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WINTER 2018

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## A Word from President & CEO Lynn O'Connor Vos

Since joining MDA as president and CEO in October, I've had the sincere pleasure of spending time with and learning from our families, leading clinical experts, renowned researchers, dedicated sponsors, and passionate MDA staff and volunteers. The progress we're making together is unprecedented, and I know it is only the tip of the iceberg. Working together, I see incredible opportunities to push the limits of neuromuscular disease research and provide an even better health care experience for individuals and their families.



Many of you have probably been wondering what the future holds for MDA and how we will continue to advance research, innovation, family care and support in 2018 and beyond. By partnering with our community, we are mapping a strategic plan and specific focus areas to transform and save lives. You'll hear more about this in detail from me throughout the year.

Additionally, I hope you will read the Q&A on page 33 in which I answer questions from *Quest* readers about my experience, where I see the greatest opportunities for MDA to help families and what you can expect in the days and months to come. By combining my passion for patient care and work as a clinical nurse with deep experience leading health care companies in a new era of technology and innovation, I am committed to accelerating treatments, cures and care together for every MDA family.

I look forward to getting to know you and encourage you to reach out to me and share your feedback and ideas. Stay in touch with me on Twitter ([@lynnvos](https://twitter.com/lynnvos)), and be sure to read MDA's blog Strongly ([strongly.mda.org](http://strongly.mda.org)), where I'll be posting as a regular contributor. At MDA, progress is our promise. I look forward to working with and for you to deliver on that promise to bring hope and answers for a brighter, healthier future.

"I see incredible opportunities to push the limits of neuromuscular disease research and provide an even better health care experience for individuals and their families."

– Lynn O'Connor Vos

Sincerely,

Lynn O'Connor Vos  
President and CEO  
Muscular Dystrophy Association

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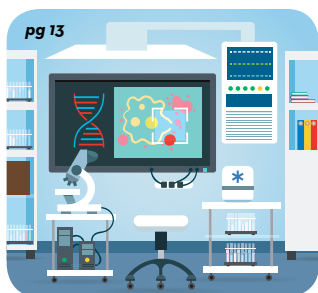
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## MORE ONLINE

### CATCH THE DO-IT-YOURSELF SPIRIT

MDA families are used to being problem-solvers. In a Quest online exclusive article, we explore how real MDA families and those who support them are using innovative DIY adaptive equipment solutions – both high-tech and low-tech – to improve everyday life and gain independence. Read “A World of Opportunities” at [mda.org/quest](http://mda.org/quest).

On the cover: The Labbadia family, pg 20



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**Giving Strength to Others**

MDA's mission is to free individuals – and the families who love them – from the harm of muscle-debilitating diseases. Every day, people who don't have neuromuscular disease – whether they are family, friends or acquaintances of someone who does – go to amazing lengths to support that mission. On MDA's Strongly blog ([strongly.mda.org](http://strongly.mda.org)), some of these generous individuals explain why they do what they do.

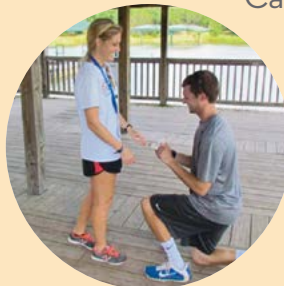


Ron Minz, a lieutenant with the Baltimore County Fire Department, loves visiting MDA Summer Camp on Fire Fighter Day. After a camper in a wheelchair asked to ride in a

fire truck, Minz built a 28-foot-long accessible fire truck, which he takes to Baltimore and Washington, D.C., area MDA Summer Camps every year.

*"The look on the kids' faces is well worth the time and effort and money spent. I do what I do because I like what I do, and it's the right thing to do."*

Newlywed Erin Foster met her husband, Brandon, at MDA Summer Camp, where they



both volunteered as counselors for years. That time is so meaningful to them that, in lieu of wedding gifts, they asked for donations to MDA.

*"There is so much joy seeing these kids at camp. It shows us how we want to live our lives both now and in the future."*

At the age of 25, Richard Leon became a Summer Camp counselor for the first time. Now, at the age of 30, Leon is a city council member in Cape Coral, Fla. He participates in MDA Lock-Up, supports local Fill the Boot drives and encourages other city leaders to get involved with MDA.



*"The lessons I have learned about compassion, patience and how precious life is have allowed me not only to become a better person but a better representative for the people."*

In October 2017, Katie Williams ran the Chicago Marathon with MDA Team Momentum. She was introduced to MDA by her college friend Joe Akmakjian, former MDA National Ambassador.

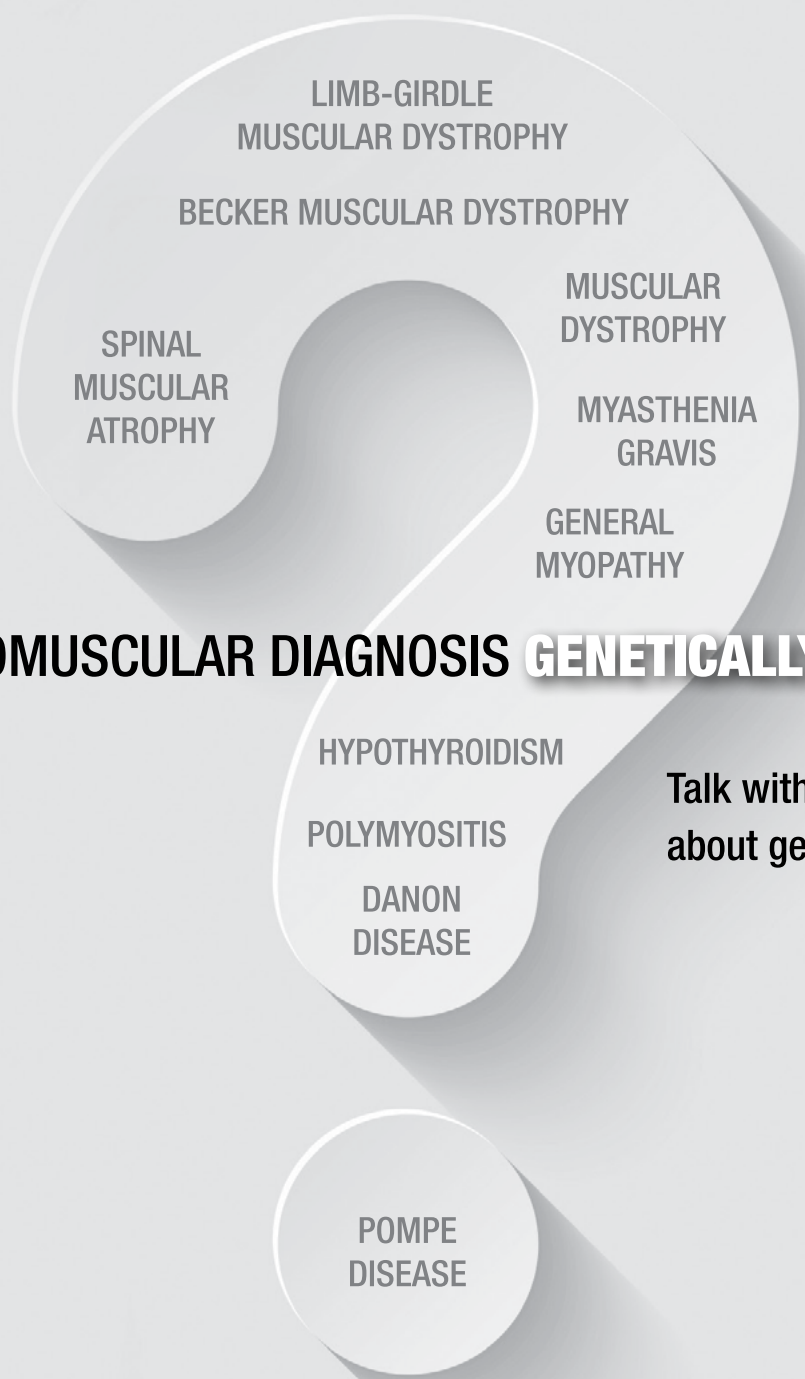


*"I've been wanting to do more for others and thought this might be an awesome way to do so."*

**Get Involved**

Find your passion, and get involved in the MDA community in a way that is meaningful for you. Learn how you can help families live longer and grow stronger at [mda.org/get-involved](http://mda.org/get-involved).

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## MDA Research In Action

# MDA Research Dollars Power Progress

### New MDA grants will impact a range of neuromuscular diseases



In November, MDA announced 13 new MDA research and development grants, with a total funding commitment of \$3.5 million, that are now supporting research projects around the world.

The new projects cover a broad range of diseases in MDA's program and are intended to impact the greater neuromuscular disease landscape.

The new awards constitute MDA's Summer 2017 grant cycle and are in addition to other awards announced throughout 2017.

- **Announced Jan. 24:** An MDA clinical research network grant totaling \$918,000 over three years will spur advances in myotonic dystrophy (DM) research. The investment, which provides continued support for the Myotonic Dystrophy Clinical Research Network, will support five medical centers that specialize in DM research and clinical care. The network is led by Charles Thornton, professor of neurology at the University of Rochester, who serves as the network's director. Goals are to gain a more detailed understanding of the DM disease process and to collect data needed for clinical trials in order to inform what outcome measures, biomarkers and endpoints will be most appropriate.
- **Announced March 9:** MDA's Winter 2017 round of grants, totaling 29 awards worth more than \$7 million, began funding projects covering a number of diseases in MDA's program on Feb. 1.



- **Announced March 10:** Izumi Biosciences in Lexington, Mass., was awarded an MDA Venture Philanthropy (MVP) grant totaling \$96,360 to fund early-stage development of IZ10023, a type of drug called a “pharmacokinetic (PK) enhancer,” which could enhance the effectiveness of other drugs people with ALS are taking, such as riluzole.
- **Announced June 14:** Michael Benatar, at the University of Miami Miller School of Medicine in Florida, and Jonathan Katz, at California Pacific Medical Center in San Francisco, were awarded a clinical research network grant to support their work on the Clinical Procedures to Support Research (CaPTuRe) project, which aims to implement the “ALS Toolkit,” a system to collect clinical data in such a way that it can be used for ALS research. The two-year award totaling \$300,000 will support work conducted through Clinical Research in ALS and Related Disorders for Therapy Development (CReATe), aimed at lessening the burden on people with ALS to attend both clinical and research appointments.
- **Announced July 11:** Johanna Hamel, a neurologist at the University of Rochester in New York, was awarded a clinical research training fellowship for her work in comparative studies of RNA toxicity in DM. The two-year award – co-sponsored by MDA with the American Academy of Neurology and the American

Brain Foundation – will provide a total of \$130,000, including a \$10,000-per-year stipend for tuition, to support Hamel’s work to shed light on the molecular processes that drive DM.

