

# Quest

EMPOWERING FAMILIES WITH INFORMATION AND INSPIRATION

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SPRING 2016

**MDA**<sup>®</sup>  
For Strength,  
Independence & Life

## *camping* *unlimited*

Elizabeth Barrett gets as much as she gives as an MDA Summer Camp volunteer

**INVESTING IN THE FUTURE**  
Tips for financial planning

**SEARCHING FOR ANSWERS**  
MDA's latest grants work across diseases toward breakthroughs



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Special thanks to Kimia & Matt Othick and the entire team at San Diego's Crust Pizzeria on Carmel Mountain Road.

Athlete, inspirational speaker, T10 paraplegic, and pizza connoisseur Jeremy McGhee delivers the goods during a night out at Crust Pizzeria in San Diego. You can read Jeremy's blog and follow his adventures at <http://www.JeremyMcGhee.com>.

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MDA is leading the fight to free individuals – and the families who love them – from the harm of muscular dystrophy, ALS and related muscle-debilitating 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](http://mda.org).

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## Keeping Families at the Center of Our Mission

**O**ne of the most enjoyable privileges of my role is visiting with MDA families we proudly represent and are committed to serving. You are at the heart of everything we do at MDA. You've heard us say that, but what does it mean to families like yours?

Earlier this year, MDA launched a revitalized brand to better reflect the voices and needs of families and a bold plan to enhance the research, care and support we provide to you. This includes the experience and care you receive at MDA Care Centers (formerly called MDA Clinics) in hometowns across America.

Last month, MDA brought together 450 neuromuscular disease clinicians, allied health professionals and scientific experts at our MDA Clinical Conference to discuss new and better ways to provide care to families and enhance outcomes that will save and improve lives for the kids and adults we serve.

We are very encouraged by the robust exchange of ideas and collaboration between MDA and medical and scientific experts to achieve the highest quality and consistency in experience for families no matter where they live. We look forward to working together with families, health professionals and health care institutions in the coming months to improve our approach.

We will be sure to share those plans on [mda.org](http://mda.org) and in *Quest* and will alert you when they are ready. Some of the improvements you can expect at MDA Care Centers in the coming months include:

- **More families engaged** in playing an active role in the clinical care process to create a customized, meaningful experience based on individualized needs.
- **Increased quality and consistency** across all MDA Care Centers with new best practices for care that each clinic must meet to be considered an MDA Care Center.

- **Working with experts** to ensure more MDA Care Centers are prepared to serve as clinical trial sites, which will pave the way for more individuals and families to participate.

We know progress can't come soon enough for families. That's why we're working to strengthen our multidisciplinary team approach and our emphasis on family and individual-centered care. We are grateful for your ongoing feedback and look forward to creating the best experience with and for you.



Starting next month, MDA Summer Camp season will begin across the country, providing kids with the experience of a lifetime in a barrier-free environment where independence is valued and supported. MDA Summer Camp brings joy and affirming connections to thousands of kids each year, and it's a strong example of how we're working to give kids strength, independence and life.

Our cover story on page 20 details how one woman's tragic loss has strengthened her commitment to helping kids live beyond limits at MDA Summer Camp. Because of our dedicated volunteers and staff, kids can experience the thrill of doing things they never dreamed possible while gaining the confidence and skills to live unlimited. As an extension of camp, we are working with communities to set up young adults for success and ensure there are more meaningful opportunities for kids as they live longer, stay stronger and transition into adulthood.

Thank you for your commitment to MDA's vital cause. Please join us in keeping up the momentum at [mda.org](http://mda.org).

Steven M. Derks  
President and CEO  
Muscular Dystrophy Association

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# spring 2016

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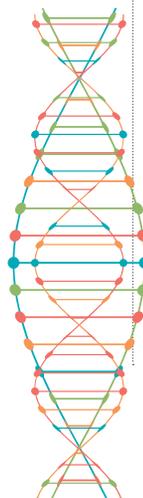
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MDA Team  
Momentum goes strong in 2016.

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Read an excerpt from Darcy Leech's autobiographical novel, *From My Mother*, a coming-of-age story about a healthy young woman in a family affected by neuromuscular disease. Visit [mda.org/quest](http://mda.org/quest) for this and other stories.

**FINANCE Q&A**  
Learn about taxes, asset development, types of federal benefits and more in a web exclusive Q&A with an expert on finance for people with disabilities. Check it out at [mda.org/quest](http://mda.org/quest).



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## ALS Allies

ALS takes away the freedom to walk, to talk, to run and dance. To laugh, to hug. To eat. To breathe. MDA fights to give those freedoms back – to give people a lifetime to live unlimited. Every year, MDA contributes millions of dollars to fighting ALS and finding breakthroughs to accelerate treatments and cures. Here are some facts and figures that show the strength of MDA's support.

## IMPACT

Approximately **12,000** individuals with ALS have access to MDA ALS Care Centers

**\$2.7 million** Total funding commitment for new ALS grants awarded by MDA in 2015

**43** Number of MDA ALS Care Centers

**\$153 million+** Total amount MDA has invested in ALS research since its inception

**37** Number of MDA's active ALS grants

**\$44.8 million** Total funding commitment for ALS grants active in the last five years

**12** Number of new ALS grants awarded in 2015

Since 1950, MDA has invested more than **\$354 million** in ALS research and support services.

**Continue the fight**  
For more information about ALS and how you can help support the MDA ALS community, visit [mda.org](http://mda.org).



*"If I had all my physical strength and everyday freedoms from before being diagnosed with ALS, I would want to give one more bear hug and kiss to each of my girls. Being able to physically pick them up and squeeze them in my arms is what I miss most in life. MDA and its wonderful, compassionate and helpful staff are an essential and integral part of my life, as well as others like mine."*

– Keith Gawrick, father of three daughters who is living with ALS

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# progress now

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

## Amyotrophic lateral sclerosis (ALS)

### Evaluating IONIS-SOD1Rx

Participants sought for early-stage trial



*Ionis-SOD1Rx is an antisense oligonucleotide, composed of short segments of synthetic genetic material (nucleic acid) that bind to RNA.*

Researchers are looking for people with **ALS** to participate in a phase 1 clinical trial, sponsored by Biogen and Ionis Pharmaceuticals, to test the experimental drug Ionis-SOD1Rx.

Ionis-SOD1Rx is designed to block production of flawed SOD1 protein in people with

ALS caused by defects in the SOD1 gene. It is administered by intrathecal injection, in which a small needle is inserted into a space in the lower back below the end of the spinal cord in a medical procedure commonly referred to as a “lumbar puncture.”

The goals of the study are to determine the safety and tolerability of the drug in people with ALS and determine how the drug distributes throughout the body.

The study is taking place at 17 trial sites in the United States, Canada and Western Europe. Of note: Participants can have either the familial or sporadic form of ALS to participate in different parts of the trial.



**For additional information on this trial, including site locations and inclusion and exclusion criteria, visit [ClinicalTrials.gov](http://ClinicalTrials.gov) and enter NCT02623699 into the search box.**

# VITALITY-ALS

Late-stage clinical trial seeks participants



VITALITY-ALS is designed to assess tirasemtiv's effects versus placebo on measures of respiratory function in ALS.

Researchers are seeking people with ALS to participate in the phase 3 VITALITY-ALS clinical trial, sponsored by Cytokinetics, to test the experimental drug tirasemtiv.

Tirasemtiv is a skeletal muscle activator that is designed to increase the sensitivity of muscle fibers to calcium, which should cause these fibers to contract even if the signal from the nervous system is weaker than normal. It's expected to result in a potential increase in muscle force generation.

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

The study is taking place at trial sites throughout the United States and Canada.

Participants must be 18 years or older, have received a diagnosis of ALS within 24 months of starting the trial and meet additional eligibility criteria.

 For additional information on this trial, including inclusion and exclusion criteria, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT02496767 into the search box.

# NurOwn Trial

BrainStorm Cell Therapeutics' NurOwn stem cell technology may improve breathing, swallowing and muscle strength in people with ALS

BrainStorm Cell Therapeutics in January published the results of its phase 1-2 and 2a clinical trials conducted in Israel to test its NurOwn stem cell technology in which stem cells derived from an ALS patient's bone marrow are modified to secrete neurotrophic factors before being transplanted back into the patient's spinal cord, muscle or both.

The trials, which did not include placebo groups, were designed to evaluate the safety and tolerability of this approach and included 26 patients in total.

Overall, the procedure and the transplanted cells were safe and well-tolerated, with only minor adverse effects noted in some patients. In addition, investigators found that treatment was associated with improvements in forced vital capacity (FVC), a measure of breathing capacity, and in the ALS Functional Rating Scale Revised (ALS-FRS-R), a measure of overall function for ALS patients.

BrainStorm currently is conducting an ongoing, double-blind, placebo-controlled trial in the United States using an identical treatment protocol. The trial may yield further insight into whether these stem cells could be a safe and effective therapy for ALS.

 For additional information on the U.S. trial, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT02017912 into the search box.



**Congenital muscular dystrophy (CMD)**

# TXA127 Granted Orphan Drug Status for CMD

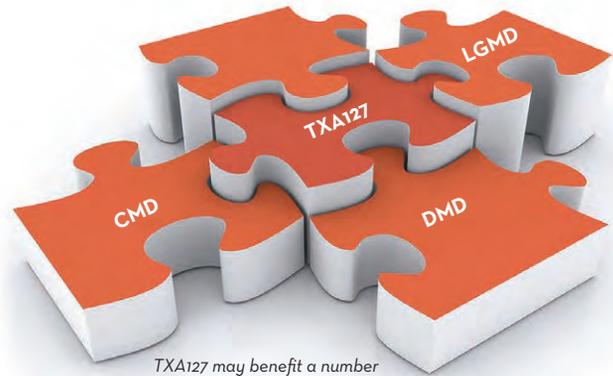
Tarix Orphan’s lead compound is under development for a number of muscular dystrophies

The U.S. Food and Drug Administration (FDA) has granted Orphan Drug status to Tarix Orphan’s lead compound, TXA127, for the treatment of laminin-deficient **congenital muscular dystrophy (MDC1A)**.

TXA127 is a pharmaceutical grade formulation of the naturally occurring peptide Angiotensin (1-7), which Tarix Orphan is developing to treat a number of orphan and genetic diseases, including congenital muscular dystrophies, Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophy (LGMD). The drug is thought to counteract pathways that cause fibrosis (scarring) and inflammation in muscles and has shown therapeutic activity in animal models of DMD, LGMD, and MDC1A.

Orphan status is granted by the FDA to promote the development of promising products for the treatment of rare diseases.

MDA has funded research into modulation of the angiotensin system to prevent fibrosis in muscular dystrophies since 2009. Most recently, a grant to Mahasweta Girgenrath at Boston University will test Tarix’s Angiotensin (1-7) together with growth



*TXA127 may benefit a number of genetic diseases including several muscular dystrophies.*

hormone in MDC1A mice to determine if combination therapies may be effective in treating this disease.

 **To learn more about Tarix Orphan’s development of TXA127 to treat CMD and other muscular dystrophies, visit [tarixorphan.com](http://tarixorphan.com).**

**Duchenne muscular dystrophy (DMD)**

# Myostatin Clinical Trial

Investigators seek to enroll boys, ages 6-9 years, with DMD



Pfizer is looking for boys to participate in a phase 2 clinical trial designed to evaluate the safety, tolerability, efficacy, pharmacokinetics (the drug levels in the body) and pharmacodynamics (effects the drug has on the body) of the investigational drug PF-06252616 in **Duchenne muscular dystrophy (DMD)**.

Boys ages 6-9 years who have been diagnosed with DMD both clinically and via genetic testing, and who can walk, may be eligible to participate.

PF-06252616 is an engineered protein designed to bind to and inhibit myostatin, a naturally occurring protein in muscle that is normally produced by the body to keep muscles from growing too large.

Current sites in the United States are located in California, Colorado, Florida, Iowa, Maryland, Minnesota, Missouri, North Carolina, Ohio, Pennsylvania and Utah. Outside the United States, trial sites are located in Canada, Japan,

the United Kingdom and Italy. Additional sites are under evaluation and will be listed on **ClinicalTrials.gov** when active. Reasonable travel and accommodations will be provided or reimbursed.

 **To learn more, or to request more information, please visit the study website: [dmdmyostatintrial.com](http://dmdmyostatintrial.com). Or, please visit [ClinicalTrials.gov](http://ClinicalTrials.gov) and enter NCT02310763 into the search box.**

# HALO Trial

Dosing and new patient enrollment suspended



Akashi Therapeutics suspended dosing and new patient enrollment in the phase 1b-2a HALO trial, a study evaluating the experimental drug HT-100 in people with DMD following a medical emergency experienced by one of the trial participants. The participant, who was receiving the highest dose in the study,

experienced serious, life-threatening health issues before passing away.

