

EMPOWERING FAMILIES WITH INFORMATION AND INSPIRATION

# Quest

MDA.ORG/QUEST ISSUE 2 • 2020

## Change for the better

Advocacy works for the  
neuromuscular disease community

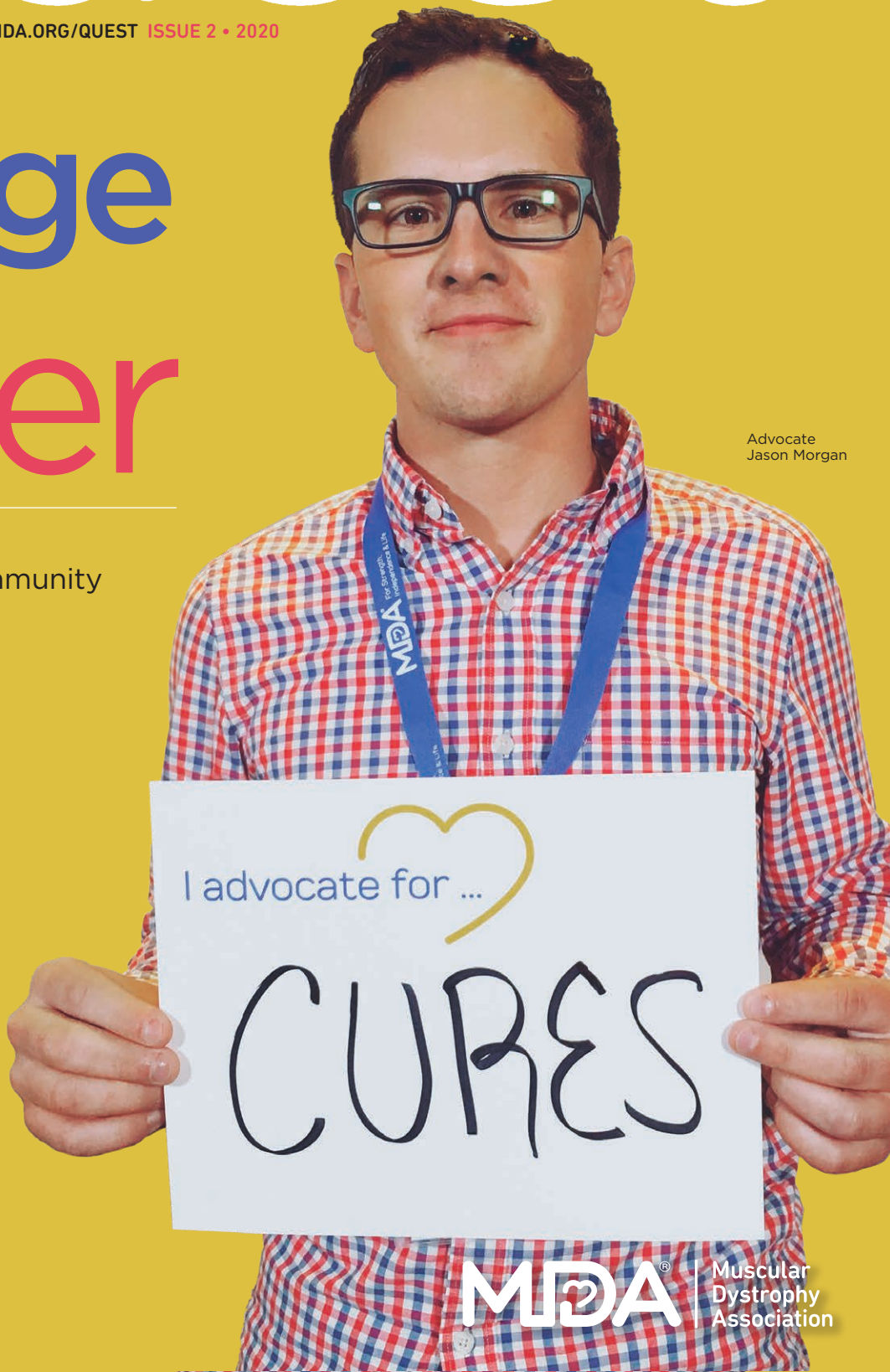
### GENETIC TESTING

Expanding access at  
MDA Care Centers

### 70 YEARS

A timeline of great  
moments in MDA's history

Advocate  
Jason Morgan



**MDA**<sup>®</sup>

Muscular  
Dystrophy  
Association

LIVING WITH MG



## The CHAMPION MG STUDY

Alexion is currently recruiting patients with anti-acetylcholine antibody receptor positive generalized myasthenia gravis (MG) 18 years of age or older for a Phase 3 study of ravulizumab-cwvz, called the CHAMPION MG Study. The study will assess ravulizumab-cwvz, compared to placebo, on the improvement of MG symptoms (MG activities of daily living). Participants may continue on their current medicines\*, as long as they are stable, and after a 26-week study treatment period all participants can receive ravulizumab-cwvz for an additional follow up period of up to 2 years. For more information and to learn if you are eligible for the CHAMPION MG Study, please contact [ClinicalTrials@alexion.com](mailto:ClinicalTrials@alexion.com) or go to [MGCHAMPION.com](http://MGCHAMPION.com).

\*Except for other complement inhibitors, rituximab, chronic Plasma Exchange or Intravenous Immunoglobulin

MDA is committed to transforming the lives of people affected by muscular dystrophy, ALS, and related neuromuscular diseases through innovations in science and innovations in care.

**EDITORIAL**

**Lindsey Baker**

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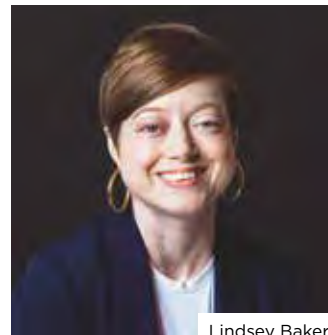


# Reflecting on Our Past and Present

Seventy years ago, a man living with muscular dystrophy picked up the phone. He gathered some friends who knew about his experience, who shared their own. And, together, they decided to change the future.

That man was New York businessman Paul Cohen, and in 1950 he and that group of supporters founded the Muscular Dystrophy Association. That year, MDA awarded its first research grant, \$1,500 to Ade T. Milhorat, an expert in muscular dystrophy at that time.