- **Announced Aug. 30:** Nicholas Johnson, assistant professor of neurology, pediatrics and pathology at the University of Utah in Salt Lake City, was awarded a human clinical trial grant totaling \$598,348 over three years to conduct a natural history study in congenital myotonic dystrophy (DM1).
- **Announced Oct. 1:** Maya Maor Nof, a postdoctoral research fellow at Stanford University School of Medicine in California, was awarded the 2017 SSSI-MDA Fellowship Award. The award, co-sponsored by Strength, Science & Stories of Inspiration (SSSI), will provide a total of \$40,000 over two years to support Maor Nof’s work to shed light on the mechanisms underlying nerve cell death in ALS.
- **Announced Nov. 3:** Iron Horse Diagnostics in Scottsdale, Ariz., was awarded an MDA Venture Philanthropy (MVP) grant totaling \$233,200 to support development of a prognostic (predictive) test for ALS. Iron Horse Diagnostics Chief Scientific Officer Andreas Jeromin will serve as the principal investigator on the project.



**MDA currently is funding about 150 research projects worldwide. Read more about MDA grants at [mda.org/gaag](https://mda.org/gaag).**

## ALS (amyotrophic lateral sclerosis)

# Centaur ALS Trial Seeks Participants

**Trial will test therapy designed to block nerve cell death**



*Trial length is approximately 28 weeks, during which participants will visit with study investigators seven times in person and four times via phone.*

Researchers are looking for people with ALS to participate in the CENTAUR ALS clinical trial. Sponsored by Amylyx Pharmaceuticals, the phase 2 trial is designed to test the safety and tolerability of the experimental drug AMX0035 and determine whether the treatment is able to slow decline of function in people with ALS.

AMX0035 is a combination therapy, designed to block nerve cell death and reduce inflammation to slow the progression of ALS.

At seven in-person visits, participants will undergo strength testing, blood draws and other assessments to enable investigators to determine whether AMX0035 has any effects on muscle strength and respiratory function, as well as help them determine whether biomarkers that can signal nerve cell death are present.

In order to be eligible to participate, individuals must be 18 to 80 years old, have a definite diagnosis of ALS and meet other eligibility criteria.

The trial is taking place at 25 trial sites across the United States, and support for travel costs may be available.



**To learn more about this trial, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT03127514 in the “Other Terms” search box, or contact study coordinator Carly Doyle at [855-437-4823](tel:855-437-4823) or [alstrials@neals.org](mailto:alstrials@neals.org).**

Charcot-Marie-Tooth disease (CMT)

# Participants Sought for ACE-083 CMT Study

**This will be the first test of ACE-083 in people with CMT**



*Acceleron is developing ACE-083 to treat diseases in which improving muscle atrophy and weakness may provide a clinical benefit.*

Researchers are looking for people with CMT1 and CMTX to participate in a phase 2 clinical trial, sponsored by Acceleron Pharma, to test the investigational drug ACE-083.


Delivered by intramuscular injection, ACE-083 is based on a naturally occurring protein and is designed to enhance the body's own promoters of muscle growth specifically in the muscles into which the drug is administered. Targeting the tibialis anterior (a long, narrow muscle on the front of the shin) in CMT with the drug could improve ankle dorsiflexion, which could in turn lessen foot drop (diffi-

culty lifting the foot at the ankle, so that the toes point downward during walking).

Total study duration for each patient will be approximately 24 weeks, including a four-week screening period, a 12-week treatment period and an eight-week follow-up period after the last dose.

To be eligible to participate, individuals must be at least age 18, have genetically confirmed CMT1 or CMTX, or have a first-degree relative with genetically confirmed CMT1 or CMTX and clinical signs/symptoms of CMT1 or CMTX, and meet additional criteria.

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

 To learn more about this trial, go to [ClinicalTrials.gov](https://clinicaltrials.gov) and enter NCT03124459 in the "Other Terms" search box, or email [clinicaltrials083@acceleronpharma.com](mailto:clinicaltrials083@acceleronpharma.com).



*Strengthening of foot and ankle muscles through exercise could potentially lessen the disability caused by CMT and improve quality of life for children with the disease.*

## Exercise May Preserve Strength in CMT

**In study, progressive resistance exercise strengthened muscles over a two-year period**

Results from an MDA-supported study conducted at the University of Sydney (Australia), show that progressive resistance exercise not only is safe, but it can help to significantly reduce the muscle weakness experienced by children with CMT.

In the study, which involved a total of 60 children with different types of CMT ages 6 to 17, participants completed a regimen consisting of exercising three times per week for six months, using a custom-built exercise cuff for the foot (similar to ankle weights commonly available in sports stores). After initial supervised training sessions, the children completed the exercise regimen at home.

The investigators found that six months of moderate-intensity progressive resistance exercise could help not only slow the progression of muscle weakness by up to 30 percent compared to CMT patients who did not exercise, but it even led to increases in strength for the exercised muscles over a two-year period.

It is hoped that increasing the strength of these muscles through an exercise routine potentially could lessen the disability caused by CMT and prolong function, leading to improved quality of life.

 To read about MDA's current CMT-related research efforts, visit [mda.org/gaag](https://mda.org/gaag).

## Duchenne muscular dystrophy (DMD)

# FDA Says 'No' to Translarna

**Advisory committee determined data in support of the drug were inconclusive**



PTC Therapeutics reported on Oct. 25, 2017, that it received a Complete Response Letter from the U.S. Food and Drug Administration (FDA)

indicating that the agency is unable to approve the company's New Drug Application for ataluren (brand name

Translarna) for the treatment of DMD, in its

current form. The company has filed a formal dispute resolution.

Translarna is designed to act by changing the way muscle cells interpret genetic information, coaxing them to produce a needed muscle protein called dystrophin despite the presence

of a nonsense mutation in the DMD gene.

In trials, the drug has demonstrated mixed results. In PTC Therapeutics' phase 3 "ACT DMD" clinical trial of Translarna, the drug failed to meet its primary endpoint. However, post-hoc analysis indicated that the drug may slow functional decline in a subset of DMD patients, while other patients were not responsive to treatment.

Translarna received conditional approval in the European Union in August 2014 for use in people with DMD caused by a nonsense mutation who are at least age 5 and able to walk.

MDA has funded groundbreaking DMD research for more than 65 years, including early clinical testing of Translarna.

*The FDA indicated that evidence from an additional adequate and well-controlled clinical trial will be necessary, at a minimum, to provide substantial evidence of efficacy.*



To read more about the FDA decision, visit [strongly.mda.org](http://strongly.mda.org) and search for "Translarna."

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**Duchenne muscular dystrophy (DMD)**

# Golodirsen Shows Potential to Treat DMD

**Exon skipping drug aims to lessen muscle weakness and atrophy**

Sarepta Therapeutics reported encouraging results from a phase 1/2 clinical trial that suggest its experimental drug golodirsen may be effective as a treatment for DMD.



*Although treatment with golodirsen would not cure DMD, it could slow progression of the disease which, in turn, could extend the length of time boys with DMD could walk, eat independently and breathe without assistance.*

Golodirsen is an exon skipping drug designed to slow disease progression in DMD. Administered by intravenous infusion, it targets a section of genetic code called exon 53, and may help up to 8 percent of boys with the disease.


Results from the trial, which tested the drug in 25 boys with DMD, showed that treatment with

golodirsen for approximately one year was associated with a statistically significant increase in dystrophin protein production. Muscle biopsies confirmed that all participants responded to the drug, as measured by increased dystrophin.

The 25 boys who participated in the trial will continue to be evaluated for a total of 144 weeks, and Sarepta has said it plans to release study data at a future medical or scientific conference.

Golodirsen also is being evaluated in the ongoing phase 3 ESSENCE study in boys with DMD gene deletions amenable to skipping exons 45 or 53. This trial currently is recruiting approximately 100 people to participate at sites across the United States, Canada and Europe.

MDA has been central to development of the exon skipping approach.

 For more information about the ESSENCE trial, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT02500381 in the “Other Terms” search box.

**Friedreich’s ataxia (FA)**



*Orphan Drug Designation is important to companies like Jupiter Orphan Therapeutics, because it makes them eligible for special incentives that have been put in place to encourage the development of treatments for rare disorders.*

# FDA Grants Orphan Drug Designation to JOTROL

**Designation encourages the development of treatments for rare disorders**

The investigational drug JOTROL, under development by Jupiter Orphan Therapeutics to treat FA, has received U.S. Food and Drug Administration (FDA) Orphan Drug Designation.

JOTROL is a unique formulation of trans-resveratrol. Resveratrol is a naturally occurring compound that has been investigated in a number of neurodegenerative and neuromuscular diseases, due to its positive effects on energy metabolism and mitochondria (the “energy factories” in cells), and a host of antioxidative, anti-inflammatory and anti-aging activities.

Orphan Drug Designation may help facilitate development of JOTROL for the treatment of FA, as it provides incentives meant to encourage Jupiter Orphan Therapeutics to develop and market it.

Results from an open label phase 2 trial of resveratrol conducted by Murdoch Children’s Research Institute in Melbourne, Australia, showed that participants taking resveratrol demonstrated improvement of neurological symptoms. However, treatment with higher doses of the drug was associated with gastrointestinal (GI) tolerability issues. Jupiter Orphan Therapeutics has said it plans to conduct a larger placebo-controlled study designed to assess whether treatment with JOTROL can generate the same beneficial effects without the unwanted GI side effects.

 To read about MDA’s current FA-related research efforts visit [mda.org/gaag](https://mda.org/gaag).



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GNE myopathy/HIBM

# Ultragenyx Discontinues Ace-ER Program

**Trial results showed no benefit, as compared to placebo**

Ultragenyx Pharmaceutical announced that a phase 3 study to evaluate aceneuramic acid extended release (brand name Ace-ER) for the treatment of GNE myopathy, also known as hereditary inclusion-body myopathy (HIBM), and Nonaka myopathy, failed to meet its primary and key secondary endpoints.

Results showed no significant difference in the upper extremity muscle strength of


those treated with Ace-ER as compared with those who received a placebo. Therefore, Ultragenyx has said it will not file with the U.S. Food and Drug Administration (FDA) for approval of Ace-ER and has discontinued further clinical development of the drug.

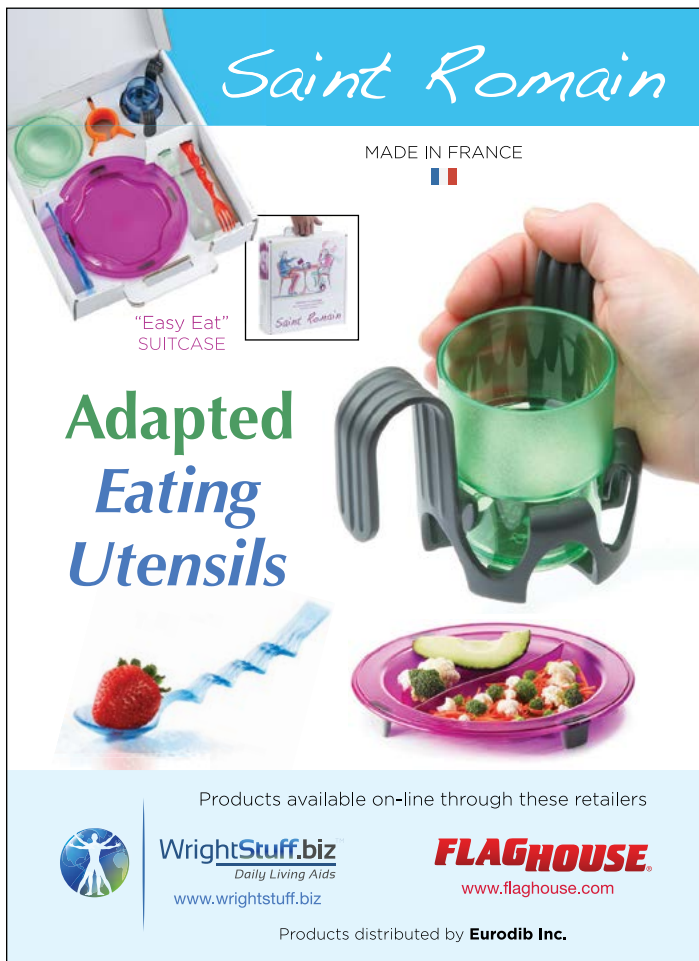
The company reported it will work with investigators and patient groups to make available the valuable natural history data and development

tools associated with the trial for the development of other therapies.