Akashi is working with the U.S. Food and Drug Administration (FDA) to determine whether the patient's health issues were related to HT-100. The company will provide additional information, including the impact on the future of this trial, once its investigation is complete.

HT-100 is a small molecule drug designed to reduce fibrosis (scarring) and inflammation, and promote healthy muscle fiber regeneration in DMD.

Families of patients who had been participating or planning to participate in the HT-100 trial are encouraged to contact Akashi at [trialinfo@akashirx.com](mailto:trialinfo@akashirx.com), the principal investigator at their clinical site, or their physician with any questions or concerns.



To learn more about Akashi Therapeutics' development of HT-100 to treat DMD, visit [akashirx.com](http://akashirx.com).

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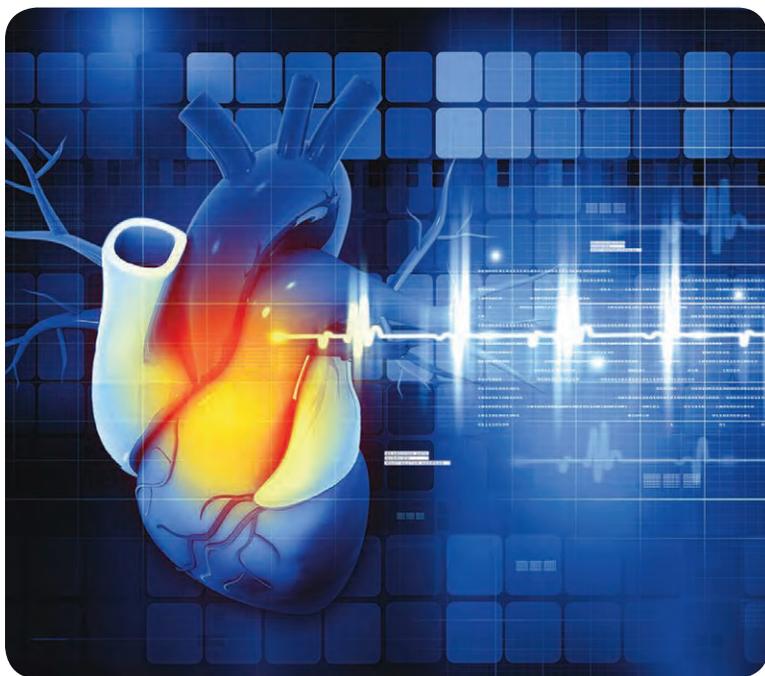
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Duchenne muscular dystrophy (DMD)

# HOPE Trial

Investigators will look at effects of CAP-1002 on DMD-associated heart disease



Researchers at the Cedars-Sinai Heart Institute in Los Angeles are looking for boys and men with DMD to participate in a new early-phase study, sponsored by Capricor Inc., called “HOPE-Duchenne.”

The Halt cardiomyopathy Progression in Duchenne study will test an investigational cardiac cell therapy called CAP-1002 to determine if it is safe and potentially effective in treating boys and men with DMD-associated heart disease.

Study participants will randomly be assigned to receive either treatment with CAP-1002 or standard care (no infusion) over a period of one year. Those assigned to receive CAP-1002 will be administered the treatment (infused into the arteries of the heart) in a hospital setting.

Investigators will measure the amount of scar tissue in the heart before and after treatment with either CAP-1002 or standard care.

Recruitment is open at the Cedars-Sinai Medical Center trial site in Los Angeles, with additional trial sites expected to open soon. Support for travel and hotel accommodations is available for study participants.

 **To learn more about this trial, including additional eligibility criteria, or to request more information, please visit the study website: [capricor.com/hope](http://capricor.com/hope) or go to [ClinicalTrials.gov](http://ClinicalTrials.gov) and enter NCT02485938 into the search box. Or, contact the research team (Mo, Matt or Ryan) at 310-248-8080, [Matthew.Hakimi@cshs.org](mailto:Matthew.Hakimi@cshs.org) or [Mohamad.Rashid@cshs.org](mailto:Mohamad.Rashid@cshs.org).**

# Revised Advisory Committee Date, Action Date for Eteplirsen

Extension follows submission of new clinical effectiveness data

The U.S. Food and Drug Administration (FDA) has rescheduled the Advisory Committee meeting to review eteplirsen for the treatment of DMD for April 25, 2016. This follows news that the agency extended the PDUFA goal date for eteplirsen by a standard extension period of three months. The new date by which the FDA must make a decision about whether to approve eteplirsen is May 26, 2016.



The extension provides time for the FDA to complete a full review of eteplirsen. It follows Sarepta’s submission of new four-year clinical effectiveness data, which has been designated as a major amendment to the New Drug Application for eteplirsen.

Eteplirsen is an “exon-skipping” drug that targets a section of DNA called exon 51, and may help up to 13 percent of DMD patients. Exon skipping is a treatment strategy in which sections of genetic code are “skipped,” allowing cells to manufacture partially functional dystrophin, the muscle protein missing in DMD. Exon skipping is not a cure for DMD, but potentially could lessen the severe muscle weakness and atrophy that is the hallmark of the disease.

 **For more about Sarepta’s development of eteplirsen to treat DMD, visit [sarepta.com](http://sarepta.com).**

# Expanded Access

## ACCESS DMD provides deflazacort to U.S. patients

Marathon Pharmaceuticals has reported that it is expanding patient access to deflazacort, its investigational therapy for DMD. Medical centers across the country are now participating in the open-label expanded access program (EAP) called ACCESS DMD to provide deflazacort to qualified U.S. patients at no charge.

Deflazacort, a corticosteroid, works as an anti-inflammatory and immunosuppressant. Based on published clinical studies, it appears that deflazacort may be an important new treatment option for patients with DMD. Side effects reported to date include cushingoid appearance, abnormal hair growth, weight gain, skin redness, nasopharyngitis, irritability and cataract formation.

In the United States, deflazacort is considered an investigational therapy, as it has not been approved by the U.S. Food and Drug Administration (FDA).

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



Expanded access programs provide a mechanism for early access to an investigational drug to treat patients with a serious or immediately life-threatening disease or condition that has no comparable or satisfactory alternative treatment options.



To learn more about ACCESS DMD, including a list of clinical sites participating in the program, visit [AccessDMD.com](http://AccessDMD.com), or call (844) 800-4363.

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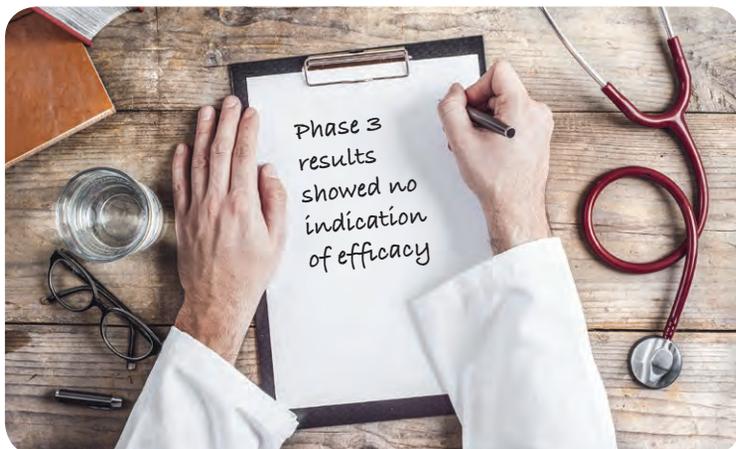
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Duchenne muscular dystrophy (DMD)

# Tadalafil Trial



Eli Lilly reported results from its phase 3 study of tadalafil (brand name Cialis) in approximately 330 patients with DMD.

Analysis of the initial data from the placebo-controlled, double-blind period of the trial, did not show any evidence of

efficacy for tadalafil to slow the decline in 6 Minute Walk Distance (6MWD) compared with placebo through 48 weeks. The study also showed no evidence of efficacy in other secondary assessments of motor function, including the North Star Ambulatory Assessment and timed function tests (10 meter walk/run, rise from floor, and 4-stair climb). The complete efficacy and safety data from the trial will be reviewed

over the coming weeks.

Because the trial provided no evidence that once-daily tadalafil treatment has a meaningful effect to slow disease progression compared with placebo, the open-label extension (OLE) phase of the study has been stopped. All investigators were informed of the decision and provided with guidance on discontinuing the patients from the study.

 **To learn more about Eli Lilly's development of tadalafil for DMD, visit [lilly.com](http://lilly.com).**

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## Friedreich's ataxia (FA)

# Retrotope Advances RTOO1

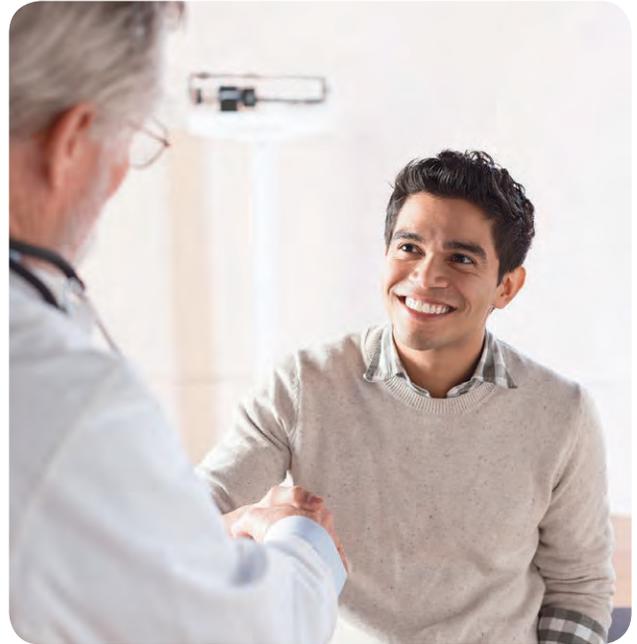
### Opens enrollment for highest-dose cohort in ongoing study

Retrotope Inc. has successfully completed the first dose cohort and has opened enrollment for the highest-dose cohort in its ongoing 28-day study of orally dosed RTOO1 in **Friedreich's ataxia (FA)**. The company reported that RTOO1 has been well-tolerated and no serious adverse events or toxicities were observed.

The phase 1-2 clinical trial is designed to test the safety and tolerability of RTOO1 in people with FA.

In FA, free iron associated with the disease contributes to degradation of lipids in mitochondrial and cellular membranes. A chemically stabilized form of a natural membrane fatty acid that is resistant to lipid peroxidation, RTOO1 is designed to stabilize cellular membranes against attack and restore cellular health.

Trial sites are located at the University of South Florida in Tampa, and the Collaborative Neurosciences Network in Long Beach, Calif.



 For more information on this study, visit [ClinicalTrials.gov](https://clinicaltrials.gov) and enter **NCT02445794** into the search box.



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## Hereditary inclusion-body myopathy (HIBM)

# Encouraging Results for Ace-ER in Phase 2 Trial

Extended release aceneuramic acid appeared to slow loss of muscle strength

A phase 2 double-blind trial funded by Ultragenyx Pharmaceuticals showed that treatment with acid extended release (Ace-ER) tablets led to dose-dependent increases in sialic acid levels and slowed loss of muscle strength in individuals with GNE myopathy.

GNE myopathy, also known as **hereditary inclusion-body myopathy (HIBM)**, distal myopathy with rimmed vacuoles (DMRV) and Nonaka distal myopathy, is caused by a mutation in the GNE gene, which prevents the synthesis of sialic acid. Ultragenyx's drug is designed to replace some of the missing sialic acid and potentially improve muscle strength and function over time, as muscles pick up increased amounts of sialic acid from the serum and incorporate it into proteins and fats.

Compared to trial participants who received a placebo, patients who received treatment with 6 grams per day of Ace-ER retained muscle strength in their upper extremities after 24 weeks. The

effect was maintained over an additional 24 weeks for participants who were treated with the 6 grams per day dose compared with those who received 3 grams per day. In the lower extremities, a similar dose-dependent trend was observed but did not reach statistical significance.

It is hoped these results will be confirmed in an international phase 3 trial, which is currently recruiting. The trial expects to enroll approximately 80 participants. Please visit **ClinicalTrials.gov** and enter NCT02377921 into the search box for more information.

 **To learn more about these trial results, read Aceneuramic Acid Extended Release Administration Maintains Upper Limb Muscle Strength in a 48-week Study of Subjects with GNE Myopathy: Results from a Phase 2, Randomized, Controlled Study, recently published in the Journal of Neuromuscular Diseases, at [content.iospress.com](http://content.iospress.com).**

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## Spinal muscular atrophy (SMA)

# CK-2127107 in Testing

### Phase 2 trial seeks participants with SMA

Investigators are seeking participants for a new phase 2 clinical trial, sponsored by Cytokinetics, to test the investigational drug CK-2127107 in people with types 2, 3 or 4 **spinal muscular atrophy (SMA)**.