Since then, MDA has invested more than \$1 billion in neuromuscular disease research. Those funds have directly supported not just the discovery of the gene involved in Duchenne muscular dystrophy but the development of



Lindsey Baker

more than half of the drugs — including recent novel gene therapies — approved since the 1990s to treat neuromuscular diseases. Our Care Center Network, now in more than 150 hospitals across the United States, helps more than 50,000 individuals and families every year. Our families, friends, and volunteers show up for our community at Muscle Walks, at marathons as part of Team Momentum, at gala fundraisers, at MDA Summer Camp.

Seventy years in, we've made progress.

Seventy years in, we've still got work to do.

In this issue, as we honor MDA's legacy, we're also showcasing the ways we're still supporting research to accelerate treatments and cures, advocating to ensure our community has access to care, and provid-



Eleanor Gehrig, wife of Lou Gehrig, who lived with ALS, became involved with MDA in the 1950s. Here she helps break ground for the Institute for Muscle Disease.

ing necessary resources — including new no-cost genetic testing — to empower you as you make choices about your own future.

We're proud of our history. We're honored, today, to be part of yours, too.

Sincerely,

*Lindsey Baker*

*Quest Editor-in-Chief and General Manager  
Muscular Dystrophy Association*

## + TAKE THE 2020 QUEST READER SURVEY

Even as we look back, we look ahead — and we seek to improve. Let us know what you think of Quest, what we could do better, and what you'd like to see more (or less) of in these pages. Everyone who completes our reader survey will be entered into a drawing for a \$100 Visa gift card. Visit [surveymonkey.com/r/questreader](https://surveymonkey.com/r/questreader) or use this QR code.



Stephanie Erbacher's path to advocacy started with a playground swing for her daughter.



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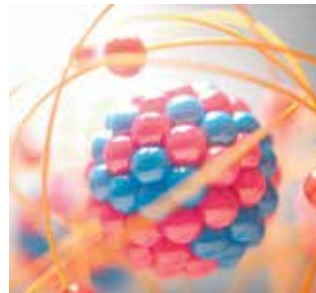
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**EDITOR'S NOTE**  
In *Quest* Issue 1, 2020, Sarah Clark-Stoney, MSW, LSW, was misquoted. Her quotes have been clarified and updated in the online article and the full, downloadable PDF version of the Issue. Find them, and more *Quest* content, at [mda.org/quest](http://mda.org/quest).

**PHOTO CONTEST**  
Share a photo of a meaningful moment for you or a loved one with a neuromuscular disease, and it could be selected to appear in a future issue of *Quest*. Learn more at [surveymonkey.com/r/questphoto](http://surveymonkey.com/r/questphoto).

MDA-057



A genetic diagnosis can make all the difference.  
Invitae is dedicated to helping people improve  
their health through genetic information.

## Invitae is proud to partner with MDA on the Detect Muscular Dystrophy program.

The Detect Muscular Dystrophy program provides sponsored, no-charge genetic testing and counseling for individuals suspected of having a muscular dystrophy.

Talk to your clinician if you suspect your child is showing signs of muscular dystrophy. Learn more about Detect Muscular Dystrophy at [www.invitae.com/detect-muscular-dystrophy](http://www.invitae.com/detect-muscular-dystrophy)



INVITAE



# Continue to Learn

At MDA, we strive to bring knowledge and resources to you in accessible and understandable ways. MDA's Engage community education program provides important, up-to-date, and relevant educational content to the neuromuscular disease community.

While we've shifted from in-person to virtual events for the remainder of 2020, the scope and availability of resources has not changed. We invite you to explore our educational offerings.

## MDA Engage Seminars

These virtual seminars address important topics such as best practices in clinical care, genetics, and research. Each seminar features regional specialists as presenters and focuses on a specialty area. While the events highlight regional medical professionals, we encourage families everywhere to attend any of the seminars.

## MDA Engage Disease-specific Symposia

Each of these virtual symposia focuses on a single diagnosis and includes presenters from across the country who focus on care, genetics, and research. Symposia also address other topics related to the specific diagnosis.



## MDA Engage Webinars

These online education sessions focus on a single topic for up to two hours, presented by experts in the field. Webinars are offered live and on demand.

There is no cost for members of the neuromuscular community and healthcare providers to attend Engage events. We do require pre-registration. [Q](#)

## +FIND AN EVENT

To register for upcoming MDA Engage activities and watch past events, visit [mda.org/engage](https://mda.org/engage).



## 2020 Engage Community Education Seminars

Regional presenters	Date	Specialty Topic
Louisiana	Aug. 22	NMD Pediatric Care & Resources
Pennsylvania	Sept. 19	Advocacy
Texas	Sept. 26	NMD Adult Care & Resources
New York	Oct. 3	Disability Benefits
Mississippi	Oct. 17	Canine & Assistive Services
Minnesota	Oct. 24	Adaptive Sports & Gaming
Florida	Nov. 7	Legal & Financial Resources

## 2020 Disease-specific Symposia

Disease focus	Date
Myasthenia gravis (MG)	Sept. 12
Spinal muscular atrophy (SMA)	Nov. 14
Amyotrophic lateral sclerosis (ALS)	Sept. 15-16



# VYONDYS 53

## (golodirsen) Injection

### DISCOVER VYONDYS 53

A treatment for Duchenne muscular dystrophy (DMD) in patients amenable to skipping exon 53



Duchenne is caused by mutations or errors in the *DMD* gene that prevent the body from producing the protein dystrophin, which is needed for muscles to work properly



VYONDYS 53 is an exon-skipping therapy. The goal of exon skipping is to allow the body to make a shorter form of the dystrophin protein



Under FDA accelerated approval, production of the protein dystrophin in skeletal muscle was accepted as a surrogate endpoint because it is reasonably likely to predict a meaningful clinical benefit. Continued approval of VYONDYS 53 may be contingent upon verification of a clinical benefit in confirmatory trials.



Speak to your doctor to find out if VYONDYS 53 is right for you. To learn more, visit [VYONDYS53.com](http://VYONDYS53.com)

## INDICATION

VYONDYS 53 is used to treat patients with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the dystrophin gene that can be treated by skipping exon 53.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.