*Natural history data and development tools for the trial will be made available to help inform the development of other therapies.*



 **If you participated in the Ace-ER trial, be sure to speak with your physician about any questions you may have regarding your trial experience and treatment plan going forward.**




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## Mitochondrial myopathy

# FDA Grants Orphan Drug Designation to Elamipretide

### In studies, treatment was associated with clinical benefits

The investigational drug elamipretide, under development by Stealth BioTherapeutics to treat muscle weakness caused by mitochondrial disease, has received U.S. Food and Drug Administration (FDA) Orphan Drug Designation.

Elamipretide is designed to modify disease by helping restore normal energy

production in mitochondria and decrease oxidative stress, an imbalance between the production of free radicals and the body's ability to neutralize them. Mitochondria are small, essential organelles that populate every cell of the body and are often referred to as the "powerhouse of the cell," since they generate most




Orphan Drug Designation allows for special incentives to encourage the development of treatments for rare disorders.

of the energy in an organism.

Orphan Drug Designation may facilitate development of elamipretide for the treatment of mitochondrial myopathy by providing incentives meant to encourage companies like Stealth BioTherapeutics to develop and market drugs for rare disorders.

Stealth BioTherapeutics currently is recruiting participants for its RePOWER trial, an observational study of people with primary mitochondrial disease. RePOWER is designed

to better characterize and correlate symptoms and signs of myopathy, genetic test results and the use of commonly prescribed treatments. Participants in the RePOWER study may have the opportunity to participate in a future phase 3 trial for elamipretide.

 To learn more about the observational study of individuals with mitochondrial disease, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT03048617 in the "Other Terms" search box.

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**Myasthenia gravis (MG)**

# FDA Approves Soliris

**Soliris becomes the first in a new class of drugs to be approved for MG in the U.S.**

Alexion Pharmaceuticals announced on Oct. 23, 2017, that the U.S. Food and Drug Administration (FDA) approved eculizumab (brand name Soliris) as a treatment for adults with generalized myasthenia gravis (MG) who are anti-acetylcholine receptor antibody-positive.

Soliris was tested in clinical trials in people who had previously failed immunosuppressive treatment and continued to suffer from significant unresolved disease symptoms such as difficulties seeing, walking, talking, swallowing and breathing. Participants taking Soliris had improved scores on scales designed to assess quality of life and symptom burden including double vision, ptosis (drooping of the eyelids), swallowing, speech, breathing, and use of arms and legs.

Soliris is a terminal complement inhibitor that targets a part of the immune system called the complement system, which is



*Treatment with Soliris will not cure generalized MG, but it may improve disease symptoms, the ability to carry out activities of daily living and quality of life.*

responsible for helping antibodies clear damaged cells and is inappropriately activated by antibodies present during MG. Soliris is thought to work in MG by inhibiting the complement pathway to prevent destruction of the neuromuscular junction.

If you are interested in adding Soliris to your treatment regimen, you should speak with your health care provider, who will initiate treatment, if appropriate.



**If you are interested in Soliris, speak with your health care provider about whether the treatment is right for you. You may learn more by visiting [info.soliris.net/gmg-patient](http://info.soliris.net/gmg-patient) or calling 1-888-SOLIRIS to speak with a nurse case manager through OneSource, a patient support system from Alexion Pharmaceuticals, the manufacturer of Soliris. The Alexion nurse case managers can answer your questions about Soliris.**

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Pompe disease (acid maltase deficiency, AMD)

Spinal muscular atrophy (SMA)

## FDA Grants Orphan Drug Designation

Designation encourages the development of treatments for rare disorders



The investigational combination drug therapy ATB200/AT2221, under development by Amicus Therapeutics to treat Pompe disease, or AMD, has received U.S. Food and Drug Administration (FDA) Orphan Drug Designation.

Amicus Therapeutics' combination drug strategy pairs ATB200, a synthetic acid alpha-glucosidase (GAA) enzyme that is deficient in Pompe disease, with AT222, a companion drug that helps the therapeutic GAA enzyme be more effective. AT222 is a "pharmacological chaperone," designed to protect the GAA enzyme while it is circulating in the bloodstream. Data from previous studies suggest that co-administration

of the two drugs results in enhanced uptake and activity in muscle tissue of the replacement enzyme.

Orphan Drug Designation may help facilitate development of ATB200/AT2221 for the treatment of Pompe disease, as it provides incentives meant to encourage Amicus Therapeutics to develop and market it.

A phase 1/2 clinical trial currently is underway to test the combination therapy in Pompe disease.



For more information about the phase 1/2 trial, visit [ClinicalTrials.gov](https://clinicaltrials.gov) and enter NCT02675465 in the "Other Terms" search box.

## STRIVE SMA Gene Therapy Study

Participants sought to test efficacy of gene replacement therapy

Researchers are looking for individuals with type 1 SMA to participate in the phase 3 STRIVE clinical trial, sponsored by AveXis, to >

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## Spinal muscular atrophy (SMA)

*continued from previous page*

test efficacy for its SMA gene replacement therapy AVXS-101.

Administered by one-time intravenous infusion (a needle inserted into a vein), AVXS-101 is designed to deliver a gene that can produce the Survival Motor Neuron (SMN) protein. SMN is critical to the function of the nerves that control muscles and is missing in individuals with SMA. Increased production of the protein may lead to improvements in muscle strength and function.

All participants in the open-label STRiVE study will receive treatment with the experimental therapy. Study-related visits, tests and treatments will be provided to participants at no cost.

To be eligible to participate, individuals must be under 6 months old, have one or two copies of the SMN2 gene, and meet additional criteria.

Trial sites are located in California, Colorado, Illinois, Maryland, Ohio, Oregon and New York. Travel assistance may be available for families who don't live near one of the research centers.



**To learn more about this trial, go to**

**ClinicalTrials.gov and enter NCT03306277 in the "Other Terms" search box, or visit [studysmanow.com](http://studysmanow.com) and email the trial coordinator at the site nearest you.**

## FIREFISH SMA Study

### Participants needed for phase 2 trial to test RG7916

Researchers are looking for individuals with type 1 SMA to participate in the phase 2 FIREFISH clinical trial, sponsored by Hoffmann-La Roche, to test the investigational drug RG7916.

A liquid medication, RG7916 is delivered once daily by mouth or through a feeding tube.

RG7916 is an SMN2 splicing modifier that distributes throughout the entire body. It is designed to modify SMN2 messenger RNA splicing and increase levels of the needed SMN protein. (RNA is the chemical step between DNA and protein production.)

All participants in the open-label FIREFISH study will receive



*Goals of the trial include assessment of the safety, tolerability and efficacy of RG7916 in type 1 SMA.*

treatment with the experimental therapy. In addition, they will receive all study-related procedures and exams, including physical exams, motor function tests, CMAP (testing that assesses the interaction of muscles and nerves during activity), blood sample testing and eye examinations.

Participation is expected to last 24 months. To be eligible, individuals must be ages 1 to 7 months, have a confirmed diagnosis of SMA, have two copies of the SMN2 gene (confirmed by testing) and meet additional criteria.

U.S. trial sites are located in California and New York, and travel support may be available for families who don't live nearby.

**To learn more about this trial, go to [ClinicalTrials.gov](http://ClinicalTrials.gov) and enter NCT02913482 in the "Other Terms" search box.**

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# Startup Support

*How to take the lead and create a support group for your community*

BY LESLIE KRONGOLD, Ed.D.



Author Leslie Krongold (far right) meets with a support group.

**O**nce I was diagnosed with myotonic muscular dystrophy (DM), I sought a support group to learn more about my disease and meet others with the same diagnosis. Shortly after I began attending, the facilitator left the group and I was asked to take over her responsibilities. Talk about trial by fire.

This experience dramatically changed the course of my life. Not only did I meet wonderful families impacted by neuromuscular diseases, but I connected with organizations providing services and resources for people with varying levels of abilities. Twenty years later, support

group facilitator still is a title I proudly wear.

## START YOUR GROUP

What I've witnessed anecdotally in my own support groups is backed by evidence-based research: Social support experienced by support group participants improves coping skills, lowers depression and enhances quality of life. But not everybody has access to these benefits.

Tracey R. Young, of Beaumont, Texas, recalls how excited she was to meet and learn from peers at her first myasthenia gravis (MG) support group meeting. The problem was that the meeting was in Houston, 100 miles away. That prompted Young

to launch an MG support group in her town.

She started by contacting the Houston support group facilitator, who introduced Young to a larger network

of Texans with MG and their families and friends. Through this network, Young identified a co-facilitator, who was a caregiver for her grandmother, and the two of them “got the word out to get people talking,” Young says. This included sending out Facebook messages and notifying other people in the area. Once they found a meeting place and set up monthly meeting times, they connected with two patient advocacy organizations, as well as the local neurologist, to help spread the word about the meetings.

Delegating responsibilities has been the key to the group's success. For example, “We have a greeter, and that person's job is to greet everyone who comes in,” Young

## VIRTUAL SUPPORT GROUPS

When meeting in person is not feasible, some groups create a virtual presence. A virtual group leader determines the platform and organizes meetings or activities, similar to an in-person group. Technology provides multiple ways to do that. Here are a few:

- Start a private Facebook group and invite others to join ([facebook.com](https://www.facebook.com)).
- Start a Meetup group that allows others in your area – or even around the world – to join ([meetup.com](https://www.meetup.com)).
- Use a free audio conferencing service to schedule group conference calls.
- Set up Skype video chats ([skype.com](https://www.skype.com)).

## CONSIDERING LOGISTICS

Although the stereotype of a support group may conjure an image of people sitting in a circle taking turns sharing feelings, many groups also incorporate education and involve guest speakers, or meet in restaurants for social connections. Determining the meeting format is part of establishing your group's logistics:

**Identify a meeting location.** The best meeting place is a public location that is ADA accessible (including the restrooms). Explore hospitals, libraries and places of worship. Take parking and public transportation options into consideration.

**Determine your meeting frequency.** Try not to overschedule in the beginning. Once your group starts to gel, you may opt to meet more frequently. Be sure to publicize your meeting at least one month ahead of time to give attendees time to plan.

**Choose a meeting format.** Select a time and day that works well for you. No time will work for everyone, but your presence is critical. The average duration of a support group meeting is two hours. Some families will travel up to two hours to attend the meeting; make it worth their while.