CK-2127107 is a fast skeletal muscle troponin activator, designed to increase the ability of muscle to contract by sensitizing it to calcium. In five completed phase 1 trials conducted in healthy volunteers, CK-2127107 proved safe and



able to increase muscle force. Although the approach does not fix the underlying molecular problem in SMA, drugs that enhance muscle function could likely be used in combination

with other therapies that act on the genetic cause of the disease.

Investigators plan to enroll 72 patients in the trial; each will randomly be assigned treatment over a period of eight weeks with CK-2127107 or a placebo. Multiple assessments of skeletal muscle function and fatigability will be performed including respiratory assessments, upper limb strength and functionality for non-ambulatory patients, as well as six-minute walk and

timed-up-and-go for ambulatory patients.

The new study aims to evaluate safety and tolerability and determine whether CK-2127107 has beneficial effects in SMA.

 **Study participants must be 12 years or older and meet other eligibility criteria. To learn more about this trial, including inclusion and exclusion criteria and trial site locations, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT02644668 into the search box.**

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## Spinal muscular atrophy (SMA)

# NeuroNEXT Study

**Baseline results enhance understanding of SMA natural history**

Baseline results from the NeuroNEXT SMA study designed to follow and capture how motor function declines in infants with SMA during the first two years of life, showed that at their initial doctor visit, infants with SMA already had reduced motor function compared to healthy infants.

In addition to motor function measurements, the study evaluated various electrophysiological and molecular biomarkers with the goal to establish the relationship between these biomarkers and motor function in SMA infants.

Infants who participated in the trial will continue to be followed over time to capture additional measurements and information.

There are currently a number of exciting therapies in development for SMA. However, one hurdle has been the establishment of reliable biomarkers that can be followed over time and used as outcome measures in clinical trials. This study begins to shed light



*Research suggests that treatment for SMA may be more successful if it is delivered as early as possible.*

on the natural progression of the disease and how this correlates with measurable biomarkers – information that will be essential to the rapid development of effective therapies for SMA.



To learn more about current SMA research efforts, visit [mda.org/disease/spinal-muscular-atrophy/research](http://mda.org/disease/spinal-muscular-atrophy/research).



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# Strike a Pose

*Adaptive yoga provides benefits for everyone, regardless of physical limitations*

BY BARBARA & JIM TWARDOWSKI, RN



*Editor's Note: Always consult with your MDA physician and MDA Care Center team before beginning any kind of exercise program.*

**T**wenty-eight-year-old Chelsea Singer

is not the typical yoga instructor. Her feet are deformed as a result of contractures and stiffened joints. Her hands shake, her knees frequently dislocate, and she lacks sensation in her extremities. Diagnosed with **Charcot-Marie-Tooth disease (CMT)** at age 11, Singer grew up believing she was frail and found physical activity, such as hiking, painful.

Extremely self-conscious

and concerned about her physical abilities, Singer never went barefoot or even considered joining a gym. Reluctantly, she attended a yoga class with a friend and her life was changed. She took classes several times a week and found her sleep was no longer compromised by physical pain. Astounded with how much better she felt, Singer decided to start a new career and teach yoga professionally.

For nearly three years, Singer has taught adaptive yoga classes in conjunction with the Muscular Dystrophy Association and its Charcot-Marie-Tooth support group of Tampa/St. Petersburg,

Fla. One of her greatest joys is working with kids at MDA Summer Camp.

## YOGA FOR YOU

How does someone who uses a wheelchair or other mobility aids such as crutches and walkers, get on a mat and twist their body into a yoga pose?

"It's not just about the physical movement, although that can really benefit people," Singer says. "Yoga is more about the inner experience than the outer experience. For people who have disabilities, I focus on deep breathing and meditation. Even when a pose requires the use of muscles impacted by neuromuscular disease, I tell my students

they can close their eyes and visualize the body part that won't move the way they think it should."

Adaptive yoga classes tailor the instruction to the needs and abilities of the individual. If someone has balance issues, they might lean on a chair for support. People who use wheelchairs can do many poses in a seated position. Even those who cannot move their own limbs might have a volunteer or friend who holds their arm and moves it for them.

### MIND AND BODY BENEFITS

Practicing yoga has the potential to provide physical, psychological and social benefits for people with neuromuscular disorders, says Scott Holsten, a physical therapist at the MDA Care Center at Carolinas Medical Center and registered Yoga Alliance teacher. Neuromuscular disorders cause weaknesses resulting in muscle and structural imbalances in the body. These imbalances can limit optimal functional movement and even produce pain. The alignment-centered poses

of yoga, which stretch and strengthen specific body parts, can be physically beneficial.

"This can include stretching tight calves to improve ankle flexibility for better standing, stretching hip flexors that become tight from sitting more frequently to manage back pain and possibly strengthening trunk muscles weakened from lack of use due to sitting in a wheelchair," says Holsten.

Breath control is also a crucial component to practicing yoga. The various types of breathing techniques act as a form of exercise or respiratory training that might improve function.

Yoga's psychological benefits come from increased mindfulness and meditation. "The mindfulness practiced in yoga class can be transitioned to the world off the yoga mat," says Holsten, who finds that people who practice yoga learn to be less reactive to the world.

### EXERCISING AND SOCIALIZING

Taking a yoga class allows one to get away from daily

## CONNECTING WITH A CLASS

Finding a yoga class where you feel comfortable takes a bit of searching. The class descriptors may include the word "adaptive" or "accessible," or they may not. Chair yoga is designed for seated participants and might be a good fit for individuals who use wheelchairs for mobility. Many yoga classes marketed to "seniors" are less intense. Some yoga classes are created for those who have a specific medical condition such as arthritis or multiple sclerosis, and may work equally well for someone with a neuromuscular condition. Here are some tips for finding a yoga class that works for you:

- Do a Google search using the terms yoga and the name of your town.
- Ask your health care team and local MDA office if they know of any classes offered in your area.
- Contact health clubs, city recreation departments, community centers and colleges with leisure learning programs to see if they offer classes.
- Use the Yoga Alliance ([yogaalliance.org](http://yogaalliance.org)) website to search for teachers and schools.



responsibilities and make the time to engage with others, which also may lead to new friendships.

During the yoga class, watch what the other students are doing. If everyone is moving their right arm, you don't want to be moving your left. However, don't get caught up in comparing what you can do with what others are doing. Comparison can lead to going too deep into the pose to look like someone else and result in injury.

"Often going too deep into a pose causes correct alignment to be lost and the benefit of the pose is reduced. For example, rounding the back to get deeper into a hamstring stretch causes the stretch to shift from the hamstrings to the back. Listen to your body. You are there

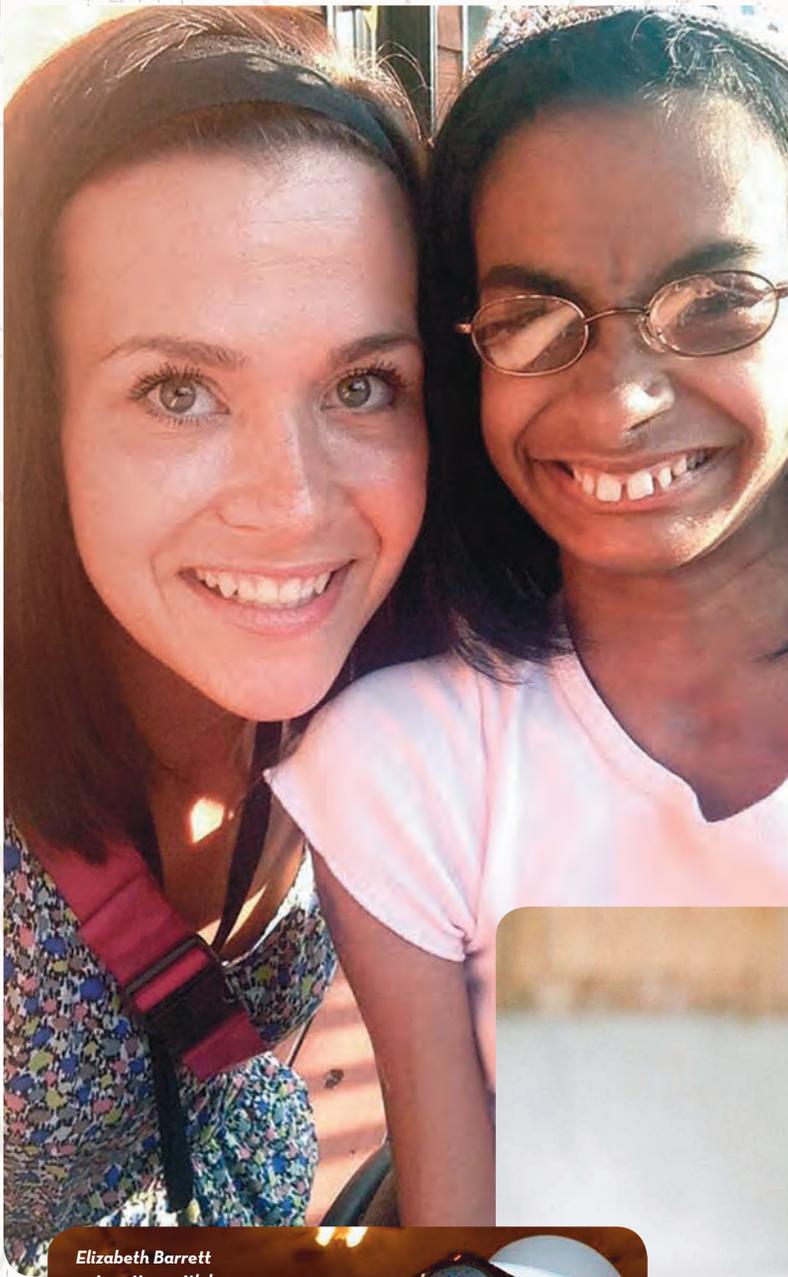
for your benefit not someone else's," says Holsten.

Before joining a yoga studio, talk to the staff about the type of classes available. Speak to the instructor and discuss your limitations. "I always arrive early to class and remind the instructor that my knees easily dislocate," says Singer. Teachers speak to a lot of people. A few minutes discussing your capabilities helps ensure the activity is safe and the instructor can make the necessary modifications to guide you through the class. **Q**

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

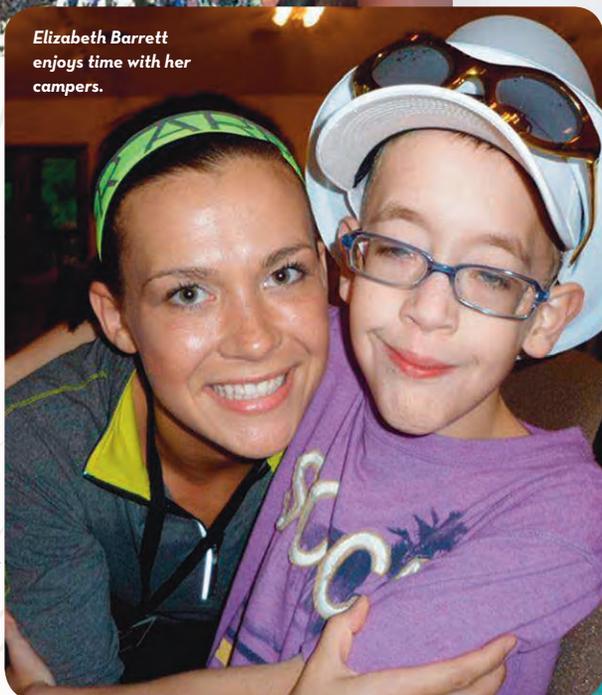


Chelsea Singer has taught adaptive yoga classes through the Muscular Dystrophy Association and its Charcot-Marie-Tooth support group of Tampa/St. Petersburg, Fla.

