all of my independence was gone. Looking back, though, I know that my independence is not defined by my physical abilities, even though they seem inseparable.

My independence is found in my will and drive to do what I can, to accept help

"My independence is found in my will and drive to do what I can, to accept help when I need it and to embrace interdependence as my mantra." — Matt Curico

when I need it and to embrace interdependence as my mantra.

Interdependence is a term that is making its rounds throughout the disability community. The concept is simply that no person — no matter their ability — is meant to tackle life alone. The concept states that people of all abilities have something priceless to bring to the table that is human relationships.

To accommodate me, my parents literally had to tear down a wall and rearrange their lives. My pride took a lot of hits from the emotional and financial toll I was taking on my family. But that perception is quite flawed. Interdependence says that as much as I needed to accept assistance with my daily routine, my family was also depending on me to do what I could. To try when I was tired of struggling. To speak with love and encouragement when bitterness was overwhelming my spirit.

Independence the way many in our culture view it is an extremely self-seeking concept. It reverberates with a voice that says, "I don't need you; I can do this myself." While interdependence announces, "I can do this better with you at my side; let's grow together from this experience."

In many ways my injury was a nightmare, but it never reduced my worth or what I could bring to the table of human relationships. I know firsthand the fear that causes us to avoid trying something new and how even the concept of independence can be a chain that holds us down. If I could call you to any action, it would be to embrace interdependence as the true form of what human relationships are supposed to look like.

For a while, I could only see what I felt my injury took away from my already "limited" independence. Now, I see that I looked my greatest fear in the eyes and conquered it. I got an extra three or four months to see family I rarely get to see. I got to take a breath and evaluate my life and come back stronger than ever.

I had the wild idea of documenting the harshest parts of my recovery, which I did, and I posted the 10-minute mini-documentary on Facebook for my organization, Break the Roof, in November 2016. (View it at **youtu.be/bM5lo5HyupU**.) I wanted to take my experiences and give people without disabilities a behind-the-scenes look into our obstacles. More importantly, I wanted to show others like myself that if injury is your greatest fear, you have nothing left to fear.

If you have nothing left to fear, what are your deepest desires that would make you soar? Utilize interdependence to go full force after what makes you feel independent and free. Maybe it is telling stories, knitting, working with kids, raising animals or counseling friends. What matters is that it makes you feel like the fullest version of you. Go after it, and leave fear behind. Q

Matt Curcio advocates for individuals with disabilities.

Matt Curcio, 26, is a speaker, writer and advocate living with a non-specific congenital myopathy in Nashville. In 2016, he founded Break the Roof, a disability advocacy organization. For more information, visit ***breaktheroof.org*** *or* ***mattcurcio.org****. His recovery mini-documentary is also accessible through these websites.*

More Online

Read stories from around the MDA community — and share your own — on Strongly, the MDA blog. Visit **strongly.mda.org** to find personal stories from people living with neuromuscular diseases, research news, fun videos and more. If you're interested in sharing your story on Strongly, contact us at **strongly@mdausa.org**.

Summer Strong

MDA Summer Camper forms lasting bonds with her counselors

> *"I remember the first year I went, I was really shy, and they helped me realize how fun camp was and that it was worth it."* – Elvira

Elvira, a 16-year-old with spinal muscular atrophy (SMA), has been attending MDA Summer Camp near her hometown of Amarillo, Texas, since she was 7. Between campers, counselors and other volunteers, she has made a lot of friends and memories over the years, which is her favorite thing about attending camp.

"[The people I've met] have helped me so much and impacted my life," she says. "I remember the first year I went, I was really shy, and they helped me realize how fun camp was and that it was worth it."

Elvira, like many campers, has formed strong relationships with her counselors and fellow campers. Shelby Bryant and Hanna Garcia, two of Elvira's longtime counselors, have known her for years, but it didn't take long for them to develop a bond.

Elvira (bottom right) with some of the friends she made at MDA Summer Camp.

Elvira (middle) has built friendships with her counselors Hanna Garcia (left) and Shelby Bryant (right).

"You all get really close at camp, even though you're only there for a short amount of time," Elvira says. "It's because we don't care how we act in front of each other, and we're all outgoing with each other."