**Make a plan.** Create an agenda to structure the time. Allow for introductions, presentations or guest speakers, and discussion. Start and end the meeting on time; don't penalize the punctual. Bring healthy refreshments, and consider sharing this responsibility with participants.

explains. "It does two things: It makes new people feel welcome, and it gives that person purpose beyond themselves to actually commit to being at the support group meeting."

## SHARE THE WORK

Especially for a peer facilitator — someone who shares the same diagnosis as the group — it's helpful to have a co-facilitator. Suzette Smith of Indianapolis, whose adult son has myotonic dystrophy, launched a support group 15 years ago. After experiencing

burnout, she rebooted the group two years ago with the help of a co-facilitator. "That helps tremendously," Smith explains. "You have a buddy to talk with if no one shows, and if you end up with a full room, you have help keeping things organized and a good flow."

A support group facilitator's main function is to plan and guide the meeting so that all participants leave feeling a little better than when they arrived. Although having tasty refreshments can go a long way toward satisfying

attendees, the critical aspect to any meeting is good, clear communication. This includes your communication with the group, creating a safe environment to encourage participants to speak, and ensuring that any guest speakers provide helpful information.

## A SAFE SPACE

In my experience, the biggest challenge in facilitating a support group is handling open conversation. A facilitator's goal is to draw out the quietest people. There is often a fine line between keeping the conversation energized and interesting while not allowing someone to dominate. My skills in this area have improved over the years. Strive to become a better listener and gauge the unspoken communication in the room. And sometimes you may need to exercise the refrain, "Let's take this offline," where appropriate.

To establish a safe environment for everyone, draft ground rules to discuss with the group. Most important is for everyone to respect confidentiality and refrain from crosstalk when someone is speaking. Melissa Byrne, a clinical social worker in Richmond, Va., who facilitates an ALS support group, invites participants to contribute to the guidelines through a survey. The feedback "shapes group topics, provides insight into their experiences and encourages us to make changes when appropriate," says Byrne.

## Contact MDA's Resource Center

MDA's Resource Center is an informational hub for families living with neuromuscular disease. Contact the MDA Resource Center at [800-572-1717](tel:800-572-1717) or [resourcecenter@mdausa.org](mailto:resourcecenter@mdausa.org). MDA's trained resource specialists are available Monday through Friday, 8 a.m. to 5:30 p.m., Central time, to answer questions and provide one-on-one support and resources. They typically are able to answer questions within 24 hours of a request (or on the next business day). If you attend or lead a support group in your area, contact us today so we can include that information in our resource referral database.

## DO IT FOR YOUR COMMUNITY

Receiving a difficult diagnosis for yourself or a loved one can make you feel alone. But you're not. There is a community of individuals in a similar situation. And if you can't find a support group for that community, chances are others need it just as much as you do. So don't be afraid to become a support group facilitator. It will become a title that you proudly wear, too. [Q](#)


*Leslie Krongold, Ed.D., 55, lives in Alameda, Calif. After 20 years of support group facilitation and training peer and caregiver support group facilitators, she's taken her passion to a podcast/blog at [GlassHalfFull.online](#).*



# THE Team APPROACH

*Multidisciplinary care improves quality of life  
for individuals with neuromuscular diseases*

BY KAREN HENRY



Brendan is a curious, bright-eyed 7-year-old with a fun-loving attitude and a buoyant smile. He was diagnosed with Duchenne muscular dystrophy (DMD) when he was 23 months old. His mother, Colleen Labbadia, describes his diagnosis as long and heartbreaking but adds that there has been a bright spot. “Brendan is seen at the MDA Care Center at Nemours Children’s Hospital in Orlando, and we absolutely love it,” she says. “They have been our silver lining.”





*“Brendan is seen at the MDA Care Center at Nemours Children’s Hospital in Orlando, and we absolutely love it. They have been our silver lining.”*  
– Colleen Labbadia

Nothing about living with neuromuscular disease is easy or convenient. Effectively managing progressive muscle weakness as well as the various associated complications individuals may experience requires an equally varied list of specialists.

Brendan’s health care providers include a neurologist, physical therapist, occupational therapist, cardiologist, orthopedist, endocrinologist and nutritionist. These professionals work as a team to provide Brendan with

comprehensive multidisciplinary care.

The MDA Care Center at Nemours is just one of the more than 150 MDA Care Centers across the United States and Puerto Rico that provide multidisciplinary care to individuals with neuromuscular diseases.

### WHAT IS MULTIDISCIPLINARY CARE?

Multidisciplinary care brings together the expertise of professionals from a wide

range of disciplines in one location to provide coordinated, patient-centered care. A recent study showed that this is the gold standard model of care for individuals with complex medical needs.

MDA is committed to ensuring that the MDA Care Center network operates with a multidisciplinary approach. MDA Care Centers include strong medical teams, but they don’t just have doctors. In addition, each MDA Care Center includes a social worker, a physical therapist and a care center coordinator. (See “Specialists at a Glance.”)

Beyond this framework, the specialists included on an MDA Care Center team vary based on the conditions they manage and the age range of the individuals they work with. Likewise, not all MDA Care Centers function in exactly the same way. At some MDA Care Centers, individuals will see all their relevant specialists during every visit, while at others, the directing physician will recommend that individuals see some specialists more or less frequently.

For example, at the MDA Care Center at Johns Hopkins University in Baltimore, Md.,



## THE IMPORTANCE OF SOCIAL WORKERS

For individuals and families living with neuromuscular diseases, having access to the right support is paramount to living life to the fullest. But so is everyday help, and that’s where an MDA Care Center social worker comes in. A social worker is a counselor, advocate, educator, facilitator, resource specialist and problem-solver all rolled into one.

Hillary Zebberman is a licensed clinical social worker at the MDA Care Center at University of California, Los Angeles (UCLA). “I’m not a therapist, but I can identify problems and direct parents where to go to get help,” she says.

Zebberman looks at the overall health and stability of children and their families as it pertains to their finances, social and emotional health, and education. She works with each individual and their family to come up with resources to make life easier, such as information about adaptive equipment, financial aid and community resources. She also provides education to schools about neuromuscular diseases to ensure students have access to the accommodations they need to be successful.

“There are so many things that social workers can do to add a dimension of support,” she says.



the disciplines represented on its multidisciplinary care team include neurology, pulmonology, cardiology, physical therapy, occupational therapy, genetic counseling and orthotics. MDA Care Center Director Thomas Crawford, M.D., a neurologist, says his approach is to refer patients to specialists when their needs in a particular area extend beyond the basics.

“When you go to see a lot of specialists, it can be kind of overwhelming,” Dr. Crawford says. “I like the model of having a person like myself who thinks about all things related to muscular dystrophy and spinal muscular atrophy, so when you come and see me, we are going to cover all of those territories. If we need to spend extra time on a particular domain, I’m going to get a specialist who knows even more about that domain. In that way, we don’t waste time.”

## COMMUNICATION IS KEY

Regardless of how they are structured, the most successful multidisciplinary teams prioritize good communication, and they are able to provide a comprehensive and coordinated care plan because they have spent time thinking about what it means to work as a team, what it takes to provide good care and how to deliver care effectively.

The multidisciplinary team at Nemours is a good example of this. “Not only are all of Brendan’s doctors under one roof, but they share everything — their notes, their assessments, what medications they put him on,” Labbadia says. “Everyone is on the same page.”



## BENEFITS OF MULTIDISCIPLINARY CARE

When everyone is working together toward a patient-centered care plan, the benefits for individuals and families are far-reaching. Research has shown that multidisciplinary care can have a positive impact on those with neuromuscular disease in a number of ways.

### 1. Quality of life

Jerry Creehan, a 65-year-old retired medical equipment salesman and father of three, can attest to the impact of multidisciplinary care. After being diagnosed with ALS in January 2017, he started visiting the MDA ALS Care Center at Virginia Commonwealth University Medical Center in Richmond, Va. Creehan’s multidisciplinary care team includes a neurologist, respiratory therapist, physical therapist, registered dietician, social worker, research coordinator and MDA family care specialist.

From his very first appointment, Creehan knew

*“I have been in and around hospitals all my career, but I have never experienced that level of care in a clinical setting.”*  
— Jerry Creehan

he had something special. Not only was he impressed with the amount of time his neurologist and other care providers spent with him, but he was amazed at the level of compassion, care and respect each one showed.

“I have been in and around hospitals all my career, but I have never experienced that level of care in a clinical setting,” he says. “I had been told that being seen by a multidisciplinary care team can add length to life for an ALS patient. I knew right then and there why. It didn’t mean that they were going to catch me in some crucial stage and intervene medically, but all

## Expert Care from Day One

We know early diagnosis, highly specialized care and access to promising clinical trials help ensure the best possible outcomes for individuals and families living with muscular dystrophy, ALS and related neuromuscular diseases. MDA has more than 150 Care Centers across the United States and in Puerto Rico located at top hospitals and health care facilities. Go to [mda.org](http://mda.org) and type in your state or ZIP code in the “Find MDA in Your Community” box to find an MDA Care Center near you. You also can contact the MDA Resource Center at **800-572-1717** or [resourcecenter@mdausa.org](mailto:resourcecenter@mdausa.org) for assistance with locating an MDA Care Center in your area.

## SPECIALISTS AT A GLANCE

How is a pulmonologist different from a cardiologist? It can be confusing. Here is a guide to some of the specialists you are likely to meet at your MDA Care Center.



### **Neurologist or Physiatrist**

Most MDA Care Centers are directed by a neurologist or physiatrist. These physicians specialize in diagnosing and treating neuromuscular diseases.

### **Social Worker**

Social workers help with the practical aspects of illness and disability (insurance reimbursement, financial aid, equipment and housing needs, transportation, home care, community resources, etc.). They also can help families cope with diagnosis and disease progression.



### **Physical Therapist**

Physical therapists identify muscle weakness and joint tightness and develop treatment plans to maintain and improve muscle strength and range of motion. They also can provide adaptive equipment recommendations.



### **Care Center Coordinator**

These coordinators may be nurses, nurse practitioners, genetic counselors or other providers. They offer families support and information and can help connect people to community resources that offer additional assistance.



### **Cardiologist**

Cardiologists specialize in the heart and cardiovascular system. They monitor heart health and function and treat heart conditions that occur as a result of neuromuscular disease.



### **MDA Family Care Specialist**

MDA's Family Care Specialists attend MDA Care Center sessions to represent MDA and serve as a resource to families receiving care there.



### **Occupational Therapist**

Occupational therapists provide strategies and tools to help people accomplish daily activities in their home and work environments.



### **Pulmonologist**

Pulmonologists specialize in disorders of the lungs and respiratory muscles. These doctors typically work with respiratory therapists to monitor lung and respiratory health and recommend breathing exercises and breathing aids, when needed.



that love and care makes me want to come back.”

### **2. Proactive care**

When clinicians work as a team, they are able to take a more proactive approach to care. This type of teamwork helped 75-year-old Patricia

Mishler optimize her communication strategies before she lost the ability to speak as a result of ALS.

Mishler was diagnosed with ALS three years ago. Her neurologist at the MDA ALS Care Center at Vanderbilt University Medical Center

in Nashville referred her to Sarah Whyte, a certified and licensed speech-language pathologist. Whyte told her about voice banking, a process where an individual records their speech to be used as a personal synthetic voice. Mishler's speech was intact at the time, so she and Whyte decided to act quickly to take advantage of the timing and the technology.