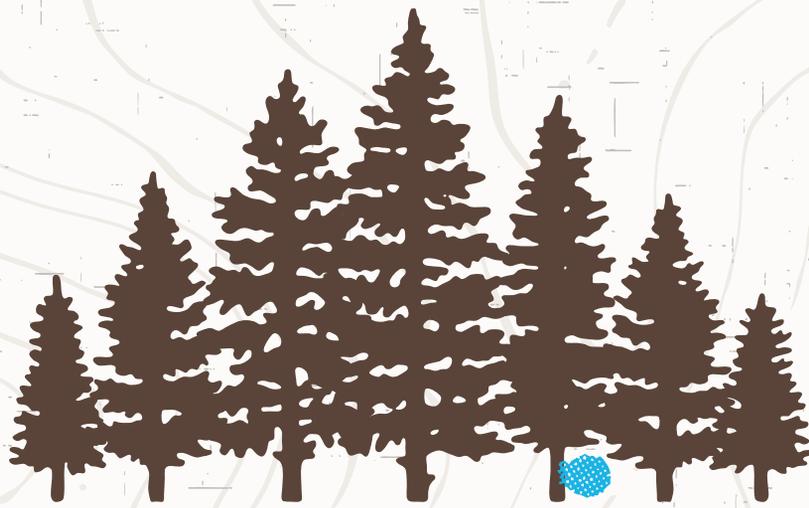


*Elizabeth Barrett  
gets as much as  
she gives as an  
MDA Summer  
Camp volunteer*

BY DONNA SHRYER



*Elizabeth Barrett  
enjoys time with her  
campers.*



# CAMPING

## UNLIMITED

There are so many noble reasons to be an MDA Summer Camp volunteer, and Elizabeth Barrett can easily chat up every last one. As a six-year veteran volunteer at the St. Louis-area MDA Summer Camp, now held annually at Pinecrest Camp in rural Madison County, she immediately launches into how MDA's life-changing summer camps give kids with muscular dystrophy and related

life-threatening diseases that limit muscle strength and mobility one blissful week when they can play and live unlimited in an environment without barriers. And without a moment's hesitation, Barrett extols the virtues of MDA itself, an organization that she says "works so hard to help find a cure for muscular dystrophy, ALS and other muscle-debilitating diseases."

Talk with her long enough, though, and Barrett begins to sound more like one of her happy campers. That's when she gleefully turns to camp activities, from painting stage sets for a camp-wide talent show to painting her fingernails as part of a

before-bed, girls-only gabfest. She'll rave about the camp food, saying, "it's incredible — plus there's ice cream at every meal." And with a giggle in her voice, she'll dive into stories about swim time, her favorite camp activity. "Just look around the pool! All you

see are smiling faces. How can anyone not love that? Nothing makes me happier than being a kid with the kids. It's the best week of the year. I know everyone says that. I think you have to experience it to understand it."

## A HEALING EXPERIENCE

Barrett's journey to MDA Summer Camp was bitter-sweet, as it began with her father's ALS diagnosis in 2010. In 2011, at age 20, she put college on hold and returned home to share caregiving responsibilities with her mom. "We got a newsletter from our local St. Louis MDA talking about camp," Barrett recalls. "Next thing I knew, I was interviewing to be a counselor and then brought on for the next summer session."

More than a few naysayers challenged Barrett's decision to volunteer. "A lot of people said that it would be detrimental to my sanity — since I was already caring for my dad. At the time, though, I saw a week at MDA Summer Camp as a break and maybe a chance to learn more about my dad's disease. So I went. After only a day or two, I realized how therapeutic the whole experience was for me. The kids taught me to forgive the disease, and that helped me find so much more happiness during the time I had left with my father."

After Barrett's father lost his brave battle with ALS in 2013, her dedication to MDA Summer Camp only increased, recalls Therese Michalski Gabriel, co-director of the St. Louis-area MDA Summer

## CARE PACKAGE



As co-director of the St. Louis-area MDA Summer Camp and family support & clinical care coordinator at the St. Louis MDA office, a vital part of Therese Michalski Gabriel's job is to oversee camp volunteers. The group largely consists of millennials — that much-studied demographic that's often dubbed the "Me Me Me Generation" due to its supposed self-focus. Just don't mention this label to Gabriel.

"When people call these wonderful young adults the 'Me Me Me Generation,' I challenge them to come visit our camp," she says. "Every day, I watch these millennials gently and carefully lift campers who can't walk or selflessly skip their own lunch to feed campers who can't lift a fork. Our volunteers are there for our kids 100 percent."

It's the same story across MDA's nearly 75 weeklong summer camps — offered at no charge to families thanks to loyal, generous supporters who fund the camps as well as caring, empathetic volunteers who assume all camper care, including physical and emotional support. These camps give kids with limited muscle strength and mobility a life-changing experience in an environment without barriers and where anything is possible.

If seeing is believing, MDA Summer Camp gives volunteers just as many benefits, from a week filled with amazing adventures to lifelong friendships and a greater sense of independence. "We have a solid core of counselors who volunteer year after year," Gabriel says. "They get together during the year, too. Camp gives our volunteers a chance to forge great relationships."

To learn more about being an MDA Summer Camp volunteer as well as volunteering at MDA events in your area, go to [mda.org](http://mda.org) and enter your ZIP code in the "Find MDA in Your Community" box. And, if you'd like to sign up to volunteer, go to [mda.org/get-involved/become-a-volunteer](http://mda.org/get-involved/become-a-volunteer) and submit the electronic form.



Camp and family support & clinical care coordinator at MDA's St. Louis office. "I think volunteering was an important part of Liz's healing process. Nothing can diminish the loss of her father, but I do think Liz sees camp as something positive that came from tragedy. It was her dad's disease that brought her here — someplace where she's surrounded by people who understand these diseases and how they affect family, too."

### THE GREATEST WEEK ... PLUS A LOT OF EXTRAS

In 2015, Barrett's passionate attachment to MDA Summer Camp landed her a promotion to unit leader, which involves overseeing a group of counselors. "She really stepped up, sharing her own experiences with the newer volunteers," says Gabriel. "Liz brings so much energy to the table."

Gabriel quickly adds that Elizabeth also brings "fun extras" to the table — literally. "She set up a GoFundMe page last year to raise money for camp goodies, like whip cream for our sundaes and extra craft supplies. Liz raised \$1,000 in a very short amount of time and funded a lot of extra fun last summer."

Another "extra" that Barrett never expected was a new view on what's really important to her. "Camp has given me a whole new outlook on life. I appreciate the small things so much more now," she says.

### RELAX AND ENJOY LIFE

Barrett credits MDA camper Bela, who has a form of



Elizabeth with Bela

neuromuscular disease coupled with learning challenges, as one of her most influential "teachers" when it comes to learning to value what can be done rather than fretting over what cannot be done. Bela, now 17, will attend MDA Summer Camp for her fifth and final time this summer, since 17 is the camper cutoff age.

"Bela was the first camper I was paired with, and I wasn't prepared for how slowly she walks — because of her neuromuscular disease. That first summer with Bela, I kept wanting to put her in a wheelchair so we could go faster," Barrett recalls. "Bela didn't want that. She was perfectly capable of going where she wanted to go. I just had to be patient. Once I realized that, I slowed down — so Bela and I could enjoy life together instead of racing to the next activity. That was an awesome discovery for me; it changed my life."

With this revelation came the freedom to kick back,

*"My daughter is a very outgoing person — she enjoys anything that gives her independence, and Liz encourages Bela to be as independent as she wants to be."*



— Vanessa Cook, mom of MDA camper Bela

enjoy camp and relax into the role of Bela's "big sister," which is how Bela's mom, Vanessa Cook, refers to Barrett. "Elizabeth plays such an important role in Bela's life," Cook says. "My daughter is a very outgoing person — she enjoys anything that gives her independence, and Liz encourages Bela to be as independent as she wants to be."

For instance, Barrett applauds Bela's self-proclaimed love for dancing, singing and swimming, with the latter a particular accomplishment. "I can float on my back with no one holding me!" Bela gleefully shares. Cook agrees that these are all activities where her daughter shines, although Bela's mom adds that it's not only the activities Bela loves — it's the whole package, including the people Bela dances, sings and swims with. "Bela and her camp girlfriends share a tight bond — like family. She feels the same about her counselors. We're in touch with a lot of camp friends all year long. We just saw Elizabeth for Bela's birthday party. They're like sisters and that relationship means so much to my daughter."

#### THE RIPPLE EFFECT

During the six years since Barrett first signed on as an MDA Summer Camp counselor, she's taken the reins and built a great life. Part of her happiness stems from a new career as a certified personal trainer, specializing in weight loss, total body conditioning and rehabilitative training. A large part of her professional success, Barrett says, springs from her experiences at MDA Summer Camp. "The campers helped me understand that every body is different, and certain bodies have certain limits. That made me a better personal trainer. I'm much more patient with my clients — more patient than I ever thought I could be."

As Barrett talks about everything MDA Summer Camp gives her, she sometimes slips into a whispered apology. "It sounds so selfish when I talk about everything MDA camp gives me." Bela would likely disagree that her friend has even one selfish bone in her body, since the mere mention of seeing Barrett again prompts a resounding "Yea!" Gabriel gets a little teary-eyed as she describes how "beautiful it is to watch Elizabeth tenderly care for the campers." Perhaps the only answer is to call the MDA Summer Camp experience a draw, or better yet, a win for everyone involved. **Q**

*Donna Shryer is a freelance writer in Chicago.*

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

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# investing IN THE FUTURE

Dr. Chris Rosa, vice chairman of MDA's Board of Directors and the University Assistant Dean for Student Affairs at the City University of New York, was among the speakers on hand to help launch MDA's new brand in January. In his remarks that day, Dr. Rosa shared the story of his own journey with muscular dystrophy, starting with his diagnosis at age 9.

"My prognosis wasn't good, and my physicians seemed to be hyper-concerned about something called my 'CK levels' — creatine kinase, which is an enzyme that's released when muscle is destroyed," Rosa recalled. "But thanks to the care that I got through MDA's remarkable care centers and through path-breaking research, I've lived way beyond my life expectancy, and I've lived to achieve really remarkable things on my own terms, things that we all cherish — to live and love with my family, to build a career, to fall in love, and to have my heart broken and to live to love again. All the things that we cherish about rich and meaningful lives.

"So these days when people talk about 'what I'm concerned about,' I'm far more concerned about the level of my 401(k) than the level of my CK."

When it comes to thriving despite a muscle-debilitating disease — and the financial concerns that come with that — Rosa is not alone. Improvements in the medical management

of neuromuscular diseases mean that individuals are living longer lives, pursuing their dreams and achieving personal goals.

"We've watched science change before our eyes," says Kate Sohl, whose 8-year-old son, Macarthur, has **type 2 spinal muscular atrophy (SMA)**. "Who knows what advances will be made by the time our son is 15? We hope for the best and plan for the worst."

Buoyed by an optimism in today's advances that is tempered by tomorrow's unknowns, the Sohl family, just like Dr. Rosa, sees long-term financial stability as an increasingly important goal. Knowing how best to approach financial planning while managing a neuromuscular disease is rarely easy or straightforward. Thankfully, there are some practical ways to get started.

## FEELING OVERWHELMED?

Michele Boardman couldn't wait to go back to work. A nasty cold had forced the 30-year-old to stay home on the couch for two days.

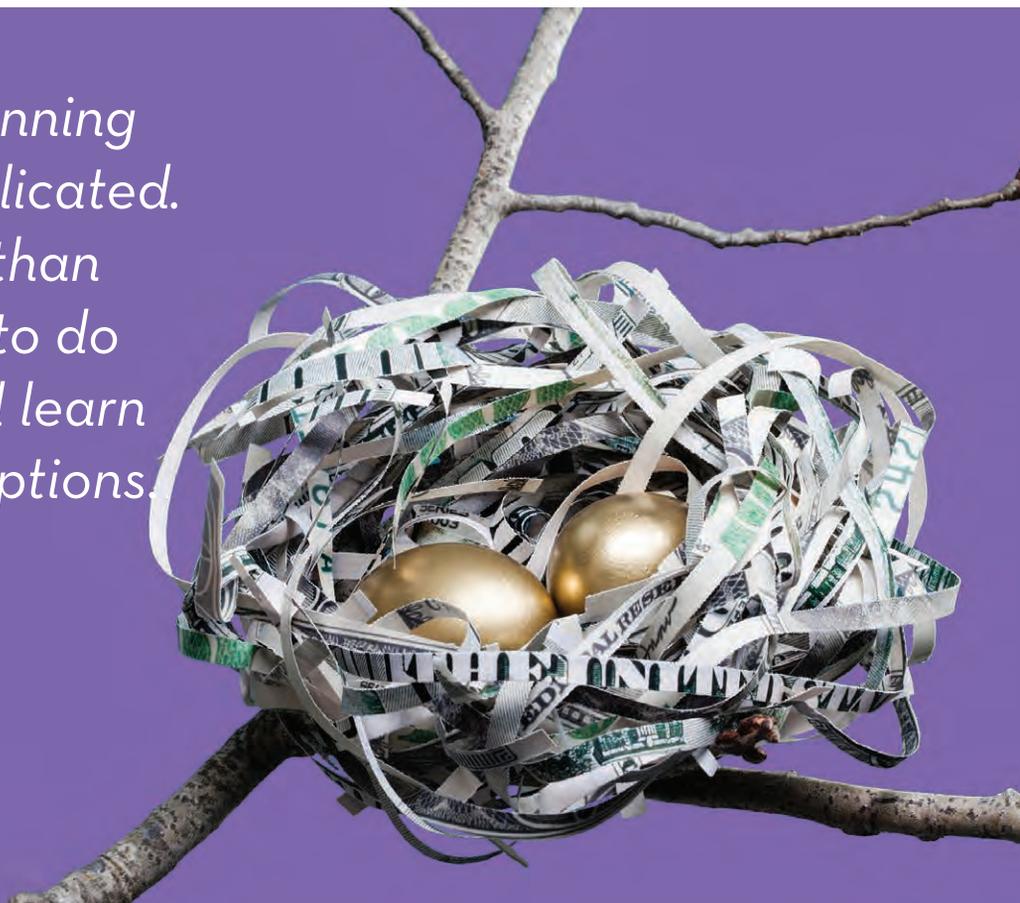
"I couldn't look at Facebook for one more minute," says Boardman, a full-time community work incentives coordinator at AHEDD, a nonprofit organization that serves people with disabilities. "Working is so therapeutic for me in so many ways."