## IMPORTANT RISK INFORMATION

- Allergic reactions, including rash, fever, itching, hives, and inflammation and/or peeling of the skin have occurred in patients who were treated with VYONDYS 53. Seek immediate medical care if signs and symptoms of allergic reactions occur.
- Damage to the kidneys was seen in animals who received golodirsen. Although damage to the kidneys was not seen in clinical studies with VYONDYS 53, potentially fatal kidney damage has occurred with other drugs that work in a similar way. Your doctor may recommend urine collection and blood testing before starting treatment followed by urine testing every month and a blood test every 3 months to monitor your kidneys.
- Adverse reactions that have occurred in at least 20% of patients treated with VYONDYS 53 and more often than in patients who received an inactive intravenous (IV) infusion were headache (41%, 10%), fever (41%, 14%), fall (29%, 19%), pain in the abdomen (27%, 10%), infection of the nose and throat (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).
- Other adverse reactions that occurred in greater than 5% of patients treated with VYONDYS 53 and more often than in patients who received an inactive IV infusion were pain at the IV site, back pain, pain, diarrhea, dizziness, stretch or tear in a ligament, bruising, flu, pain in the mouth and throat, stuffy or runny nose, scrapes or scratches of the skin, ear infection, seasonal allergy, fast heartbeat, reactions related to the IV catheter site, constipation, and broken bones.
- **You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch) or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).**

Please see the brief summary of Prescribing Information for VYONDYS 53 on the adjacent page.



## **VYONDYS 53 (golodirsen) Injection 50 mg/mL (100 mg/2 mL) Brief Summary**

**INDICATIONS AND USAGE:** VYONDYS 53 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

**CONTRAINDICATIONS:** None.

**WARNINGS AND PRECAUTIONS: Hypersensitivity Reactions:** Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy.

**Renal Toxicity:** Renal toxicity was observed in animals who received golodirsen. Although renal toxicity was not observed in the clinical studies with VYONDYS 53, renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Renal function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of renal function in DMD patients. Measurement of glomerular filtration rate (GFR) by 24-hour urine collection prior to initiation of therapy is recommended. Monthly monitoring for proteinuria by dipstick urinalysis and monitoring of serum cystatin C every three months is recommended. In the case of a confirmed dipstick proteinuria of 2+ or greater or elevated serum cystatin C, a 24-hour urine collection to quantify proteinuria and assess GFR should be performed.

**ADVERSE REACTIONS: Clinical Trials Experience:** Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in the clinical trials of another drug and may not reflect the rates observed in practice.

In the VYONDYS 53 clinical development program, 58 patients received at least one intravenous dose of VYONDYS 53, ranging between 4 mg/kg (0.13 times the recommended dosage) and 30 mg/kg (the recommended dosage). All patients were male and had genetically confirmed Duchenne muscular dystrophy. Age at study entry was 6 to 13 years. Most (86%) patients were Caucasian.

VYONDYS 53 was studied in 2 double-blind, placebo-controlled studies.

In Study 1 Part 1, patients were randomized to receive once-weekly intravenous infusions of VYONDYS 53 (n=8) in four increasing dose levels from 4 mg/kg to 30 mg/kg or placebo (n=4), for at least 2 weeks at each level. All patients who participated in Study 1 Part 1 (n=12) were continued into Study 1 Part 2, an open-label extension, during which they received VYONDYS 53 at a dose of 30 mg/kg IV once weekly.

In Study 2, patients received VYONDYS 53 (n=33) 30 mg/kg or placebo (n=17) IV once weekly for up to 96 weeks, after which all patients received VYONDYS 53 at a dose of 30 mg/kg.

Adverse reactions observed in at least 20% of treated patients in the placebo-controlled sections of Studies 1 and 2 were (VYONDYS 53 [N=41]%, Placebo [N=21]%) : headache (41, 10), pyrexia (41, 14), fall (29, 19), abdominal pain (27, 10), nasopharyngitis (27, 14), cough (27, 19), vomiting (27, 19), and nausea (20, 10).

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were: administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

Hypersensitivity reactions have occurred in patients treated with VYONDYS 53.

**USE IN SPECIFIC POPULATIONS: Pregnancy: Risk Summary:** There are no human or animal data available to assess the use of VYONDYS 53 during pregnancy. In the U.S. general population, major birth defects occur in 2 to 4% and miscarriage occurs in 15 to 20% of clinically recognized pregnancies.

**Lactation: Risk Summary:** There are no human or animal data to assess the effect of VYONDYS 53 on milk production, the presence of golodirsen in milk, or the effects of VYONDYS 53 on the breastfed infant.

The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for VYONDYS 53 and any potential adverse effects on the breastfed infant from VYONDYS 53 or from the underlying maternal condition.

**Pediatric Use:** VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping, including pediatric patients.

Intravenous administration of golodirsen (0, 100, 300, or 900 mg/kg) to juvenile male rats once weekly for 10 weeks (postnatal days 14 to 77) did not result in postnatal developmental (e.g., neurobehavioral, immune function, or male reproductive) toxicity. However, at the highest dose tested (900 mg/kg/week), golodirsen resulted in the death of animals because of renal impairment or failure. In surviving animals (including one animal at the lowest dose tested), there was a dose-dependent increase in the incidence and severity of renal tubular effects (including degeneration/regeneration, fibrosis, vacuolation, and dilatation), which correlated with changes in clinical pathology parameters, reflecting a dose-dependent impairment of renal function. In addition, decreases in bone area, mineral content, and mineral density were observed at the highest dose tested (900 mg/kg week) but with no effect on bone growth. A no-effect dose for renal toxicity was not identified; the lowest dose tested (100 mg/kg/week) was associated with plasma exposures (AUC) approximately 2.5 times that in humans at the recommended human dose of 30 mg/kg/week.

**Geriatric Use:** DMD is largely a disease of children and young adults; therefore, there is no geriatric experience with VYONDYS 53.