“Because I saw her very early in her disease progression, we were able to bank her voice, which she now uses with an eyegaze device,” Whyte says.

Patricia uses her eyegaze device to generate speech and operate her computer with her eyes. “My MDA Care Center is always ready to help me improve the quality of my life,” she says via email.

### **3. Streamlined care**

Having multiple experts observe an individual's movements and functions during a single visit increases a clinician's ability to recommend and implement therapies and adaptive equipment.

Lynn Lukins, a licensed physical therapist at the Kids Center for Pediatric Therapies in Louisville, Ky., works as part of the pediatric MDA Care Center at the University of Louisville. The therapists there use a team approach to assess the needs of the children and families they serve.

“We all observe the child during each screening, so while the speech therapist is watching swallowing, breathing and vocalizations, I can be looking at posture, weight shifting and preferred position for play, and the OT can be

Patricia Mishler with her husband, Roger



watching fine motor activities,” she says. “We are then able to more effectively and quickly provide evaluations and recommendations to the physician and family.”

#### 4. Practical efficiency

Among the advantages to being seen by a multidisciplinary care team, the most impactful might be reducing

the travel burden associated with seeing multiple specialists. “Most of the people in our community have mobility issues, and transportation can be challenging,” says Theresa Cox, clinic coordinator at the MDA Care Center at Johns Hopkins. “Being able to come to the clinic and see all of your providers on the same day is really helpful.”

To ensure the necessary services are available during each visit, Cox coordinates appointments for each individual. “It can take me a couple of months to coordinate just one of our multidisciplinary clinics because we tailor each visit to meet the patient’s needs,” she says.

Labbadia says that kind of care coordination is well worth the wait. “Getting all of these amazing doctors together talking about specialty care for your child is wonderful. That kind of expert care allows us a sigh of relief because we are not left hanging when issues come up. It’s done right then and there.”

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

*“My MDA Care Center is always ready to help me improve the quality of my life.”*

*– Patricia Mishler*

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Jared Aronson

# creative CONTROL

BY DONNA SHRYER

*Jared Aronson embraces life with imagination and laughter*

“I believe that if you are laughing, you are living.”

This is 34-year-old Jared Aronson’s mantra. And he’s not particular about what deserves a good guffaw.

He’ll laugh out loud over a clever play on words, a ridiculous Twitter post or even a temporary misfire as he masterminds yet another invention. Inventing is what fuels Aronson’s mojo. “I love problem-solving,” he says.

Aronson’s life situation often sparks his inventive imagination. He has Duchenne muscular dystrophy (DMD), has been using a wheelchair since age 6, has been ventilated since age 18, and he can no longer move his limbs or digits. But that’s all he cares to share about his disease. It’s a reality he lives with, not what defines him. “Just because you have a disability doesn’t mean you don’t have creativity and passion,” Aronson emphasizes.

And passion he has. When he talks about his creative endeavors, an effervescent fervor bubbles up like a fine glass of champagne. In Aronson’s case, it’s a fitting analogy. There’s so much to toast!

## TEeing UP

To begin at the beginning, Aronson is an artist. He’s always been drawn to drawing. So, in 2004, he blended his love of sketching with his entrepreneurial spirit and opened the virtual door to



*A Madhouse Tees design*

# 8 INNOVATIVE ASSISTIVE TECHNOLOGY PRODUCTS

Today's technological advances don't just empower solo inventors like Jared Aronson – they also have led to some seriously life-enhancing assistive technology (AT) products. AT products, which include all manner of assistive, adaptive and rehabilitative devices, share one goal: enabling individuals with disabilities to perform tasks that were formerly beyond their physical capabilities.

**Here's a roundup of some of the most innovative AT products on the market:**



**1. GlassOuse.** These smart eyeglasses allow hands-free control of Bluetooth-enabled devices. The cursor on the device screen follows the user's head movements. The mouse is activated by biting or pressing the "click" button. ([glassouse.com](http://glassouse.com))



**2. HouseMate.** This petite device enables users to control their environment and operate appliances with a smartphone. It works with WiFi, Bluetooth, infrared and z-wave connections. Users can operate the system with switches, joystick or voice. ([touchthefuture.us/product/housemate-pro-z-for-android](http://touchthefuture.us/product/housemate-pro-z-for-android))

**3. Jamboxx Pro.** This harmonica-style electronic instrument connects to a computer and is activated with side-to-side head motions. The device comes with its own software, a range of sounds and backing tracks. It also integrates with Apple's GarageBand. ([jamboxx.com](http://jamboxx.com))

**4. LipStick Mouse.** This computer mouse works with just the slightest lip movement. Because no sip-and-puff switch is required, the LipStick also can be manipulated with thumb and forefinger. ([touchthefuture.us/product/lipstick](http://touchthefuture.us/product/lipstick))

**5. LUCY4.** This hands-free keyboard is operated by a small, lightweight laser pointer that clips to a head-band or eyeglasses. Slight head movements (only a couple of centimeters) activate the keys on the keyboard. It can also be controlled by joystick, mouse or switch. ([touchthefuture.us/product/lucy4](http://touchthefuture.us/product/lucy4))



**6. Magic Flute.** The Magic Flute is a hands-free electronic wind instrument that enables an individual to play music with small head movements and breaths. Breath strength can be adjusted to allow vent users to play, and switch control can be used for functions. ([touchthefuture.us/product/magic-flute](http://touchthefuture.us/product/magic-flute))

**7. Neuronode.** Developed by Control Bionics, the Neuronode is a wearable device that uses the body's bioelectric electromyographic (EMG) signals to control a computer. The NeuroNode wireless sensor is placed on the skin over the muscle chosen to act as the switch. When the user attempts to move that muscle, NeuroNode detects EMG signals, even if there is no visible muscle movement. ([controlbionics.com/neuronode](http://controlbionics.com/neuronode))

**8. QuadJoy.** This advanced sip-and-puff mouse provides computer access and functionality via head motions, inhalations and exhalations. Features include 100 percent plug-and-play USB, Bluetooth connectivity and programmable capabilities that allow a user to personalize functions. ([enablemart.com/quadjoy-3-usb-and-bluetooth](http://enablemart.com/quadjoy-3-usb-and-bluetooth))



Madhouse Teeshirt Designs LLC ([madhousetees.com](http://madhousetees.com)), an online store that sells screen-printed T-shirts with his original artwork.

Originally, Aronson generated his drawings with pen and ink. Then he moved onscreen, using Adobe Photoshop and Illustrator. However, as his muscle strength diminished, he lost the ability

to maneuver the computer mouse. Give up? Dissolve Madhouse Tees? Absolutely not. This was the sort of challenge that triggers Aronson's problem-solving skills.

"I needed to come up with a way to draw," Aronson recalls. First, he taught himself how to program Arduino microcontrollers, an open-source electronics

platform and the software used to program it. Arduinos are designed to make electronics more accessible to artists, designers, hobbyists and anyone interested in creating interactive objects or environments.

"After messing around for a while, I figured out the programs so I could control my computer and develop

my own sip-and-puff mouse," Aronson says. "I created the device for about \$50, and it works great." (Retail prices for sip-and-puff devices generally range from \$200–\$800.)

## DRIVEN TO SUCCEED

With Madhouse Tees under control, Aronson needed a new challenge. Enter Rhino Buttons, another assistive device he designed out of necessity.

"I used to drive my chair with a joystick, but when I got too weak for that, I changed to another driving system using a switch," Aronson explains. "After a while, I realized that these \$85 switches were pretty high maintenance. If I didn't clean the buttons often and carefully, they stuck. So, I'd be driving around, the switch would stick, and I'd hit a wall — or whatever else was in the way. I thought it was a riot."

But even while he was laughing, the wheels in Aronson's brain were turning. An out-of-control wheelchair makes for funny antics, but it was also dangerous. He set out to design a safer, more reliable switch.

To start the creative process, Aronson purchased a 3D printer to produce various button prototypes. Using his knowledge of electronics, he experimented with different designs and electronic components. His design needed to replicate the feather-light sensitivity of the switches already on the market but without the sticking issue. After two years of trial-and-error, he finally had a product that solved the sticking problem.

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Read "A World of Opportunities," a Quest online exclusive article, to learn how other MDA families are using innovative do-it-yourself adaptive equipment solutions to improve everyday life. Find it at [mda.org/quest](http://mda.org/quest).

It was time to go public. And with that, Aronson opened his second online store, **rhinobuttons.com**, where shoppers and retailers can purchase his uniquely designed switches.

To make his product accessible, Aronson priced his switches below his competitors. "I want everyone who needs this switch to be able to afford it," he says.

**FUELING THE SPARK**

While the need to invent "a better mouse trap" typically ignites Aronson's drive to create, he's quick to share credit with a few key influencers. To begin with, there's his father, who often steps up to literally assemble what Aronson sees in his mind's eye. Then there's his whole family, which includes his parents, five

siblings and too many dogs to count. "I grew up in a busy house, and I was never treated differently than anyone else. I never thought of myself as the 'disabled one.' That had a big impact on me; I think it's why I'm so motivated. If I had an idea, everyone in my family would say, 'Just do it.'"

Aronson also draws inspiration from members of the Maker Movement, a trend in which individuals or groups create technology products using upcycled materials from computers, game consoles or other electronic devices. Ben Heck, an internet celebrity among the Maker community, is one of several Makers that Aronson follows closely. Every Friday, he clicks into YouTube and watches "The Ben Heck Show," where Heck presents a new project.

Although Heck's show covers a wide range of builds, he occasionally focuses on projects for individuals with disabilities. In one such episode, Heck built a power wheelchair with foot controls. "A viewer wrote to Heck because he needed a hands-free wheelchair, so he could move around and hold his new baby," Aronson recalls. "Heck is a genius. He's amazing."

So what equally amazing invention is next on Aronson's list of things to do? "I just bought 3D animation software. I've been told I have a pretty good sense of humor, so I'm going to write these scripts for animated characters and create little vignettes. It's my new adventure. Let's see where it takes me." **Q**

*Donna Shryer is a freelance writer in Chicago.*

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# The first and only treatment for children and adults with spinal muscular atrophy (SMA)<sup>1</sup>

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

### INDICATION

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

### IMPORTANT SAFETY INFORMATION

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

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

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

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

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

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



## IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

### USES

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

### WARNINGS

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

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

### COMMON SIDE EFFECTS

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

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

### OTHER INFORMATION

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

### QUESTIONS?

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

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# meet

## MDA's New President & CEO

*Six Questions with  
Lynn O'Connor Vos*



Global health care leader Lynn O'Connor Vos joined the Muscular Dystrophy Association last fall as its new president and CEO. With a clinical nursing background and decades of experience building and leading health companies to drive technology and innovation in patient care, Lynn says her entire career has led to this unprecedented moment with MDA.



Lynn is pictured with Pat Quinn (seated), co-founder of the Ice Bucket Challenge, and Mark Longmore (Pat's personal care attendant).

Lynn spent the past 23 years as CEO at Greyhealth Group (ghg), a leading health care communications agency where she forged relationships and partnerships with institutions, providers and nonprofits to effect change and make a lasting impact in health care.