Boardman can personally attest to the importance of the services she promotes, as well as those MDA offers: She had her first muscle biopsy at age 8 and by 16 she had been diagnosed with **type 2I limb-girdle muscular dystrophy (LGMD)**.

"I love helping my clients find sustainable careers, navigate the complicated benefits available from the government

*Financial planning can be complicated. Today more than ever, it pays to do research and learn about your options.*

BY MOLLY BLAKE



and work out their finances,” says Boardman, who is also working toward her license as a professional counselor. But when it comes to planning for a stable financial future to complement a longer life expectancy, things can get tricky.

Boardman is all too familiar with her clients’ challenges.

Work means a paycheck, which is normally a boon, but Boardman recalls meeting a benefits counselor before accepting her current position out of school and

learning that earning income when you have a disability and related needs can also work against you because of Medicare/Medicaid income thresholds.

“I was in a panic,” Boardman says. “I had been hearing for so long that ‘you can’t work [because of your disability]’, and I really wanted to be independent.” The two talked about everything from cash payments to income and dependent care services, like Boardman’s personal care assistant who comes every morning and evening.

“It was so overwhelming,” recalls Boardman.

Since then, she has made incredible strides. After graduating in 2011, Boardman, who also plays on the Philadelphia Power Play wheelchair floor hockey team, lived with her father briefly before moving into her own apartment. She budgets carefully for rent, food and gas bills, and to avoid frivolous spending, she rarely carries cash. But the question of saving for the future is still fraught with complications.

## ORGANIZATION IS KEY

Keep all your important records together in one binder or cabinet so everything is easy to locate. Organize it logically and include the following:

- Relevant medical information: diagnosis, medications, limitations/abilities
- Doctors’ phone numbers/addresses
- Letter of intent (see main story for details)
- Health and hygiene information
- Insurance documents
- Trusted family members and their contact information
- Wills and other estate planning documents
- Accountant and financial planner contact information (if applicable)
- Budget information and/or spending diary
- Important receipts
- Tax documentation
- Benefits summary analysis (if applicable)

## FINDING A SOLUTION

Complex situations like Boardman’s demand attention — sooner rather than later, stresses Annette Hines, a special needs and estate planning attorney in Massachusetts. “Waiting to map out a financial strategy can lead to big problems,” she says.

“There is a lot that goes into having a child with a disability, and you have to navigate your way through the system,” says Sohl, who worked with Hines to set up a special needs trust for Macarthur that won’t threaten any of his public benefits, like the 20 hours of personal care assistance he receives each week.

## THE ABLE ACT UPDATE

The Achieving a Better Life Experience (ABLE) Act of 2013 was signed into federal law in December 2014. The bill provides the authority under the tax code for people with disabilities to have “ABLE accounts” – tax-advantaged savings accounts for qualified expenses in areas like education, housing and transportation. Money in an ABLE account also does not affect qualification for federal benefits for Medicare.

For people to take advantage of these accounts, states must pass their own ABLE Act laws allowing financial institutions to create and market the accounts. ABLE Act laws have now been enacted in 35 states, and most states are planning programs that are open to residents of any state. By the end of 2016, at least five states are expected to have active programs.

Families interested in establishing an ABLE account should research states’ requirements, eligibility, funding processes, expectations and account limitations.

“ABLE accounts are not a one-size-fits-all solution,” says Kristin Stephenson, MDA vice president of policy and advocacy. “The name of the game for people who require those supports has been not to save money because that would make them ineligible for services. So [implementation of ABLE] would be a significant paradigm shift in mindset.”

To learn more about the ABLE Act, visit the ABLE National Resource Center at [ablenrc.org](http://ablenrc.org).

One good way for parents to begin planning is by writing what Cynthia Haddad, a certified financial planner with Shepherd Financial Planners, calls a letter of intent. In this document, parents should write about the kind of life they want for their child. Include information about the child’s disability, abilities, medical history, close and trusted family members, and even the values and beliefs that are important to the family. (See sidebar on page 27).

This is not an easy thing to write, she adds. “Often this is the first time parents air their concerns, and they realize that they need to plan for two generations: theirs and their child’s.”

Some families choose to meet with a financial professional. This expert can help families map out a smart financial plan, including how to identify and maximize government benefits, choose

life insurance and allow grandparents to gift money without jeopardizing government benefits.

“You can and should save for your child’s future,” says Haddad. She and Hines advise using a ROTH IRA or a special needs trust to protect savings for a child.

When it comes to saving for more immediate needs, such as purchasing a new home or car, consider using an Individual Development Account (IDA), a special savings account for people with low incomes. Savings in an IDA are matched. This means that for every dollar that you save, you receive a dollar or more, depending on the providing organization. IDA account holders may also be able to take financial education classes and receive one-on-one counseling. Learn more about IDAs at [cfed.org/programs/idas](http://cfed.org/programs/idas).

Just as you rely on a team to address health care needs, so too should you consult a team when it comes to finances, Haddad stresses. Your team may include a financial adviser, as well as an accountant who can steer families through tax deductions and other state and federal tax laws.

But many people may not be able to afford to include these financial professionals on their team. To ensure that their government benefits are coordinated and are promoting

self-sufficiency, families can work with a community work incentives coordinator, like Boardman. They help people with disabilities make informed choices about work and successfully transition to self-sufficiency. You can learn more at [ssa.gov/work/WIPA.html](http://ssa.gov/work/WIPA.html).

### ASK FOR HELP

Emily Filmore had big plans for her future. She graduated from St. Louis University School of Law and practiced estate planning despite suffering from frequent migraines, pain, and sensitivity to light and temperature changes. In 2003, Filmore developed a severe rash and was diagnosed with **dermatomyositis**, an inflammatory disease that causes muscle weakness and a distinguishing skin rash. In 2005, she stopped working in order to focus on her health.

“I say I’m retired,” says Filmore, 39.

But since her husband had a solid, relatively well-paying job as an attorney, she never considered applying for disability or financial assistance. Even when her daughter was born, Filmore notes, “My husband and I felt like we shouldn’t take money from the government.”

“That was a mistake,” says Filmore, who has since found some personal benefits by consulting a holistic nutritional expert and an acupuncturist. In order to function without pain and get a restful sleep, she depends on herbal supplements, essential oils and an organic diet, all of which are costly out-of-pocket expenses. Not to mention a

### Finance Q&A

Learn about taxes, asset development, types of federal benefits and more in a Q&A with Michael Morris, a recognized field leader on finance capacity for people with disabilities and director of the National Disability Institute, at [mda.org/quest](http://mda.org/quest).

high health insurance deductible that Filmore paid in order to receive intravenous immunoglobulin (IVIG), a common treatment for dermatomyositis. To make matters worse, when the economy soured a few years ago, her husband took a pay cut and the couple “lost money on the sale of a house because we had to move closer to my husband’s work so he could help me.”

“Every year we made many financial accommodations for my illness,” says Filmore. She recently began applying for disability, but the process is lengthy, and there are no guarantees.

“Don’t be deterred by the process,” advises Kristin Stephenson, MDA vice president of policy and advocacy. “But

doing the research on what services are available and determining a person’s eligibility is key.”

Research is also critical for people, like Filmore, who can choose their health plan through an employer or via the health insurance exchange. Keeping in mind that the ultimate goal is to find a plan that maximizes the care from providers and minimizes financial risk and out-of-pocket expenses, Stephenson encourages families not to immediately dismiss any plan. Even a high-deductible plan might work if expenses are predictable and the deductible will be met every year.

“Ask questions about everything including whether the specialists are out of network or in network,” Stephenson adds.

Another resource to consult — as well as a source of comfort — Sohl suggests, are other members of the MDA community. For instance, a few times a month, she organizes an SMA mom’s night out with mothers she’s met through MDA events and support groups. The moms get together to talk, laugh, cry and most importantly, share resources.

“I really treasure the evenings,” says Sohl. And while she admits she’s probably not doing everything right, she’s definitely got the right attitude. “We will keep learning, asking questions, researching and hoping for the best.” [Q](#)

*Molly Blake is a freelance writer in the Bay Area.*

“You can and should save for your child’s future.”

– Cynthia Haddad

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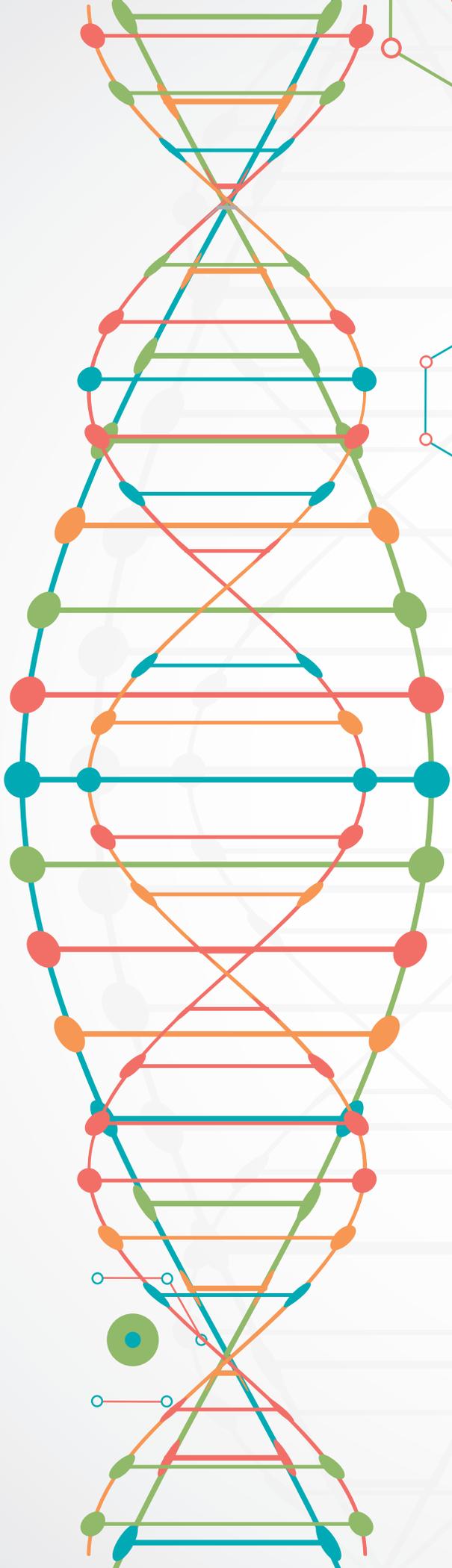