**Patients with Renal Impairment:** Renal clearance of golodirsen is reduced in non-DMD adults with renal impairment, based on estimated glomerular filtration rate calculated using the Modification of Diet and Renal Disease (MDRD) equation. However, because of the effect of reduced skeletal muscle mass on creatinine measurements in DMD patients, no specific dosage adjustment can be recommended for DMD patients with renal impairment based on estimated glomerular filtration rate. Patients with known renal function impairment should be closely monitored during treatment with VYONDYS 53.

**NONCLINICAL TOXICOLOGY: Carcinogenesis, Mutagenesis, Impairment of Fertility: Carcinogenesis:** Carcinogenicity studies have not been conducted with golodirsen.

**Mutagenesis:** Golodirsen was negative in *in vitro* (bacterial reverse mutation and chromosomal aberration in CHO cells) and *in vivo* (mouse bone marrow micronucleus) assays.

**Impairment of Fertility:** Fertility studies in animals were not conducted with golodirsen. No effects of golodirsen on the male reproductive system were observed following weekly subcutaneous administration (0, 120, 300, or 600 mg/kg to male mice or weekly intravenous administration (0, 80, 200, or 400 mg/kg) to male monkeys. Plasma exposure (AUC) at the highest doses tested in mouse and monkey are approximately 10 and 45 times that in humans at the recommended weekly intravenous dose of 30 mg/kg.

**How Supplied:** VYONDYS 53 is supplied in single dose vials containing 100 mg/2 mL (50 mg/mL)

Manufactured for: Sarepta Therapeutics, Inc., Cambridge, MA 02142 USA

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# PROGRESS NOW

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

## **Amyotrophic lateral sclerosis (ALS)**

### **Arimoclomol Gets Fast Track Designation**

Orphazyme announced Fast Track designation from the US Food and Drug Administration (FDA) for the development of the investigational drug arimoclomol to treat ALS. Arimoclomol previously received Fast Track status from the FDA for the treatment of Niemann-Pick disease type C (NPC) and sporadic inclusion-body myositis (sIBM).

Arimoclomol is a therapy that amplifies a stress response in cells known as the heat shock response. This response promotes the production of correctly folded

proteins within cells, helping to ensure that the cells function properly. The actions of arimoclomol are expected to have a disease-modifying effect in patients with ALS.

Orphazyme initiated a phase 3 trial in August 2018 to study efficacy of arimoclomol in patients with early-stage ALS. Topline results from this trial are expected in the first half of 2021.

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For more information, visit [orphazyme.com](http://orphazyme.com).

## **Duchenne muscular dystrophy (DMD)**

### **Viltolarsen Advances to Phase 3 Trial**

NS Pharma announced positive results from its phase 2 clinical trial of viltolarsen for treatment of DMD amenable to skipping exon 53. Results showed the therapy appeared to be safe and well tolerated, with no serious adverse events. Also encouraging, participants showed a significant increase in the production of dystrophin, an essential protein for keeping muscles intact, as well as improved performance on timed function tests (e.g., time to stand from supine, time to run/walk 10 meters, time to climb four stairs, the North Star Ambulatory Assessment, and a six-minute walk test).

Sixteen boys with DMD amenable to skipping exon 53 were enrolled in the trial and received viltolarsen by intravenous (IV) infusion each week for 20–24 weeks.

A phase 3 trial began in October 2019 to further assess the safety and efficacy of the drug. This trial is currently enrolling patients.

Viltolarsen has not been approved in the United States (it is approved in Japan), but its New Drug Application

was granted Priority Review by the US Food and Drug Administration (FDA). The FDA's decision is expected in the third quarter of 2020.

While being investigated by the FDA, viltolarsen is available to eligible patients in the United States through an Expanded Access Program. The program is open to all DMD patients who are amenable to skipping exon 53 and who meet other eligibility criteria.

---

To learn more about the completed phase 2 trial, visit [clinicaltrials.gov](http://clinicaltrials.gov) and enter NCT02740972 in the “Other Terms” search box. For information about the phase 3 clinical trial, enter NCT04060199 in the search box. For more information about the Expanded Access Program or to inquire about participation, email [nspharma.expandedaccess@earlyaccesscare.com](mailto:nspharma.expandedaccess@earlyaccesscare.com).



**Duchenne muscular dystrophy (DMD)**

# Positive Results from Gene Therapy Trial



Sarepta Therapeutics announced positive results from its phase 1/2a study of SRP-9001 gene therapy to treat DMD, an open-label trial known as Study 101.

Data from four patients indicated that a single intravenous (IV) infusion of SRP-9001 was safe and well

tolerated, with no serious adverse events. Additionally, all participants demonstrated increased expression of microdystrophin in muscle fibers and improvements on the North Star Ambulatory Assessment scale.

SRP-9001 is an investigational gene therapy that uses an adeno-associated virus to introduce a shortened version of the dystrophin gene (mini dystrophin) into muscle tissue of patients with DMD, partially compensating for their lack of a functional dystrophin gene.

In addition to Study 101, Sarepta Therapeutics is conducting a randomized, double-blind, placebo-controlled study of SRP-9001 (Study 102). Results from this study are expected in early 2021.

For more information about the phase 1/2a study of SRP-9001 (Study 101), visit [sarepta.com](http://sarepta.com). To learn more about the phase 2 study (Study 102), visit [clinicaltrials.gov](http://clinicaltrials.gov) and enter NCT03769116 in the "Other Terms" search box.