At MDA, Lynn will continue all the great work the organization is known for — like funding new and innovative research; partnering with the nation's top medical institutions to advance care and drive more clinical trials for MDA families; facilitating knowledge-sharing and best practices for health care experts; and providing day-to-day help and resources for individuals and families living with neuromuscular diseases. She will expand that work by growing relationships with influential partners and helping drive innovation and technology to transform and save more lives.

Lynn recently sat down to answer questions submitted from *Quest* readers and families as she takes on this new role.

### **Why did you choose to come to MDA?**

When I learned about the opportunity to lead MDA, it felt like my entire career was coming full circle. I have decades of experience in the commercial and nonprofit sectors transforming health care organizations and driving lasting change, but I started my career as a pediatric nurse. Focusing on patient and family care at MDA feels like returning to my roots.

With tremendous progress underway as an unprecedented number of new therapies and drugs to treat neuromuscular diseases are becoming available, I believe my deep understanding of the drug approval process and the way to work with industry partners, clinicians, researchers and providers will help break new ground in neuromuscular disease research and care. I came to MDA to help improve the lives of more families and transform their health care experience. I'm looking forward to partnering with our community to make that happen.

### **How do you plan to get to know MDA families?**

MDA families are at the heart of what makes this organization so special. I've had the distinct fortune of meeting many families already at local and regional MDA events, and my goal is to continue to listen and learn from our families as much as possible. I will be spending time with

MDA's National Community Advisory Committee this year and families at our upcoming conferences and events throughout 2018. I'm also excited to attend several MDA Summer Camps, which I understand are the best week of the year.

In addition to my time with families, MDA just completed a survey of thousands of individuals living with or caring for someone with a neuromuscular disease to better understand needs, gaps in care, challenges and opportunities to pave the way for how MDA can truly facilitate change for our families. I'm looking forward to sharing the findings with you and what it means for new and enhanced programs at MDA later this spring.

### **After four months on the job, what do you see as the most exciting or promising opportunities for MDA and our community?**

My first priority since joining MDA has been to listen, learn and become immersed in all areas and levels of our organization. I've spent a lot of time with MDA staff, volunteers, neuromuscular disease experts, industry and corporate partners — and of course, families — to determine where we can make the greatest impact for the MDA community. From day one, I've found myself having many "ah-ha" moments about the brightness of our future. We have so many possibilities in new therapies, growing knowledge in genetics, opportunities to apply innovation in care and the ability to bring the same boldness that once



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Visit [TakeabreathDMD.com](http://TakeabreathDMD.com) for respiratory information  
and to sign up for news updates.

paved the way with our telethon to engage and activate our community and sponsors in new ways.

**What is your vision for 2018? What new things can we expect from MDA moving forward?**

I believe the future is about defying limits, just as MDA families do every day. MDA families, disease experts and supporters will be critical in helping us map the road ahead. Progress is our promise, and we are working on a bold plan and critical focus areas to deliver on our mission. My vision is that MDA will continue to be the world leader in muscle research and the pre-eminent provider of resources and support to families. Here are a few things I hope to accomplish in the

next year and beyond:

- Expanded relationships with our incredible partners and new partnerships in the biotech and pharma industry that speed better care and new drugs to market
- A better health care experience for families, particularly as millennial moms become the new chief health officers of their families and individuals are needing and expecting a more accessible and integrated health care experience
- Increased access to the best information and resources when and where people need it — we can do much better than we are today
- New technology across all we do for patients, families and health care providers
- Innovative ways to elevate our cause and family stories to motivate a new

generation of supporters, just as we disrupted the market with our telethon more than 50 years ago.

**We've heard you have deep experience and passion in technology. How do you see technology impacting MDA's work and improving care and support for MDA families?**

Technology is going to have a big role in health care and will be the muscle behind MDA going forward. I am a huge champion of using digital strategies to improve public health. I helped create Text4baby, a mobile platform in which informative and health-related texts are regularly sent to expectant and new mothers, which resulted in improved health outcomes for mothers and infants. Those kinds of technologies that help both individuals and health care providers improve the health care experience and outcomes are what I plan to bring to the forefront at MDA.

**What's your favorite thing to do when you're not working as MDA's CEO?**

I love tennis and play every week. I enjoy watching the sport and have attended all the majors. In addition, I love to travel and spend time at home with my husband and family. [Q](#)

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*To connect with MDA President and CEO Lynn O'Connor Vos, follow her on Twitter ([@lynnvos](#)), read her regular posts on MDA's blog [Strongly \(strongly.mda.org\)](#) and sign up to receive email from MDA ([mda.org/email](#)).*

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8 AM - 8 PM EST Monday - Friday  
to speak with an  
EMFLAZACares case manager.

EMFLAZA is indicated for the treatment of Duchenne muscular dystrophy in patients 5 years of age and older.

Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

Please see the Important Safety Information on the next page.



6 mg | 18 mg | 30 mg | 36 mg tablets  
22.75 mg/mL oral suspension



## INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA™ (deflazacort)

### INDICATION

EMFLAZA™ is indicated for the treatment of Duchenne muscular dystrophy in patients 5 years of age and older.

### IMPORTANT SAFETY INFORMATION

**Contraindication:** Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

**Do not stop taking EMFLAZA, or change the amount you are taking,** without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid “withdrawal syndrome”. Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal. A steroid “withdrawal syndrome,” seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.

- **Hyperglycemia:** Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.
- **Increased Risk of Infection:** Tell your healthcare provider if you have had recent or ongoing infections or if you have recently received a vaccine or are scheduled for a vaccination. Seek medical advice at once should you develop fever or other signs of infection, as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.
- **Alterations in Cardiovascular/Kidney Function:** EMFLAZA can cause an increase in blood pressure, salt and water retention, or a decrease in your potassium and calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.
- **Behavioral and Mood Disturbances:** There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.
- **Effects on Bones:** There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.
- **Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.
- **Ophthalmic Effects:** EMFLAZA may cause cataracts or glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.
- **Vaccination:** The administration of live or live attenuated vaccines is not recommended. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.
- **Serious Skin Rashes:** Seek medical attention at the first sign of a rash.
- **Drug Interactions:** Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medicines you are taking, including over-the-counter medicines (such as insulin, aspirin or other NSAIDS), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during the treatment.

**Common side effects that could occur with EMFLAZA include:** Facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds.

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

For medical information, product complaints, or to report an adverse event, please call **1-866-562-4620** or email at [usmedinfo@ptcbio.com](mailto:usmedinfo@ptcbio.com).

You may also report adverse events directly to FDA at **1-800-FDA-1088** or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

# **EMFLAZA™ (deflazacort)**

## **Consumer Brief Summary of the FDA-Approved Product Information Initial US Approval: 2017**

### **What is EMFLAZA?**

EMFLAZA™ is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 5 years of age and older.

### **When should I not use EMFLAZA?**

- Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA

### **What should I tell my healthcare provider before taking EMFLAZA?**

It is important to tell your healthcare provider if you have had recent or ongoing infections, develop a fever, have recently received a vaccine or are scheduled for a vaccination, or experience any other side effects.

### **What warnings should I know about EMFLAZA?**

**Do not stop taking EMFLAZA, or change the amount you are taking**, without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid “withdrawal syndrome”. Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal. A steroid “withdrawal syndrome”, seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids.

For patients already taking corticosteroids during times of medical stress, the dosage may need to be increased.

**Cushing’s Syndrome:** Cushing’s syndrome occurs with prolonged exposure to exogenous corticosteroids, including EMFLAZA. Symptoms include high blood pressure, truncal obesity and thinning of the limbs, purple striae, facial rounding, facial plethora, muscle weakness, easy and frequent bruising with thin fragile skin, posterior neck fat deposition, osteopenia, acne, amenorrhea, hirsutism, and psychiatric abnormalities.

**Hyperglycemia:** Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.

**Increased Risk of Infection:** Medical advice should be sought immediately if you develop a fever or other signs of infection as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.

### **Alteration in Cardiovascular/Kidney Function:**

EMFLAZA can cause an increase in blood pressure and water retention or a decrease in your potassium or calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.

**Behavioral and Mood Disturbances:** There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.

**Effects on Bones:** There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.

**Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.

**Ophthalmic Effects:** EMFLAZA may cause cataracts or glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.

**Vaccination:** The administration of live or live attenuated vaccines is not recommended. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.

**Serious Skin Rashes:** Seek medical attention at the first sign of a rash.

### **What are the side effects that could occur with EMFLAZA?**

- facial puffiness or Cushingoid appearance
- weight increased
- increased appetite
- upper respiratory tract infection
- cough
- frequent daytime urination
- unwanted hair growth
- central obesity
- colds

### **What other medications might interact with EMFLAZA?**

Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medication you are taking, including over-the-counter medicines (such as insulin, aspirin, or other NSAIDs), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during treatment. Do not take EMFLAZA suspension with grapefruit juice.

**The information presented is not comprehensive. Talk to your healthcare provider for more information or see [www.EMFLAZA.com](http://www.EMFLAZA.com) for the full FDA-approved product information.**

For medical information, product complaints, or to report an adverse event, please call **1-866-562-4620** or email at [usmedinfo@ptcbio.com](mailto:usmedinfo@ptcbio.com).

You may also report adverse events directly to FDA at **1-800-FDA-1088** or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).



# access MDA

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

Volunteer Whitney Jorgensen dedicates time to MDA in many ways, including as an MDA Summer Camp counselor.

## From Summer Camp to Shamrocks

MDA Ambassador's MDA Summer Camp experience inspires her work with the Shamrocks campaign

When Whitney Jorgensen, a 25-year-old MDA Shambassador from Farmington, Utah, visits stores in her area that participate in the MDA Shamrocks campaign, she knows firsthand how much the fundraising program does for individuals and families in the MDA community.

"It's a really good experience to be able to go to these stores and talk to cashiers and

managers and be a face of the program and explain that this money is helping kids that I know," she says.

Jorgensen, who works for University of Utah Health, has volunteered as a counselor at MDA Summer Camp every year since 2011. Her cousin, whose father has muscular dystrophy, introduced her to MDA, and Jorgensen, her sister and her cousin have all been Summer Camp volunteers. In Jorgensen's seven years of volunteering, she has seen the impact Summer Camp has on the campers. One of her favorite memories is watching

a camper ride a horse for the first time – something he had wanted to do his whole life.

"That memory leads to all of the other experiences, of making things possible," says Jorgensen. "Nothing is impossible at Summer Camp."

Jorgensen explains that sharing these experiences with the employees she visits at Shamrocks-participating businesses helps put their fundraising efforts into perspective.

"I see the work that the employees put into the program, and I can show them where the money is going and what their efforts are providing

for these kids," she says. "I love when cashiers reach their goals, and we get to celebrate that together. It's a win for all of us. Those connections are what keep me going with MDA Shamrocks."

 **For the past 35 years, MDA has partnered with more than 25,000 retail locations to raise more than \$300 million through the MDA Shamrocks program. Look for participating partners in your community this MDA Shamrocks season, and visit [mda.org/shamrocks](http://mda.org/shamrocks) to learn how you can get involved.**



# Having a Ball

## Bowlers raise money for families at the Oshkosh MDA Bowl-A-Thon

Eric Salzwedel, a 29-year-old marketing director for a Madison, Wis., nonprofit, became an MDA Summer Camp counselor by chance. When he was a senior in high school, he saw a flyer about Summer Camp in his guidance counselor's office, and it caught his interest. His first day of camp coincided with his high school graduation, so after walking off the stage with his diploma, he jumped into his parents' car and headed to camp.