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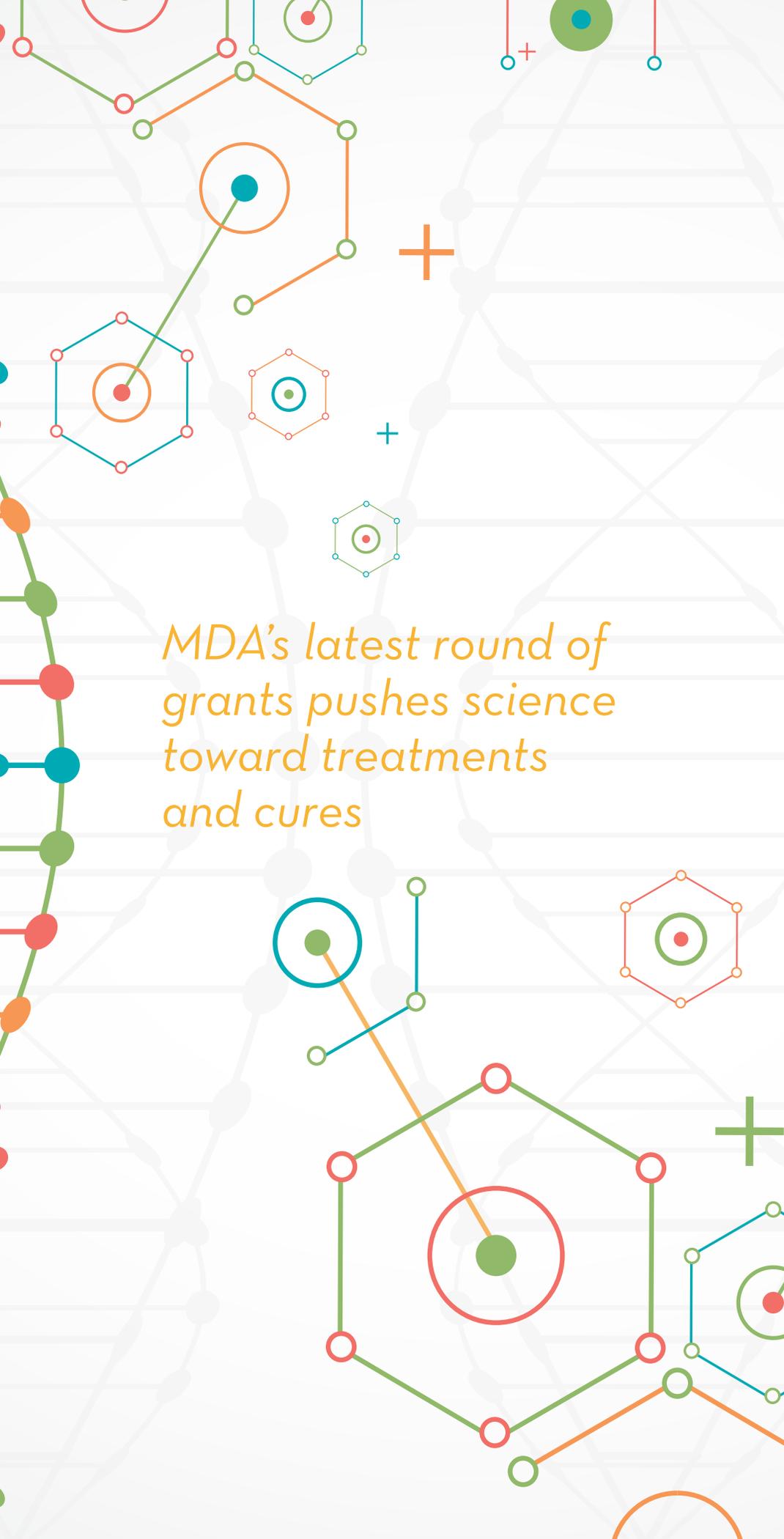
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*MDA's latest round of grants pushes science toward treatments and cures*



# searching

BY AMY MADSEN

## FOR ANSWERS ACROSS DISEASES

MDA's research progress during the first quarter of 2016 includes a solid step aimed at accelerating treatments and cures for muscular dystrophy, ALS and related muscle-debilitating diseases in its program. In February, MDA announced the award of 41 new research, development and research infrastructure grants totaling nearly \$11 million. The grants cover projects that take aim at more than a dozen specific diseases under MDA's umbrella and are expected to spur advances across the broad range of diseases MDA covers.

The \$11 million commitment, MDA's largest such award in three years, underlines MDA's dedication to investing in research across diseases with the expectation that increased understanding in one disease will inform progress and lead to research and medical breakthroughs in other disease spaces.

### COVERING ALL THE BASES

Importantly, a number of different angles come into play in the search for treatments and cures.

MDA's 41 new grant awards strengthen its research commitment in several areas, all critical to composing a comprehensive framework within which drug development can succeed. The key focus areas addressed by the new research projects include:

- elucidation of disease mechanisms
- determination of disease causes



*MDA's 41 new grant awards strengthen its research commitment in several areas, all critical to composing a comprehensive framework within which drug development can succeed.*

- generation of new research models and tools
- identification and validation of new drug targets
- development of biomarkers
- support of young investigators
- collaboration with biotech and industry

#### TAKING A CLOSER LOOK



**KIM STAATS**, a postdoctoral researcher at the University of Southern

California in Los Angeles, was awarded an MDA development grant to study potential causes for sporadic **amyotrophic lateral sclerosis (ALS)**. Using a cutting-edge approach to identify genetic contributors in sporadic ALS, Staats has found a new gene called SEC14L5 with ties to C9ORF72, the most common cause of familial ALS. Now she will elucidate the role of an identified SEC14L5 mutation in motor neuron death in ALS, increasing understanding of ALS disease mechanisms and facilitating the development of therapeutic strategies.



**SUSAN HAMILTON, L.F.** McCollum Chair in Molecular Physiology at

Baylor College of Medicine in Houston, was awarded an MDA research grant to further define the molecular mechanisms underlying **central core disease (CCD)** and other ryanodine receptor (RyR1)-related myopathies, and develop new treatments. Hamilton will test three

different types of therapeutic interventions in two mouse models of CCD. Since the two mouse models demonstrate different ways that mutations in RyR1 can lead to dysfunction, and the three drugs are already approved for use or currently in clinical trials for other diseases, these studies could lay the groundwork for rapid development of therapies in CCD and other RyR1-related myopathies.



**GABSANG LEE**, assistant professor in the department of neurology at

Johns Hopkins University in Baltimore, was awarded an MDA research grant to create a human cellular model of **Duchenne muscular dystrophy (DMD)**. Using stem cells derived from blood, he will generate patient-specific muscle cells, confirm that the cells accurately reflect the disease, and validate the utility of the cells using drug screening and gene knockout. Such a “DMD-in-a-dish” model could facilitate fast and accurate testing of investigational therapies.



**PETER JONES**, associate professor in cell and developmental biology and

neurology at University of Massachusetts Medical School in Worcester, was awarded a research grant to develop a mouse model for **facioscapulohumeral muscular dystrophy (FSHD)**. Jones and colleagues have engineered a mouse model that

contains the human DUX4 gene, based on the widely accepted understanding that FSHD is caused by increased expression of the gene. Now the team will work to develop different versions of the mouse to represent various levels of human FSHD disease severity. Successful completion of the project will provide the FSHD field with valuable tools for understanding what happens in the disease and for preclinical screening of different classes of potential therapies aimed at DUX4 and downstream targets.



**PRADEEP REDDY DUBBAKA VENU**, a research associate at the

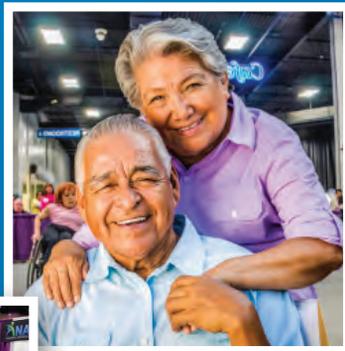
Salk Institute for Biological Studies, La Jolla, Calif., was awarded an MDA development grant to test a potential method for preventing transmission of **mitochondrial myopathies** by selective elimination of defective mitochondrial DNA in oocytes (immature egg cells). The approach involves using enzymes called nucleases to act as “molecular scissors” that are able to precisely enter mitochondria and specifically identify and eliminate mutated mitochondrial DNA. The feasibility of the approach has been demonstrated in mouse embryos, where it successfully prevented the transmission of targeted mitochondrial DNA to the next generation. Dubbaka Venu and colleagues will test the safety and efficacy of the approach in human oocytes from mitochondrial disease patients.

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Los Angeles Convention Center

If successful, this work could lead to the first-in-history correction of genetic diseases at the oocyte level and the potential eradication of mitochondrial diseases.



**TAMAR SZTAL,** a postdoctoral fellow at the School of Biological

Sciences, Monash University, in Melbourne, Australia, was awarded an MDA development grant to screen more than 1,200 FDA-approved drugs in a zebrafish model of **nemaline myopathy** to determine which are most effective at increasing muscle function and reducing disease severity.



**STEVE CANNON,** professor of physiology at the David Geffen

School of Medicine at the University of California, Los Angeles, was awarded an MDA research grant to investigate the underlying mechanisms in **periodic paralysis**. Preliminary studies in research models have revealed a profound loss of muscle force within minutes of recovery from exposure to high carbon dioxide levels, which Cannon and colleagues suggest may be a surrogate for the exercise-induced attacks of weakness that occur in patients. Cannon and his team will strategically select drugs that may block this process, and will

test the potential of these drugs to foreshorten or prevent attacks of periodic paralysis.



**CHRIS LORSON,** professor of veterinary pathology, and molecular biology

and immunology at the University of Missouri in Columbia, was awarded an MDA research grant to optimize an approach currently in clinical trials for **spinal muscular atrophy (SMA)** by delivering an enhanced form of drug combined with other potential synergistic therapies. This work could provide evidence for an innovative combinatorial approach to SMA that would address a broad range of patient needs.

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## MAKING EVERY GRANT COUNT

MDA funds the best science in pursuit of urgently needed answers for kids and adults with neuromuscular diseases that take away strength, independence and life.

Each and every grant award funds a project that could hold the key to better outcomes, to more options, to improved quality of life — to treatments and cures — for one or more of the many muscle-debilitating diseases in MDA's program.

MDA is now supporting more than 150 research projects in 11 countries around the world. [Q](#)

*Amy Madsen currently serves as marketing communications manager for MDA's research program.*

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# Home Smart Home

*New products aim to make tasks around the home easier for everyone, including those with neuromuscular diseases*

BY SHAILA WUNDERLICH

**A**s the world awaits the full realization of the Internet of Things (IoT) — the computer-based platform on which all home automation will reside — the individual products and innovations that will one day comprise it are slowly trickling out. These smart home technologies will likely end up in all homes in the future; however, the difference in their effect on

a typical homeowner compared to one living with muscular dystrophy, ALS or a related muscle-debilitating disease is the difference between additional convenience and a game-changing transformation.

Here, we take a look at some of the

latest products promising to make life at home easier, more convenient and more independent for individuals with neuromuscular diseases.

## DOORBELL CAMERAS

For the general population, doorbell cameras are usually used for monitoring the doorstep while away from the house. For people with neuromuscular diseases, they are about managing the doorstep from the inside.

Ring's Doorbell Camera (\$199, Lowe's) lets homeowners communicate audio-visually with visitors at their door. Vivint's Doorbell Camera (\$1,500, [vivint.com](http://vivint.com), 844-720-7988) takes it a step further by including a motion detector, door-and-window sensors, auto locks and 24/7 live surveillance.

"Individuals may lack endurance and strength to stand and see who's on the other side of the door and may also lack the grip strength and dexterity to manage locks and door handles," says Jessica Garcia, an occupational therapist at the MDA Care Center at the University of New Mexico Hospital. "Technology like this gives them the ability to accomplish these tasks with minimal effort and range of motion."

## AUTOMATED THERMOSTATS

The inability to monitor and self-regulate room temperature can be a troublesome reality with certain types of neuromuscular diseases. A new series of automated thermostats addresses this issue

Ring's Doorbell Camera



### KNOWLEDGE SOURCE THINKING AHEAD

The only thing more exciting than the influx of smarthome products on the market is the ever-growing number of experts, think tanks and scientists working feverishly to make the ideal of a fully automated home a reality. One project being worked on to achieve this goal is the "Cognitive Computing Research Study," which pairs tech giant IBM with the CTA Foundation (the philanthropic arm of the Consumer Technology Association) to investigate the connection between technology, humans and their environment.

Much of the study's efforts will center on cognitive computing's ability to predict human condition and behavior. "If it can take all the various data points of your daily activity — smart appliances, thermostats, wearable [technology] — and use those points to predict events,

such as when you might be looking to cook dinner or leave the house or do the laundry — imagine the applications," says Stephen Ewell, executive director of the Consumer Technology Association Foundation. "But beyond that, could it also help recognize — possibly through a shift in gait, for instance — when a person is close to taking a fall?"

The study's findings are expected to be published in a report some time within the next year, which means in the years to closely follow, individuals with neuromuscular diseases may expect to see more (and better) home appliances, robotics and wearable devices available that have the power to streamline their daily routines and make it easier to live independently.



The Nest Learning Thermostat

by “learning” and predicting a person’s temperature needs on its own.

The Nest Learning Thermostat (\$249, [store.nest.com](http://store.nest.com)) takes about a week to study your temperature preferences and then it programs itself. And the longer it’s used, the smarter it gets.

“In our care centers we talk a lot about prioritizing our actions throughout the day,” says Caroline Brandel, an occupational therapist at the MDA Care Center at Georgetown MedStar. “You have to think of it as having a tank with a certain amount of energy. What actions are most worth depleting it for? If it comes down to adjusting the temperature versus eating or bathing, the thermostat is probably going to get left behind.”