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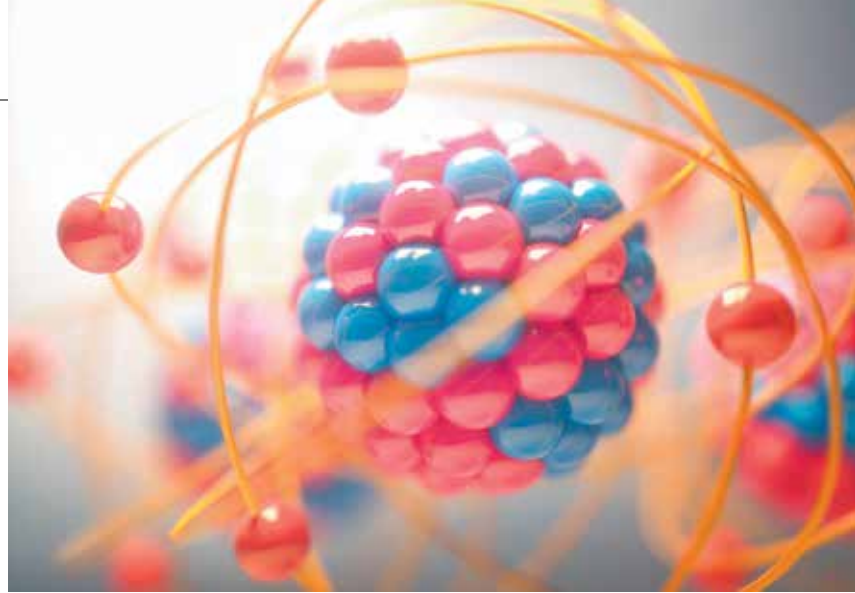
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# Long-term Data on Vamorolone Shows Few Side Effects

ReveraGen BioPharma announced the completion of two-and-a-half years of vamorolone treatment in 41 boys with DMD.

Vamorolone is a synthetic steroid that binds to the same receptors as corticosteroids but does not activate all the same downstream gene pathways in the cell. As a result, vamorolone appears to elicit fewer side effects than corticosteroid therapies.

The long-term data from the study indicated that daily oral administration of vamorolone, even at its highest tested dose, was safe and well tolerated with no serious adverse events. The phase 2b trial is ongoing, but preliminary data have shown that vamorolone may not have some of the safety concerns of corticosteroids, such as deflazacort (Emflaza) and prednisone, while exhibiting similar or even enhanced efficacy.



ReveraGen BioPharma initiated a phase 3-like trial of vamorolone in 2018 that is still recruiting boys with DMD (ages 4-7). This study will measure the safety and efficacy of vamorolone treatment in comparison to placebo or corticosteroid therapy (prednisone) over the course of six months.

To learn more about the phase 3-like trial that is enrolling, visit [clinicaltrials.gov](https://clinicaltrials.gov) and enter NCT03439670 in the "Other Terms" search box.

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## Duchenne muscular dystrophy (DMD)

# Gene Therapy Shows Promise

Pfizer announced positive results from a phase 1b trial assessing treatment with PF-06939926 in ambulatory boys with DMD.

PF-06939926 is an investigational gene therapy that uses an adeno-associated virus to introduce a shortened version of the dystrophin gene (mini dystrophin) into patients with DMD, partially compensating for their lack of a functional dystrophin gene.

Pfizer initiated the phase 1b open-label, non-randomized trial in 2018 to assess safety and tolerability of ascending doses of a single intravenous (IV) infusion of PF-06939926. Other objectives included measurement of dystrophin expression and distribution, as well as assessments of muscle strength, quality, and function.

Preliminary data indicated that the IV administration of PF-06939926 was well tolerated during the infusion period

and showed improvements at 12 months post-infusion, including sustained levels of mini dystrophin expression and improvements on the North Star Ambulatory Assessment scale.

Pending regulatory approval, Pfizer plans to advance its phase 3 program and begin dosing patients in the second half of 2020.

To learn more about the trials, visit [clinicaltrials.gov](http://clinicaltrials.gov) and enter NCT03362502 in the "Other Terms" search box.

## Limb-girdle muscular dystrophy (LGMD)

# Gene Therapy Well Tolerated in Phase 2 Trial



Sarepta Therapeutics announced positive results of SRP-9003, a gene therapy designed to treat LGMD type 2E (LGMD2E).

LGMD2E is caused by mutations in the beta-sarcoglycan gene (*SGCB*), which prevent production of beta-sarcoglycan (beta-SG) protein. Beta-SG is normally found in muscle cells and works to protect muscle fibers from injury as they contract and relax.

SRP-9003 uses an adeno-associated virus to introduce a functional *SGCB* gene into skeletal and cardiac muscles in patients with LGMD2E. SRP-9003 is designed to achieve particularly robust production of beta-SG protein in heart cells to counteract the cardiac complications common in LGMD2E.

This was the therapy's first study in human patients. Study participants were between ages 4-15. Objectives included assessing the safety and tolerability of a single intravenous (IV) infusion of SRP-9003, as well as measurement of beta-SG, measurement of creatine kinase (CK)

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levels (an indicator of muscle damage), and assessment of patient performance on timed function tests.

Results showed the therapy to be well tolerated, with elevated liver enzymes and nausea being the most common suspected adverse events. Participants also showed increased expression of beta-SG in muscle fibers, marked reductions in CK, and improvements on timed function tests.

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For more information about the trial of SRP-9003, visit [clinicaltrials.gov](https://clinicaltrials.gov) and enter NCT03652259 in the “Other Terms” search box.

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## Myasthenia gravis (MG)

# Positive Results for Generalized MG Treatment

Momenta Pharmaceuticals announced positive results from its phase 2 Vivacity-MG trial assessing treatment with nipocalimab (M281) in patients with generalized MG. Preliminary data indicated that treatment with nipocalimab, using four different dosing protocols over an eight-week period, resulted in improvements in patients’

Myasthenia Gravis Activities of Daily Living (MG-ADL) scores, which was the study’s primary endpoint. Additionally, nipocalimab was shown to be well tolerated and safe in all testing groups, with no serious adverse events reported.

### +MG UPDATE

Learn more about MG treatment advances in “The Expanding Therapeutic Landscape for Myasthenia Gravis” on the next page.

Nipocalimab is an engineered human immunoglobulin (IgG) anti-FcRn antibody that functions to reduce the levels of other IgG antibodies in the blood, including the

autoantibodies that cause generalized MG. Nipocalimab is expected to improve signaling between nerve and muscle cells, thereby improving muscle function in patients with generalized MG and alleviating the signs and symptoms of the disease.

The phase 2 study is expected to be completed in the third quarter of 2020.

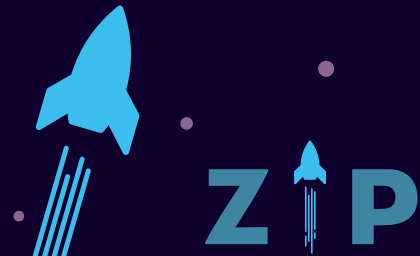
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To learn more about the trial, visit [clinicaltrials.gov](https://clinicaltrials.gov) and enter NCT03772587 in the “Other Terms” search box.