That summer opened his eyes. "When I had a chance to do things with the campers, I saw that they're just like anybody else: They want to have fun, do projects, play games and crack jokes," says Salzwedel. "I saw the impact [camp] had on these kids, but I don't think they realized the impact it had on me."

He volunteered again after his freshman year at the University of Wisconsin-Oshkosh, but in 2008, after his sophomore year, he wasn't able to attend because of work. "But I knew I could make a difference," he says. "I love bowling, and I thought, 'I could do a bowl-a-thon.'"

That year, he planned and hosted the first annual Oshkosh MDA Bowl-A-Thon. Salzwedel gathered his friends and fellow students and filled about 10 lanes with bowlers, raising \$938 for MDA. Since then, the Bowl-A-Thon has exploded, more than doubling its fundraising in the second year and reaching about \$22,000 in 2017. Salzwedel estimates that they've had close to 200 bowlers each of the last few years, making it the largest bowl-a-thon in Wisconsin. And, in total, the Bowl-A-Thon has raised more than \$117,000 for MDA research and support programs.

"When I started this, being involved at UW-Oshkosh, I had lots of friends who helped spread the word to get their groups to come out and bowl," he says. "It has grown tremendously since then. We have people come from different states, and for some, it's almost like an alumni event."



An Oshkosh MDA Bowl-A-Thon team shows their team spirit.

Now, the event includes a pre-celebration at a local bar the night before, a raffle during the event that raises more money for MDA, and multiple sponsorships. Local MDA families also join in on the fun, and Salzwedel hosts a dinner for them after the event. Now that he lives 90 miles north of Oshkosh in Madison, planning the event isn't easy, but his passion for MDA and fundraising keeps it going.

"I try to drive up once a month to meet with our planning committee to make the Bowl-A-Thon as successful as it can be," says Salzwedel. "It's something I love, and to know that after 10 years we've raised more than \$100,000, with literally blood, sweat and tears going into it, it's a great feeling."



**From hosting a bowl-a-thon to planning a bake sale, you can fundraise your way to help kids and adults living with muscular dystrophy. We'll give you the tools to make the fundraiser a success and cheer you on every step of the way. Learn more at [mda.org/yourway](http://mda.org/yourway).**



Will Farr and his running buddy, Owen

# Pushing the Limit

## MDA Team Momentum runner pushes his son across the finish line at the Marine Corps Marathon

After running three marathons with MDA Team Momentum since 2014, Will Farr was ready for a bigger challenge.

Farr had previously pushed his 14-year-old son, Owen, who has Duchenne muscular dystrophy (DMD), in a racing chair for a 4-mile race as part of a charitable initiative of the men's workout group F3 Nation. In a video made to promote the initiative, Owen was asked if he thought his dad would push him in a marathon. ➤



# 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?

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



## Will my information be private?

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



**YOU JOINING**



More information for research

A better understanding of ALS

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

Of course, Owen's answer was "Yes," and Farr decided to prove Owen right. In October, at the 2017 Marine Corps Marathon in Washington, D.C., Farr pushed Owen for the entire

26.2-mile race.

"It was really exciting," Farr says.

"For one, there were a whole lot of other people pushing chairs, and it was fun to be around them on the race course. We kind of stood out and you get a lot of crowd support. But also, it was fun being

there with Owen, waving to people we knew. It was really a good time for us to be together."

While pushing Owen through the marathon was tough work, Farr says seeing the big crowds cheering for him and his son kept him going.


"It was fun. I liked seeing the people cheering for me," says Owen.

Beyond taking in the enormous amount of crowd support, Farr also had a lot of support from his friends and family, who donated to his run. In this race, he raised more than \$5,300 for MDA Team Momentum. Combined with his previous races, this brought his total to more than \$26,000 raised to support MDA's mission.

## THE RACE IS ON

Register today at [mdateam.org](http://mdateam.org) for one of these upcoming races:

- NYRR United Airlines NYC Half, New York, March 18, 2018
- Reebok Ragnar So Cal, Huntington Beach, Calif., April 6-7, 2018
- Chicago Spring Half Marathon/10K, May 20, 2018
- Boston's Run to Remember, May 27, 2018
- Bank of America Chicago Marathon, October 7, 2018

 **With MDA Team Momentum, the miles you run can raise awareness of MDA and raise funds to further research breakthroughs and provide health care and everyday support for kids and adults in your hometown. Registrations are now live for 2018 events, and a limited number of entries are available. Visit [mdateam.org](http://mdateam.org) to register.**



Rene Runions

## Walking Strong

**A family connects through MDA Muscle Walk**

For Rene Runions, a 19-year-old sophomore at Saint Louis University (SLU), MDA has been a part of her life since she was diagnosed with Charcot-Marie-Tooth disease (CMT) in

the sixth grade. She started attending MDA Summer Camp in her home state of Illinois and later was named the Illinois State Ambassador.

"It was cool," she says. "I went around Illinois and spoke at different conventions. I talked with fire fighters, I went to Muscle Walks, and I went to businesses like Jiffy Lube and Lowe's and thanked people [for their support]."

MDA Muscle Walk is also an important event for Runions and her family. She estimates that about a third of the family on her mother's side has muscular dystrophy, including her mother, grandmother and likely her brother. Since her diagnosis, the family has walked as Team Run Rene at the MDA Muscle Walk of Springfield. In those nine years, they've only missed one walk, and they have raised more than \$10,000 for MDA families.

Runions also attended the MDA Muscle Walk of St. Louis last year with a classmate who has muscular dystrophy. At SLU, she is studying nutrition

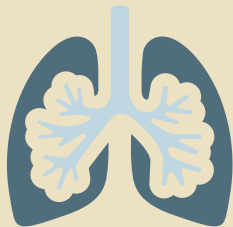
and dietetics, and she plans to become a doctor.

"In high school, I started wanting to be a pediatric neurologist, because I know from experience that there aren't that many," says Runions. "That was a motivator, and in my senior year [of high school] I took human anatomy, and neurology was the most fascinating part of that."

Runions still makes it back home for her local Muscle Walk of Springfield, which she enjoys because it gives her a chance to reunite with friends she made at MDA Summer Camp.

"It's always great to go to the MDA walks, because I get to see everybody from camp," she says. "A lot of campers feel like they're at home when they're at Summer Camp – we could just be kids, and if you wanted to do something, you could. That's what I remember most."

 **MDA Muscle Walks are happening across the country throughout the year. To find one near you and get involved, visit [mdamusclewalk.org](http://mdamusclewalk.org).**



# A chance to **advance DMD therapy**

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

## The *SIDEROS* study

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

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



## The therapy

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

## Who can participate?

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

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





Allie Williams (middle, in purple shirt) helped to build this inclusive playground.

## Designing Her Dreams

**Former MDA Summer Camper designs accessible spaces and devices**

Allie Williams, a 25-year-old who lives with Charcot-Marie-Tooth (CMT) disease, earned her master’s degree in biomedical engineering from Texas A&M University in 2017. Williams always knew she wanted to help individuals with disabilities, but it wasn’t until her junior year of high school that she found the right fit.

“At first, I didn’t even know biomedical engineering was a thing,” she says. “I really liked science and math, and I thought about being a doctor, but I realized quickly that I don’t like blood or anything like that. I was looking for how I could help people with disabilities, and I came across biomedical engineering.”

Her first inspiration to help people with disabilities through engineering came from her experience at MDA Summer Camp.

“I started going to camp when I was 8, so seeing myself and all the other kids getting to do what they wanted to do – there were

literally no limits,” Williams says. “That’s why I went into biomedical engineering. We always say ‘Camp is the best week of the whole year,’ and I want to make it like that for us all of the time.”

Williams studied biological engineering at Louisiana State University (LSU) for her undergraduate degree, where she was first introduced to the ways her work could help people with disabilities. As part of her first class in the subject at LSU, Williams and her classmates met with local representatives from area schools and designed accessible playgrounds. After the class ended, Williams’ professor hired her to work on the same project and get the playgrounds built.

“We took the comprehensive designs from the classes, and we wrote grant proposals so we could build the playgrounds,” says Williams. “That was where community engineering came in, making sure it’s an inclusive environment. That’s really my dream, working on design, whether it’s a medical device or an inclusive or universally designed playground, that will improve the quality of life for those living with neuromuscular diseases.”

Williams hopes to start a nonprofit organization to design and build inclusive playgrounds someday. But for right now, she is focusing on her job as a research and development engineer at Exothermix in College Station, Texas. In this role, she uses technology to solve problems such as reducing contamination of stem cells used for stem cell therapy. When she’s not working or visiting her hometown of Baton Rouge, La., Williams likes to spend time enjoying the natural beauty of Texas.

“I got a dog when I moved here, so we go to the dog park every day,” she says. “And we go on walks. There are a lot of nature trails and things like that here, so we like to hang outside.”



Visit [mda.org/young-adults](http://mda.org/young-adults) to explore MDA’s young adult resources, covering everything from education and employment to independent living.

## Productive Partnership

**Acosta Sales & Marketing offices find unique ways to support MDA families**

Since first partnering with MDA in 1985, Acosta, a full-service sales and marketing company focused on packaged consumer goods, has raised more than \$82 million for MDA’s mission.

Acosta’s staff support MDA through fundraising events, marketing promotions and even

volunteering at MDA Summer Camps. Here are just some of the ways Acosta offices across the country have showed their support for MDA:

### Golfing and giving

Every year, Acosta’s Marlborough, Mass., office hosts a golf

*continues on page 48*



Marlborough, Mass., Acosta employees with former MDA State Ambassador Emma Russell (center)





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October 26-28, 2018



Eden Prairie, Minn., Acosta employees at MDA Summer Camp

MDA, with more than \$150,000 raised at the 2017 event.

“MDA is a part of our heritage,” says John Bierfeldt, senior vice president for Acosta’s East Region. “The folks in our company who have grown up at Acosta know how important MDA is to us, and I feel really good about the work our team has done.”

**Catering for camp**

Over the years, employees at Acosta’s Eden Prairie, Minn., office have volunteered at their local MDA Summer Camp. For the past two years, the office has gone a step further by sponsoring a lunch for the campers and volunteers, in addition to

tournament at The International Golf Resort in Bolton, Mass. The event brings together Acosta client partners and retailers for a day of golf and dining, ending with a silent auction. Over the last 17 years, the tournament has raised more than \$2.5 million for

bringing fun activities, including a dunk tank.

“The dunk tank was an absolute hit,” says Ryan Tesdall, director of the Eden Prairie office. “I got dunked quite a few times.”

In addition, Acosta’s Eden Prairie office raises money for MDA through a corporate shopper program with their partner SuperValu. Since 2014, this program has raised more than \$850,000, including their largest-ever yearly donation of \$300,000 in 2017.

**Putting the ‘fun’ in fundraiser**

At Acosta’s Salt Lake City office, employees get together multiple times a year for fun, seasonal fundraising events that support MDA. From Mardi Gras parties to Easter egg hunts, the office is in the giving spirit year-round.