### SMART SWITCHES & BULBS

A new wave of smart LED bulbs and switches are already making a huge difference in people’s home lives since their arrival on the market two to three years ago. Philips’ Hue lighting system uses LED bulbs which communicate with a free application that serves as the interface through which lighting can be turned on and off, dimmed or put on a timer (\$199.95 for a White and Color Ambiance Starter Kit, [amazon.com](http://amazon.com)).

Sengled’s system expands upon this concept by offering bulbs that are not only control-

lable wirelessly through an app, but also double as wireless home media devices (\$49.99 to \$149.99, [sengled.com](http://sengled.com)). For example, Sengled offers LED bulbs that boost Wi-Fi signals or come equipped with a webcam and speaker. Brandel loves the idea for the lighting capabilities alone. “Simply not having to move through an environment that’s not lit provides safety from so many potential falls,” she says.

The WeMo Insight switch (\$49.99, available from Lowe’s in May 2016) is a Wi-Fi enabled outlet that controls, monitors and manages home appliances, electronics and home systems, such as air conditioning and heat, from any smart device. Simply plug a device into its connected outlet, and that device becomes operable from a laptop or smartphone.

### KITCHEN APPLIANCES

Kitchen electronics companies LG and Innohome have been working on the release of two products holding significant promise toward an individual’s ability to cook

and eat. LG’s Signature Refrigerator features a floor sensor that triggers its doors to open when it detects a foot beneath it. “It’s a great feature for people with armloads of groceries but also for people who might have difficulty with arm control,” says Stephen Ewell, Executive Director of the Consumer Technology Association Foundation. The front door of the refrigerator also lights up when knocked upon, letting the “knocker” peek inside without having to open the door.

In the same way new thermostats learn an individual’s temperature preferences over time and adjust accordingly, Innohome’s Stove Guard learns a person’s cooking habits and monitors for potential safety hazards. The small device, which attaches to any oven appliance, detects dangerously high temperatures and steep temperature rises and, depending on the situation, either shuts off the appliance, adjusts it or alerts the user. “Within the spectrum of MD there are plenty of individuals who can cook for themselves,” Brandel says. “For these folks, these products could be huge.”

Both the Signature Refrigerator ([lg.com](http://lg.com)) and Stove Guard ([Innohome.com](http://Innohome.com)) are expected to come out within the next year. Stove Guard will be priced around \$250; LG will release the price on its Signature Refrigerator near 2017. [Q](#)



Sengled LED bulbs

*Shaila Wunderlich is a freelance writer in the St. Louis area.*



# access MDA

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

## Through the Fire

A fire fighter shares the highs and lows of his experience with neuromuscular disease



Dennis Bracety had been fighting fires in Tucson, Ariz., for almost 10 years when he first started to experience symptoms of what he would eventually find out was **dermatomyositis**, a neuromuscular disease that causes inflammation of the muscles and skin.

“My symptoms started in November 2010 at our annual Turkey Bowl Thanksgiving event,” Bracety says. “I felt sluggish, and I didn’t feel alert.

Something was off, and I knew it. For the next three weeks I ignored the symptoms; I’m a macho guy, I figured I would be fine. It wasn’t until Christmas Eve [that I recognized something was seriously wrong] when I was on duty, and I just couldn’t get out of bed.”

After visiting doctors for weeks, Bracety finally received his diagnosis of dermatomyositis in February 2011.

“I remember being shocked,” he says. “I thought the doctors were lying to me. I was in the best shape of my life. You never think it can happen to you.”

Shortly after the diagnosis, Bracety’s health took a turn for the worse. Within two months, he was in the ICU and had lost all muscle function as well as 90 pounds. At one point, Bracety was unable to walk, talk or even swallow — everyday freedoms many sometimes take for granted. However, by December 2011 he had improved thanks to treatment and physical therapy, and his doctors told him he would likely be able to go back to fire fighting after he recovered. It took another year-and-a-half for that to become a reality.

“For that whole year [of 2012,] I would have good days, but mostly bad days,” he says. “It ended up being two-and-a-half years of touch and go, before I was able to feel somewhat normal again, emotionally and physically.”

One of the good days that Bracety can point to was the 2012 MDA Muscle Walk in Tucson, which was the first Muscle Walk event he had attended. Although he was hesitant to go out and speak because he was dealing with depression due to his condition, a local MDA representative gave him the push he needed to do it.

“It was awesome,” Bracety says. “It felt good to feel alive again. That MDA Muscle Walk was huge for my mental health, just being in a public forum. I was done feeling sorry for myself.”

Now that Bracety is back at work – he returned in June 2013 – he’s also involved with Fill the Boot through his local IAFF Chapter 4944, and in 2015, he served on the Tucson MDA Muscle Walk Committee, helping to organize and raise funds for the walk, as well as having his station bring the fire truck to the event for the kids.

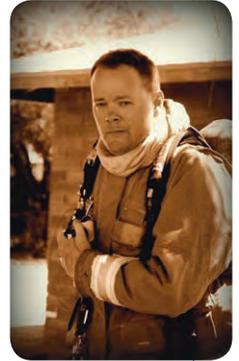
In addition to his support of MDA, Bracety wanted to share his story and experience with others by writing a book. Called *Fire Within*, Bracety’s book covers not only his personal story, but it also provides insight into universal challenges that many people with neuromuscular disease and their friends and families face each day.

“When I was first in the hospital, there were no answers,” Bracety says. “It was a sad,

## FILL THE BOOT AT THE SMITHSONIAN

Visitors to the Smithsonian’s National Museum of American History in Washington, D.C., will be able to examine an important piece of MDA history as part of the museum’s first annual philanthropy symposium, “The Power of Giving: Philanthropy’s Impact on American Life.” Among the historic

artifacts representing charities will be a firefighter’s boot used to collect money for MDA’s Fill the Boot campaign. “The Power of Giving,” which was first unveiled in December 2015 and covers giving in America from the 1870s to the present day, will be open to visitors until November 2016.



depressing scene because we didn’t know. With my book, I wanted to let people know about the importance of being their own best patient advocate and not giving up. A lot of people lose hope or motivation, and I want to bring some of that hope back, if I can.”



**To sign up for the MDA Muscle**

**Walk in your area, go to [mdamusclewalk.org](http://mdamusclewalk.org). For more information about Fill the Boot, visit [mda.org/get-involved/fill-the-boot](http://mda.org/get-involved/fill-the-boot). Find Dennis’s book, *Fire Within*, on [Amazon.com](http://Amazon.com).**



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# Join the Team

## Runners and walkers share their reasons for joining MDA Team Momentum

*"I joined Team Momentum for the Colfax race and ran the half marathon. My daughter Kinley (age 15) has mitochondrial encephalomyopathy and has attended MDA Summer Camp in both Kansas and Colorado. MDA has been a wonderful resource for our family over the years, and I felt this was a great way to raise money for MDA while getting in shape and running another half marathon. The coaching was a huge help and being part of a team made the training and the race even more fun. It was a tremendous experience for me!"*

– John Hower

*"I run for a cure. Plain and simple, neuromuscular disease is horrific. With MDA Team Momentum, every step I take and every dollar I raise brings us one step closer to tangible treatments and cures!"*

– Ryan Woodman, MDA director of business development

*"We started with MDA Team Momentum in 2014 with the Michelob ULTRA Half Marathon in Boston. I was by no means a runner, but seeing the determination on Mason's face when we went out for our daily runs before the big day was something I will never forget, and I carry with me every day. His encouraging words of 'Mommy, you can do it!' or 'It's OK you can walk, I'll walk with you...' still ring in my ears."*

– Alicia Martin, whose son Mason was diagnosed with a rare form of muscular dystrophy in 2012

## TEAM MOMENTUM UPCOMING EVENTS

Get out and walk or run with MDA Team Momentum at any of these upcoming premier races. Some of the races sell out and MDA Team Momentum has limited entries. Register at [mdateam.org](http://mdateam.org).

**Bank of America Chicago Marathon**  
Sunday, October 9, 2016

**Rock N Roll Denver Half-Marathon,**  
Sunday, October 16, 2016

**Marine Corps Marathon & 10K**  
Washington, D.C., Sunday,  
October 30, 2016

**TCS New York City Marathon**  
Sunday, November 6, 2016

**Dallas Marathon & Half-Marathon**  
Sunday, December 11, 2016

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Did You Know that Heart Failure is Very Common and Often Fatal in Patients with Duchenne Muscular Dystrophy?

# HOPE

The Halt cardiOmyopathy ProgrESSION in Duchenne is a clinical trial that will evaluate whether an investigational cardiac cell therapy is safe and potentially effective in minimizing scar and improving heart function in DMD associated heart disease.

To find a site near you, please visit [ClinicalTrials.gov](http://ClinicalTrials.gov) (Study Identifier NCT02485938)



[www.capricor.com](http://www.capricor.com)



# 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](http://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



## Do I need to update my information?

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# Passionate Professional

**A young professional discusses having a disability and thriving in the workplace**

Emmanuel Arzate, a 26-year-old from El Paso, Texas, who was diagnosed with **spinal muscular atrophy** at age 2, has always had a passion for educating and connecting with others. Currently working as a teacher/clerk at his local YWCA, Arzate's previous job as a financial adviser set him on the path toward using this passion to help people make informed decisions about their finances.

"Being a financial adviser, it was like a match made in heaven," Arzate says. "I was educating clients on their finances, but probably what I enjoyed the most was being able to see their progress. I would see people come into my office who weren't savers come back in six months with \$2,000 or \$3,000 in their accounts."

And although an accidental injury he received during a flight in December caused Arzate to leave his job as a financial adviser at Northwestern Mutual due to additional stress, Arzate's

perseverance led him to his new job at the YWCA, as well as to other opportunities, including his interest in being a motivational speaker.

"I'm very ambitious," he says. "I want a lot from life, and I have a very tenacious personality. I want to be a successful entrepreneur, a motivational speaker and politician, as well. Recently, I've been doing motivational speaking, and it's been great. I recently was chosen to be a presenter for a conference at [my alma mater] the University of Texas at El Paso (UTEP)."

Arzate received his undergraduate degree from UTEP and is currently getting his MBA there. In addition to his motivational speaking and entrepreneurship, he plans to collaborate with fellow students on a project that addresses some of the challenges he has faced when talking with potential employers. Fittingly, his plan is to do



*Emmanuel Arzate navigates the professional workplace.*

this through an educational video for employers about employees with disabilities. He was inspired by his positive experience working at Northwestern Mutual, which, in addition to supporting Arzate's workplace needs, also donated \$5,000 to last year's Fill the Boot campaign.

"I'd like the video to help human resources at companies

better understand what it's like to hire a person with a disability," he says. "I want to interview some of my employers and ask them about how the initial process [of hiring me] was and what ran through their minds."

Between the video, his motivational speaking, getting his MBA and his regular job, Arzate is a busy man, but he still gets out and enjoys spending time with his friends, as well as participating in the MDA Muscle Walk each year.

"I love going to festivals and concerts," he says. "Anything that comes to El Paso. I love going to all of these events and just having a good time with my friends."

## PREPARING FOR AN INTERVIEW

Trying to connect with a potential employer during a job interview can be difficult – whether you have a disability or not. Emmanuel Arzate shares some advice that has helped him find success when interviewing for a job.

"I've had interviews where I have the qualifications they're looking for, but there's a disconnect at the time of the interview, [for example,] when they try to shake my hand and I can't shake their hand," he says. "So from the very beginning the interview is awkward."

In order to defuse some of that awkwardness, Arzate suggests being forward with employers about what you can do and what you need in terms of accommodations.

"The thing I discovered is you have to be upfront about things," Arzate says. "You have to be the one that brings it up and discusses the accommodations [you need]. When I was preparing for interviews, I created a portfolio that showed the software I needed, my limitations and even had testimonials from previous employers of their experience working with me."

 **MDA's Transitions Center offers many resources for teens and young adults looking to start (or change) their careers. For more information, visit [transitions.mda.org/careers](https://transitions.mda.org/careers).**



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# An Awesome Cause

**A teenager brings her passion to sports and her support of MDA Muscle Walk**

Dana Parrott, a 13-year-old from Milford, Conn., who was diagnosed with **limb-girdle muscular dystrophy (LGMD)** at age 8, loves being active. She participates in track and field, she swims and recently started playing power soccer. Through her local Hospital for Special Care in New Britain, Conn., she joined teams for all of these sports, and she even found herself at nationals for her track and field team in 2015, breaking records for discus and the club throw.

“When I first got diagnosed, I thought, ‘How am I going to be able to do anything?’” Dana

says. “But I started doing these sports, and I feel like it makes me stronger and I’m getting better and better. When I did so well at nationals, I just thought, ‘This is what I love.’”