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## ADVERTISEMENT



## Pompe Pediatric Study Now Enrolling

The ZIP study will evaluate how an investigational co-administration treatment regimen of ATB200 and AT2221 may work for adolescents (12 through 17 years of age) with late-onset Pompe disease, who have either received Enzyme Replacement Therapy (ERT) (ERT-experienced) or have never received ERT (ERT-naïve) previously.

The safety and efficacy of the investigational agents ATB200 and AT2221 has not been established, and they are not approved for use in any country.

For more information, please visit [pompestudy.com/zip](https://pompestudy.com/zip) or [clinicaltrials.gov](https://clinicaltrials.gov) (Identifier: NCT03911505).



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NP-AT-US-00010320

# The Expanding Therapeutic Landscape for Myasthenia Gravis

A Q&A with Gil I. Wolfe, MD

**M**yasthenia gravis (MG) is an autoimmune disease that leads to muscle weakness.

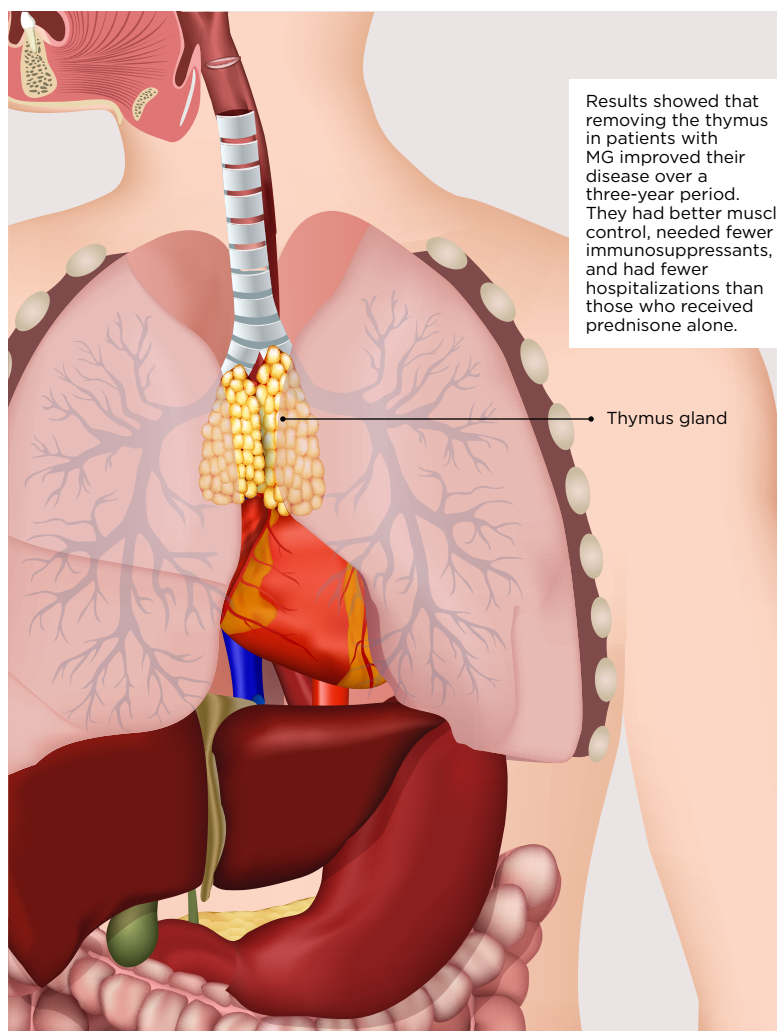
It affects about 14 to 20 out of every 100,000 people. The age of onset varies widely, but typically it is diagnosed in women in their 20s and 30s and men in their 60s or later. In about 10% to 15% of cases, MG begins in childhood.

To learn about the latest MG treatment advances, we talked with Gil I. Wolfe, MD, Irvin and Rosemary Smith professor and chairman of the Department of Neurology at the Jacobs School of Medicine & Biomedical Sciences at the University at Buffalo in New York, who led an extension study on the surgical treatment for MG that was published in *The Lancet Neurology* in 2019.

## What is MG?

It is the most common defect of neuromuscular transmission, or the messaging between nerves and muscles. That disrupted communication leads to muscle weakness, which is why the name means “grave muscular weakness.”

In MG, antibodies (which usually protect us from infections) attack and destroy acetylcholine receptors in the muscles. Nerves release acetylcholine at the neuromuscular junction (where the nerves and muscles intersect). Acetylcholine then binds to receptors on the muscle membrane, much like a key entering a lock. This activates the muscle



Results showed that removing the thymus in patients with MG improved their disease over a three-year period. They had better muscle control, needed fewer immunosuppressants, and had fewer hospitalizations than those who received prednisone alone.

Thymus gland

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Myasthenia gravis is the most common defect of neuromuscular transmission, or the messaging between nerves and muscles. That disrupted communication leads to muscle weakness, which is why the name means ‘grave muscular weakness.’

and allows it to contract. Without enough unlocked receptors, the muscle can't contract.

The first symptoms of MG typically are eye muscle weakness, double vision, and droopy eyelids. In some patients, weakness around the eyes is the only sign of the disease; we call that ocular MG. The other type is general MG, which also involves muscles in the arms, legs, and chest, including those that control breathing.

### What is the surgical treatment for MG?

The close association between MG and thymic abnormalities has been known for more than a century. For instance, patients with MG are more likely to get tumors of the thymus. About 80 years ago, observations first appeared in medical literature documenting that removing the thymus gland (a procedure called thymectomy), even if there was no tumor, seemed to benefit patients with MG. But they were small case studies that did not meet current clinical trial standards.

Still, based on those publications, thymectomy became a common treatment for MG. However, doubts remained as to exactly how effective it was in MG patients without thymic tumors.

The MGTX thymectomy study, supported by MDA and the National Institutes of Health, was designed to fill that gap in knowledge. The trial compared the effects of thymectomy plus prednisone (a corticosteroid) to prednisone alone. Results showed that removing the thymus in patients with MG improved their disease over a three-year period. They had better muscle control, needed fewer immunosuppressants, and had fewer hospitalizations than those who received prednisone alone.