“We’re a smaller office, but we have a big heart,” says Kristen Albrecht, branch manager. “[These events] started because MDA was an important part of our corporate culture, but the more we got involved with our local MDA office and got to know the people, we found it was a really fulfilling thing to be involved in.”

The Salt Lake City office has raised about \$2,000 a year with these events. In addition to the monetary donations, employees from the office volunteer at MDA Summer Camp.

 **To learn how you can support Acosta and all MDA partners, connect with your local office or visit [mda.org/get-involved/meet-our-partners](http://mda.org/get-involved/meet-our-partners).**

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# The Power of Role Models

*Seeing how others face adversity helps a writer find acceptance and hope*

BY CHRIS ANSELMO



Chris Anselmo (seated) at the 4th annual Strength, Science and Stories of Inspiration event in Cambridge, Mass., which he helped to organize.

**I**n recent months, I've experienced a noticeable decline in my arm and leg strength. My limbs feel heavy. It seems like they succumb a little bit more to gravity each day. One night, I tried to roll over in bed, and it took me six attempts — six! — to succeed. By the time I finally flipped over, I felt like I had just gone to the gym.

I know that progressive weakness comes with the territory of my disease, a type of distal myopathy called Miyoshi myopathy. In the 10 years since I began experiencing symptoms, I've adjusted to leg braces, crutches and a scooter, and I will eventually need to use a wheelchair. It's something I've accepted and

prepared for, and I remind myself often that a wheelchair will give me more freedom than I have now.

However, I'll be honest: I've been frustrated a lot lately. I may be mentally prepared for what the future holds, but it doesn't make living it any easier. The soreness, pain and weakness grind me down constantly. Some days I want to pout. I want to throw things. I want to yell at the top of my lungs.

It is in these moments of exasperation that I am reminded of the role models who have helped me through tough times before, whose examples when facing adversity gave me the strength and motivation to turn my life

around and learn to focus on the open doors, rather than the ones that closed unceremoniously in my face.

Acceptance has not come easily. At age 21, I was asymptomatic, a young adult living in Boston and enjoying life. Once the symptoms began — fatigue here, a muscle spasm there, followed by unexplained weakness — my life quickly unraveled. Depression took hold of me for several years. I was jealous of those around me, angry that my life was descending into a series of endless falls.

Then, in 2013, I was jolted back to reality when my co-worker and close friend Carly passed away from cancer. In June 2012, we were working together on a project, and by February she was gone. The way she handled such a horrific diagnosis with grace and dignity (she was only 24 — it still boggles my mind), is the most amazing thing I have ever seen.

Her passing made me re-examine how I was handling my own circumstances. I realized my life could not continue down the path I was going. Something had to change.

Although 2013 was a difficult year, it was a turning point. To this day, I consider Carly to be an angel, sent at

a specific moment to redirect the course of my life. I think of her every day, and she continues to bring me great strength in difficult moments.

Since then, I have found myself seeking out role models in my day-to-day life, in articles and in books. Many times, I stumble upon their stories by accident. In this way, I have gained several role models who encourage me on down days. Some are my good friends. Many are strangers I hope to meet someday, if only to say thank you. Others I'll never get the chance to meet, since they have already passed away. Each person encountered adversity of some sort, but they did not let whatever roadblocks they encountered derail them from maximizing every day.

*“Role models are everyday people doing everyday things. They don’t possess traits of a select few; they are just like you and me.”*

– Chris Anselmo

When I am down, they pick me up. On days when I feel like I have no strength, they move my arms and legs. They are inspiring, but not in the condescending way that the term often gets thrown around. They didn’t do anything “brave” or “heroic” or “unexpected” for someone in their situation. They merely lived life to the best of their abilities. Disability and adversity was not their whole life, just a small part of it. Carly, for example, was so much more than cancer.

Role models are everyday people doing everyday things. They don’t possess traits of a select few; they are just like you and me. They are relatable, and within their stories are lessons that anyone can benefit from. Within the MDA community there are so many whose stories have helped me in meaningful ways — Christopher Rush, Pete Frates, Mattie Stepanek — the list could go on indefinitely.

Today, I have reached an equilibrium in my life where, although I may not be thriving as well as I’d like, I’m able to endure. I’m able to persevere. I’m able to achieve my dreams and goals. I’m able to accept my circumstances, while never giving up hope for better days ahead.

I would be nowhere without my family and friends. But I owe a special debt of gratitude to those ordinary, everyday heroes whose examples I have been able to draw from — who gave me reason to believe in myself again. [Q](#)

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*Chris Anselmo, 31, lives in Connecticut with an adult-onset form of neuromuscular disease. Read more about the role models who have influenced him on his blog [sidewalksandstairwells.com](#). Anselmo also is a Quest and Strongly blog contributor.*

## HOW TO BE A ROLE MODEL: SHARE YOUR STORY

Here’s another lesson I’ve learned: We all have the capacity to be role models for others. If you have experienced adversity in your life, you can be a role model. It doesn’t require a special skillset and you don’t have to live a perfect life to make a difference.

I know what you’re thinking: “I live the most boring life ever!” or maybe, “I’m not doing anything that anyone else isn’t doing.” Well, guess what, you can still be a role model.

We have all lived an experience that someone else is just starting to go through. We’ve overcome — or are still dealing with — challenges that someone else is coming to grips with.

Sharing your journey can be daunting. But I guarantee you, if you tell your story authentically, sharing the good and the bad, the ups and the downs of what you are going through or struggling with, you will make a world of difference for someone.

A well-timed story — your story — may very well change the trajectory of someone’s life.

## Find Stories that Inspire Hope

Read stories from around the MDA community — from personal perspectives to research news — on MDA’s Strongly blog, at [strongly.mda.org](#). If you’re interested in sharing your story on Strongly, contact us at [strongly@mdausa.org](mailto:strongly@mdausa.org).

# Enter Stage Right

*A high school play sets the stage for a career in the arts*



Matt Plummer (seated) and his wife, Bethany (left), enjoy a family gathering.



## Make It Meaningful

Find your passion, and get involved in the MDA community in a way that's meaningful for you. Learn more at [mda.org/get-involved](http://mda.org/get-involved).

**M**att Plummer, a 30-year-old graphic, web and theatrical designer in Fort Worth, Texas, will never forget the role that introduced him to the world of theater and art during his sophomore year of high school: Townspeople No. 3 in Arthur Miller's *The Crucible*.

"I think a lot of people in my situation have a hard time expressing themselves," says Plummer, who lives with spinal muscular atrophy (SMA). "That experience really gave me a creative, artistic outlet to express myself and everything about me."

Plummer credits his high school theater experience with helping him find a passion that he turned into a career. "The most important thing for

me was finding a way out of my comfort zone," he says. "That led to things professionally, and personally it made me a happier and better person."

After high school, Plummer studied theater in college and earned a Master of Fine Arts in design from Texas Tech University. During his studies, he used his talents to design promotional materials and sets for stage productions. Currently, he works at Odessa College as a graphic and web design specialist.

Recently, the Odessa Arts organization selected Plummer for a project to beautify the city's traffic boxes. He designed a series of artistic coverings that promote literacy, reflecting the city's One Book Odessa program. **Q**



Above and top right: Traffic boxes designed by Plummer in Odessa, Texas.

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### MuSK-MG Patients

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### LEMS Patients

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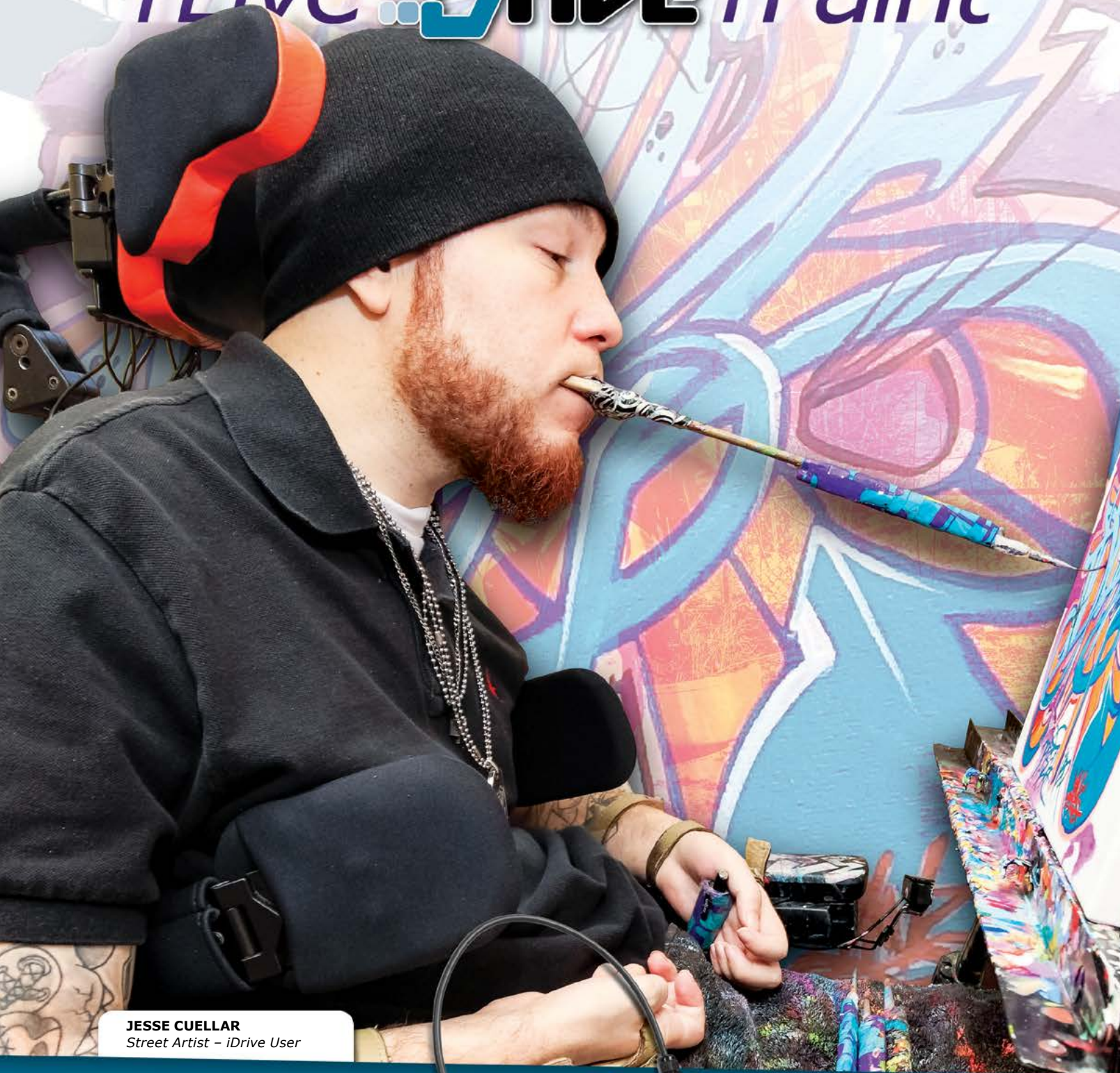
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