Garcia explains that the strong bonds she has formed with Elvira and other campers keep her coming back year after year.

"We are a pretty small Summer Camp, so it's like a family," Garcia says. "We're all so close, and seeing them have the chance to go to camp and experience everything, and seeing the smiles on their faces, it's so much fun."

While Elvira has two more years of attending MDA Summer Camp, she knows that Bryant, Garcia and the rest of her camp family aren't going anywhere. They're always there to provide support and encouragement.

"I really feel that no matter how many years it's been, we'll always be lifelong friends," Elvira says. Q

Live Unlimited at MDA Summer Camp

MDA Summer Camp is considered "the best week of the year" by campers and counselors, alike. Kids have the freedom to be kids in a place where anything is possible. Learn more about becoming a camper or volunteer at **mda.org/summer-camp**.

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For more information about Catalyst's Expanded Access Program (EAP) and/or our clinical studies, contact Catalyst at EAP@catalystpharma.com or call 1-844-347-3277. More information about both trials is also available on the Catalyst website at www.catalystpharma.com

Catalyst Cares

Catalyst Pharmaceuticals is a pharmaceutical company focused on developing prescription drugs to treat rare neuromuscular and neurological diseases, including LEMS and CMS. Catalyst has been dedicated to providing education to LEMS/CMS patients and physicians. For more information on our work, please visit us on the web at www.catalystpharma.com

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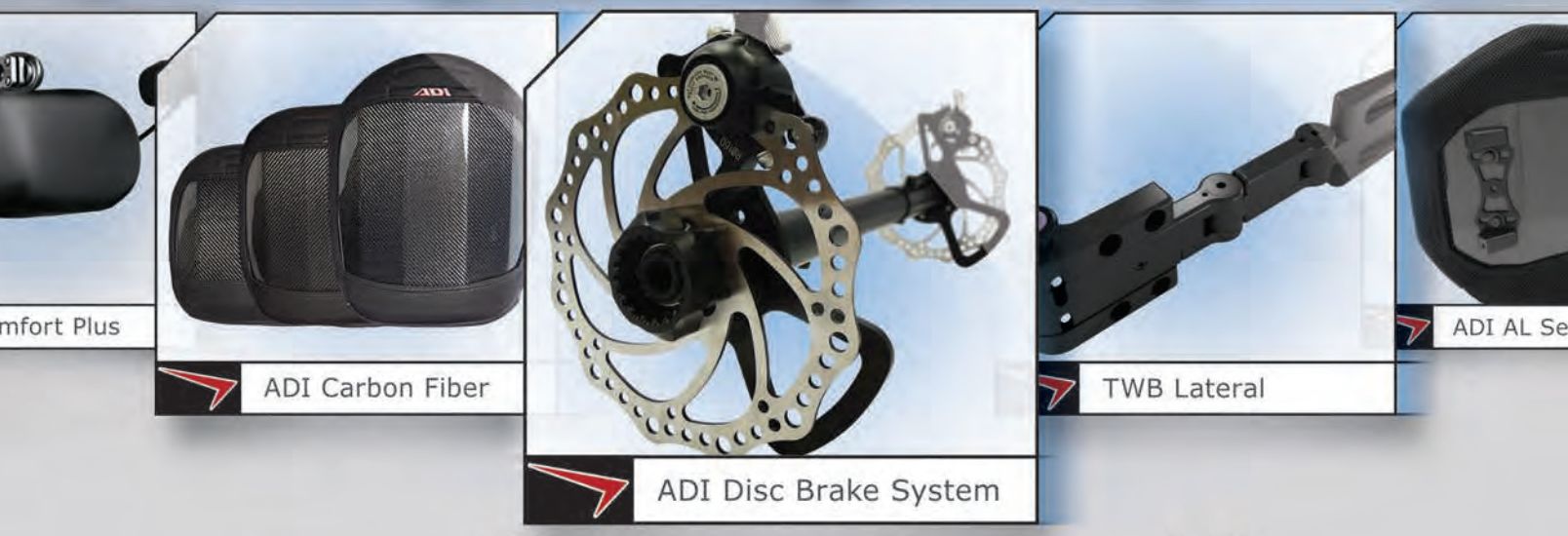

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