The MGTX extension study then followed about half the participants for another two years and found the benefits continued.

Most patients with MG won't reach remission, which is defined as a medication-free year, but we can get patients to minimal manifestation status. This means they can work and manage family obligations and the disease is not notably impairing their function. At the end of the extension study, almost 90% of patients who had the surgery were at minimal manifestation status compared to less than 60% of those who received only medication.

There also appears to be a cost benefit to thymectomy, which is less expensive than emerging therapies or a lifetime



Gil I. Wolfe, MD

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The American Academy of Neurology released a practice statement noting that thymectomy is safe and effective.

of medications. It is invasive, but we now have minimally invasive surgical techniques that are easier on patients.

### Will the study change how the disease is treated?

Yes, we are now updating guidelines for the treatment of MG. Also, the American Academy of Neurology released a practice statement on thymectomy, noting that it is safe and effective, and that patients should be aware of the option.

### How is MG treated with medication?

We start with an acetylcholinesterase inhibitor like pyridostigmine. These drugs work by keeping more acetylcholine around the neuromuscular junction, thus helping improve nerve/muscle communication and reducing the weakness patients experience. Most people with MG also require immunosuppressants, such as corticosteroids, to suppress the autoimmune response that causes the disease. But these drugs have serious side effects, especially when taken long

term, so the goal is to use them sparingly.

We also use IV gamma globulin and plasma exchange when patients have a flareup or if their symptoms don't improve with other therapies. About 15% of patients don't respond to conventional treatments.

In 2017, the US Food and Drug Administration (FDA) approved eculizumab (Soliris) for MG, a humanized monoclonal antibody that blocks the complement system, which we know plays a key role in the destruction of the muscle membrane.

### What else is under investigation?

The therapeutic investigational landscape in MG is as large and active as it has ever been, and there is a lot of excitement. In addition to anti-B cell therapies (the family of white blood cells that make antibodies) like rituximab, researchers are exploring other monoclonal antibodies, including ravulizumab — which requires less frequent infusions than eculizumab — and zilucoplan, which can be delivered subcutaneously rather than infused. Each of these agents works on the complement system.

Ongoing clinical trials of molecules that block the neonatal Fc receptor, which dramatically reduces the amount of Immunoglobulin G antibodies, also show promise. [Q](#)

# Change for the better

Stephanie Erbacher is a passionate advocate on behalf of her 12-year-old daughter, Rylie, who has spinal muscular atrophy (SMA). Her path to advocacy started when Rylie couldn't swing at a local playground in Cedar Rapids, Iowa.

"I pushed the parks department to put in an adaptive swing with back support and a solid harness in front," Stephanie says. When park officials responded that an accessible playground might be built in more than a year, Stephanie pressed on, emailing the city manager and eventually talking to the superintendent of parks. "I let them know I had researched costs of the swing and was very motivated to work with the city to see how we could find a solution that would allow disabled children to have even just a single option to be included at the playground."

Within six weeks, the city installed an adaptive swing.



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*Advocacy for — and within — the  
neuromuscular disease community*

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BY MYRNA TRAYLOR

# nope



Stephanie learned that by using her voice, she could improve one small piece of the world not only for her daughter but for all kids with disabilities in her community. And that, in a nutshell, is advocacy: working toward a solution for a collective need.

### Advocacy at MDA

When it comes to supporting the neuromuscular disease community, there are many worthy needs: raising awareness, changing rules, securing funding, supporting research.

This is where MDA's Advocacy team comes in. This group works on legislation and guidelines at the federal level to ensure that actions at the state and local levels are backed by federal law, policies, and procedures.

### +FOLLOW US ON TWITTER

Follow [@MDA\\_Advocacy](#) for the latest announcements and calls to action from MDA's Advocacy team.

"We work with the federal government, on the Congressional side and the regulatory side, on policies to improve the lives of people living with neuromuscular disease," explains Brittany Johnson Hernandez, MDA's senior director of Policy and Advocacy. "This is important because, without an organized operation that advocates can join, it is difficult to sort out the priorities and find effective ways to work."

MDA welcomes individuals to join our advocacy efforts in ways that are meaningful to them. Our current advocacy direction focuses on three priorities: access to care and



## 3 THINGS YOU CAN DO NOW

- 1. Join our advocacy network.** Sign up for email updates and find opportunities to get involved at [mda.org/advocacy](https://mda.org/advocacy).
- 2. Contact your Congressional representative.** Ask them to add provisions to upcoming healthcare or COVID-19-related legislation that help the neuromuscular disease community, such as ending surprise medical bills.
- 3. Learn more about advocacy.** The MDA Advocacy Institute is a series of webinars featuring experts discussing advocacy basics, as well as specific issues. They're available to anyone at [cqrcengage.com/mda/advocacyinstitute](https://cqrcengage.com/mda/advocacyinstitute).

Image: iStock.com/LEOcrafts

therapies from day one, accelerating therapy development, and empowerment and independence.

### From the grassroots up

Becoming an advocate often starts with an experience or cause that is important to an individual. "All you need to

“A lot of advocacy work is just connecting with the right people and explaining why these things matter — and why they should matter.”  
—Stephanie Erbacher

start is the desire to do it," Stephanie says.

"A lot of advocacy work is just connecting with the right people and explaining why these things matter — and why they should matter."

For example, one issue MDA advocates are involved in is updating standards for accessible air travel. "When we flew to Washington, DC, for a neuromuscular disease conference in 2019, my

daughter's power wheelchair was damaged on both legs of the flight," Stephanie says.

Jason Morgan, who serves as chair of the Board of Commissioners for Washtenaw County, Mich., and lives with Becker muscular dystrophy (BMD), has heard many such stories. "We



Stephanie Erbacher learned that advocacy is a way to improve the world for her daughter and all kids with disabilities in her community.

“

Direct advocacy is critical to making sure that these issues are a priority for lawmakers. We all have to work to make lawmakers aware of the issues.

—Jason Morgan

certainly should be able to bring wheelchairs onto an airplane, or at the very least be able to use the restroom,” Jason says. “There need to be clear standards on how to care for people with disabilities on an airplane. There are many instances of people being picked up out of their chairs in ways that aren’t safe. Plus, we need them to take better care of power wheelchairs, which currently aren’t handled well.”