Dana brings her passion for being active from sports into her support of MDA. For the past four years, since she received her diagnosis, she has been attending the Greater Hartford & New Haven Area MDA Muscle Walks with her aptly named team, Dana’s Team Awesome.

“With everything she’s going through, she is absolutely a trooper and we always tell her how awesome she is for dealing

with everything, so we thought maybe we should call it Dana’s Team Awesome,” says Jane Parrott, Dana’s mom.

Dana agrees with that origin story; however, she had one addendum.

“I mean, I think I’m awesome,” she says, laughing.

Dana loves attending MDA Muscle Walk each year not just because of the fun activities and events, but because of the way it brings their local MDA community together.

“I like everything [about MDA Muscle Walk,]” Dana says. “I love how the people come to help and the community comes



Dana Parrott makes a splash with her Muscle Walk team.

together. All my friends and family come to support me, and they have my back. And all the activities they have just make it that much more fun. Not only is it this great cause, but it’s also a lot of fun.”

**Join us at your local MDA Muscle Walk to bring strength to life and make an impact in your community. Visit [mdamusclewalk.org](http://mdamusclewalk.org) to find a walk near you and register today.**

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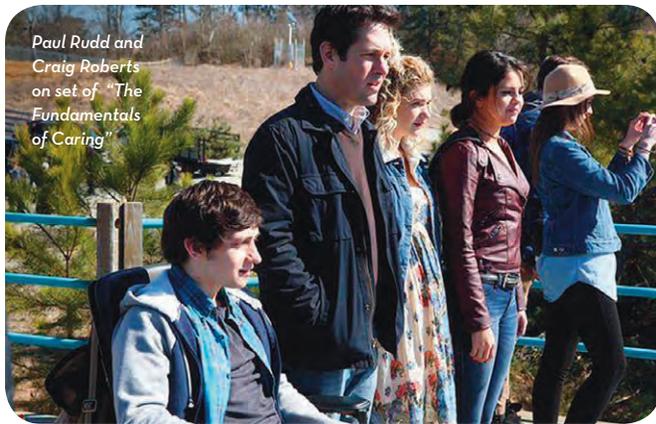
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Lindsay  
Age 12

# In The News



Paul Rudd and Craig Roberts on set of "The Fundamentals of Caring"

## FILM HIGHLIGHTING DUCHENNE MUSCULAR DYSTROPHY PICKED UP BY NETFLIX

"The Fundamentals of Caring" is an upcoming film starring Paul Rudd as a caregiver for a young man named Trevor (Craig Roberts) who has **Duchenne muscular dystrophy (DMD)**. The film focuses on the relationship between the two as they embark on a cross-country road trip together.

After premiering at the Sundance Film Festival in January, the film was quickly bought up by Netflix for digital streaming distribution. Netflix hasn't announced its release date yet, but it should be available later this year.

## NPR SHARES THE AMAZING STORY OF JILL VILES'S DISCOVERY

Listeners of "This American Life" on NPR may have heard the story of Jill Viles, a woman with **Emery-Dreifuss muscular dystrophy (EDMD)** who made an amazing discovery about her



Jill Viles and Priscilla Lopes-Schliep

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own condition. Doing her own research and using unorthodox resources such as Google images, Viles discovered a connection between herself and the Canadian Olympic hurdler Priscilla Lopes-Schliep. She determined that they both have EDMD, but due to a gene mutation, Lopes-Schliep's muscles grew rather than decreased. While at first doctors were skeptical of Viles's claims, a genetic test proved her right.

 Visit [thisamericanlife.org](http://thisamericanlife.org) for more details about this incredible story, featured in episode 577.

### LEGO REVEALS ITS FIRST FIGURE WITH A DISABILITY

Lego, the biggest toymaker in the world, revealed its first-ever figure with a disability in January. Refreshingly, the figure was released with little fanfare about its disability: a Lego figure in a wheelchair was simply included in a new playground-themed set to be released in Summer 2016.

It is clear that campaigns like #ToysLikeMe or Melissa Shang's petition to American Girl for a doll with a disability (previously covered in Quest) are making the argument to toymakers that

these products are in high demand. With Lego on board, it seems likely that more companies will follow.



PHOTO COURTESY OF LEGO

 The advertisement features three photographs: a group of people at a table, a person using the device on a porch, and a person using it on stairs. The LIFTKAR PT logo is prominently displayed in the center.
 

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With the launch of Strongly, MDA's new blog that you can find at [strongly.mda.org](http://strongly.mda.org), MDA is bringing together the stories and voices of people across the MDA community. On Strongly, you'll find everything from personal blogs by people living with neuromuscular disease to news and commentary on current research to fun stories and videos from members of the MDA community. All of the content can be easily filtered and searched, and new items are added daily.

 Check out Strongly at [strongly.mda.org](http://strongly.mda.org). And if you're interested in blogging or sharing your story for Strongly, contact us at [strongly@mdausa.org](mailto:strongly@mdausa.org).





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Not at any point during that day did I feel sad, scared or angry. The atmosphere was filled with inspiration, love, joy and laughter. I was proud to be a part of a ‘club’ comprised of such amazing people. The families of those affected by neuromuscular disease reached out to us with loving arms to let us know they were there for us in any way we needed them. With just a few words and a few minutes, I felt a bond with them like I’ve never experienced before ...

**The MDA Muscle Walk is so much more than just a fundraising event. The walk helps to heal the souls of people like me.”**  
— Molly, Fisher’s Mom



**Join us at your local MDA Muscle Walk to bring strength to life!**



Muscular Dystrophy Association

[mdamusclewalk.org](http://mdamusclewalk.org)  
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# More Than a Number

*Finding love when you feel like just another statistic*

BY RICHARD MCBRIDE



*After his ALS diagnosis and divorce, Richard McBride gets back into the dating scene.*

## Don't Go It Alone

MDA offers a number of support groups, including many that are disease- or group-specific (for caregivers, parents, etc.). To find MDA resources in your area, visit [mda.org](http://mda.org).

**I am a statistic.** In 2012, at 57 years of age, after almost two years of worsening symptoms, I was diagnosed with ALS. Being a white male at my age, in many ways I was the “typical” ALS patient. Of course with a disease like ALS, there really is no such thing as a typical case. Each of us is unique, each of us is different. This disease attacks men and women, young and old, of every race and religion. This disease attacks rich and poor alike. And yet it is a rare disease, so rare as to be deemed “orphaned,” at least until the Ice Bucket Challenge.

I am another kind of statistic. In that same year of 2012, 10 months prior to my diagnosis, my now ex-wife filed for divorce. I don't blame her; I had told her I wanted a divorce just a few months prior. I didn't know I had ALS. She didn't know I had ALS. Yet there I was battling both this terrible illness and a bitter divorce at the same time. Needless to say, I did not do well in either battle. The divorce was finalized in August 2013, after many months of angry courtrooms and even angrier negotiations.

I found myself on the wrong side of two statistics,

ALS and divorce, which merged together to leave me using a wheelchair for mobility, alone after 32 years of marriage, living hundreds of miles away from my children, needing home care, medical care and life care, struggling with losing my career, managing my ever-dwindling finances and still trying to enjoy my single life. My life, in a mere 12 months, had changed so dramatically as to be nearly unrecognizable.

For some time I struggled against these statistics, trying to meet women, date and build a future. I actually found some surprising success. As

I would say to my brother, “Apparently being handicapped is not a handicap.” The problem was that most of the women I met were not interested, nor capable, of entering into a relationship with someone like me, someone with my kind of future.

Then I met Cheryl, a woman who showed me that I could be loved, even as I was. My problem was that I did not feel for her as she felt for me. It just wasn’t there. While we remained good friends, she moved on with her life while I continued mine. For more than a year, I simply gave up, thinking

This situation did not happen right away. In fact it nearly didn’t happen at all. While Katherine was one of my caregivers, I was completely circumspect, not wanting to behave inappropriately, all the while thinking of her in a more and more romantic way. For nearly a year, she did range-of-motion exercises with me, not knowing how I felt about her. I had truly given up, and I had neither the courage nor the energy to take the risk of losing an excellent caregiver.

What I didn’t know is that she was developing feelings for me as well. Over time, as

she could no longer be my caregiver, or at least not for the foreseeable future. My response was immediate; I asked her if she would like to come over for coffee. She said yes.

Needless to say, I was surprised. Things moved forward, quickly. We went from seeing each other to dating to being in a relationship in a matter of weeks. Since that day, January 15, 2015, we have been together almost constantly, sharing our space, going out together and traveling together. We are a couple.

After giving up completely on having someone to share

*“I found myself on the wrong side of two statistics, ALS and divorce, which merged together to leave me using a wheelchair for mobility, alone after 32 years of marriage, living hundreds of miles away from my children, needing home care, medical care and life care, struggling with losing my career.”*

that it would never happen. Seasons passed and I was still alone, steadily becoming firmer in the realization that I was done with love, done with sex and done with passion.

What I didn’t realize is that there was someone with me on a regular basis who would become the very person I could be passionate about. I met Katherine, a woman I could and did fall for, a woman who could and did fall for me. Once again, I am a statistic. How Katherine and I met is so common as to be a cliché. She was one of my caregivers.

she cared for me, she came to admire my approach to life, my willingness to keep working, my open way of sharing my life with her and listening to her as she shared hers with me. Just as I was constrained by her role in my life, she was constrained as well. She would not break her professionalism. She would not be inappropriate with a client.

Then a miracle happened. Katherine fell and broke her arm. This may not seem like a miracle to you, but to me it was heaven sent. You see, as soon as she fell and broke her arm, she called to tell me

my life, here I am with a terrific woman who cares for me, supports me and spends time with me. Here I am even after I had given up on this aspect of my life. I’ve learned a real lesson from all this: It is still possible to find love, passion, companionship and partnership, even with ALS. And that too is probably a statistic. **Q**

*Richard McBride was diagnosed with ALS in November 2012 and shares his life and experiences through his blog, [richardislivingwithals.blogspot.com](http://richardislivingwithals.blogspot.com). He lives in Calgary, Alberta, Canada.*

# Camp Connection

Young disability rights activist thrives at her first MDA Summer Camp – and beyond



Sylvia Colt-Lacayo feels at home at MDA Summer Camp.

**F**or Sylvia Colt-Lacayo, a high school freshman with muscular dystrophy from Oakland, Calif., attending her first MDA Summer Camp at age 14 was a revelation.

“My favorite thing wasn’t even an activity, although all the activities were great,” she says. “It was the fact that it was a week where I never felt insecure about my wheelchair and the different things that happen with my body. I felt comfortable because everyone around me had similar issues or knew what it was like to deal with those issues. The only way I can describe it is basically for a week I get to accommodate the world, the world doesn’t have to accommodate for me.”

For Sylvia, this experience underlined the importance of the work she does to advocate for the rights of people with disabilities. Sylvia is MDA’s California State Goodwill Ambassador and also serves on the youth advisory committee for the University of California, San Francisco’s Benioff Children’s Hospital.

“Being a teen is already really hard,” Sylvia says, “and being a teen with a disability you can feel like no one understands you. I wanted to help younger kids that were dealing with this.”

In addition to helping individuals, in her role as an activist Sylvia hopes to get her message of disability awareness out to as many people as possible. The underrepresentation of individuals with disabilities in the media is of particular importance to her, and she hopes to change that by creating films of her own.

She is currently a student at the Bay Area Video Coalition and has directed a short documentary film with plans for a narrative film in the near future.

And while filmmaking, school, advocacy and her other interests keep her very busy, Sylvia is already excited about living unlimited at MDA Summer Camp this summer.

“I’m looking forward to being in that environment again and feeling comfortable,” she says.

“It’s hard to explain, but most of my friends are able-bodied and my wheelchair isn’t a barrier for them — or for me, either — but they will never fully understand the most important parts of me. [With other campers,] we relate on a higher level.” **Q**

## Check Out MDA Summer Camp

For more information about MDA Summer Camp, and how you can get involved, visit [mda.org/services/mda-summer-camp](http://mda.org/services/mda-summer-camp).

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\*Deflazacort is an investigational medication that has not been approved by the Food and Drug Administration (FDA) and is therefore not proven to be safe and effective.

To learn more about **ACCESS DMD™**, please visit [www.accessdmd.com](http://www.accessdmd.com)

The **ACCESS DMD™** team can be reached at: **1-844-800-4DMD**  
**(1-844-800-4363)**



A man with a goatee and tattoos is shown from the back, sitting in a black ADI wheelchair. He is wearing a black suit jacket over a white baseball jersey with a red number '3'. He is holding a white volleyball in his left hand. The background is a blurred crowd in a stadium.

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