To affect change in the large and heavily regulated airline industry, MDA has endorsed the Air Carrier Access Amendments Act, introduced by Sen. Tammy Baldwin of Wisconsin and Rep. James Langevin of Rhode Island. In addition, MDA has joined with other organizations to call on airlines, the Department of Transportation, and the Transportation Security Administration (TSA) to ensure that new policies and procedures during the COVID-19 pandemic accommodate passengers with disabilities.

“When we work with MDA directly through existing channels, asking for the same things in a coordinated way, we can have greater impact,” Jason says.

It’s important to remember that advocacy is not just about influencing major industries or moving national legislation. “Working at all levels is important,” Jason says. “At the local level there can be an impact with city, county, and state issues, such as accessibility of sidewalks, building construction, or savings funds for people with neuromuscular disease.”

### A high tide floats all boats

Jason credits MDA Summer Camp with sparking his interest in advocacy. He first attended after he was diagnosed with BMD at age 13 and enjoyed going to Summer Camp for more than a decade, first as a camper, then as a counselor.

Jason has worked in government for years, serving on the staff of a US Congress member, as well as in the Michigan state government. He attended the past two MDA Advocacy Conferences in Washington, DC, where he met with key



Jason Morgan (far left) and other advocates met with Congress members during the last MDA Advocacy Conference in Washington, DC.

House of Representative members and their staff.

“The most important thing about being an advocate for people living with neuromuscular disease is to raise your voice: Talk to members of Congress, raise awareness on social media, fundraise for Summer Camp, and secure funding for a cure,” he says.

“Direct advocacy is critical to making sure that these issues are a priority for lawmakers. We all have to work to make lawmakers aware of the issues.”

Even when the focus is on neuromuscular diseases other than BMD, Jason is committed to the cause. “Many of us feel that a high tide floats all boats. The best and most critical diseases need to be addressed,” he says. “Mine is not as severe as Duchenne muscular dystrophy, but the care for that should translate to BMD as well.”

Stephanie agrees that the benefits of advocacy can be far-reaching. “When you get a neuromuscular disease diagnosis, you feel this loss of control and hopelessness,” she says. “I know that advocating will help not just my daughter but other people, and move the needle in a positive direction. That’s a common theme; working toward change is therapeutic and uplifting.” [Q](#)

Myrna Traylor is a writer and editor for *Quest*.

### +MAKING OUR VOICES HEARD

Even during the COVID-19 pandemic, MDA continues to make sure lawmakers hear the voice of the neuromuscular disease community. Read more about this in “MDA’s 2020 Advocacy Agenda Update” at [mda.org/quest](https://mda.org/quest).

# illuminating Unseen Dimensions

Every day, people living with neuromuscular diseases like muscular dystrophy, amyotrophic lateral sclerosis (ALS), and related conditions juggle medications, doctor appointments, and daily challenges with the everyday business of life. We're introducing you to just a handful of those in our community — multifaceted, driven by passions, and dreaming big. Like anyone, they persevere, especially in the face of uncommon difficulty. This is a celebration of them, what brings light into their lives, and their oft-unseen but vital realities.

Read full stories at [mda.org/unseendimensions](https://mda.org/unseendimensions).



## **Jordan Reidenberg**

At the age of 6, Jordan was diagnosed with Duchenne muscular dystrophy (DMD). What seemed to be a simple problem with walking ended up being a debilitating disease that, for many, might have crushed the spirit. But not for Jordan. Meeting other kids living with DMD, through advocacy efforts and MDA Summer Camp, showed him he could belong — and just be a kid, too. An 18-year-old soon-to-be high school senior with an eye on careers that could make the world more accessible for everyone, for now, he spends time with his beloved service dog, Jolly, and is set on conquering every teen's dream: driving.

## **Rich Razumny**

While no life is lit by sunshine all the time, Rich knows that with the umbrella family provides, one can weather any storm. For 29-year-old software engineer Rich, the goal of living an idyllic family life was set early; he married his beloved Leah not long after college graduation, and their first child followed shortly after. But unexpected symptoms — a limp, dexterity issues, and fatigue — in Rich soon raised concerns. Tests showed he had ALS, a fatal neurodegenerative disease. Rich is determined to make the most of every day he has. The birth of his second child, participation in MDA's Engage and Parenting at a Challenging Time events, and proactive changes to his routine have made an immense impact on his quality of life — and how he and his family cultivate joy.



Image: iStock.com/Nozomu





### ***Hannah and Austin Stacks***

Sometimes it seems as though time is traveling at the speed of light. The Stacks know. Austin and Hannah are siblings who love Xbox, their dachshunds, and MDA Summer Camp. They were also both diagnosed with Friedreich's ataxia (FA) and are living with a loss of coordination and muscle control and, increasingly, a decrease in stamina. Still, they share a sibling rivalry that keeps family games fun (and heated). Hannah, 17, made the conscious choice to be home-schooled in an accessible environment; Austin, 13, still attending school, is a social butterfly with a passion for video games. The kids' parents, Candy and Jason, would love to stop the clock, spend more time with their children, and slow the progression of their disease. But with no cure on the horizon, the family's goals have shifted. Each day, the Stacks make mindful choices to maximize enjoyment together. They've learned what so many people neglect to remember: Time is fleeting.



## **Maria Llave**

Eighteen-year-old Maria has always lived by a simple rule: "This is what I can't do. And this is what I can do. This is me." When she was a baby, her parents took a leap of faith, abandoning their lives in their native Peru to seek top-notch medical care for Maria's spinal muscular atrophy (SMA) type 2 diagnosis. Their high-stakes chance paid off: Maria received paramount medical care at Columbia University's Irving Medical. Enduring seven surgeries for spine growth issues and helping her parents navigate a new country, Maria has never allowed limitations to interfere with what's possible. Her confidence only grew at MDA Summer Camp, preparing her for high school, where she forged stellar friendships and honed her theater skills to shine in the spotlight.



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# EXPANDING ACCESS TO

# Gen

Genetic testing plays an important role in diagnosing, treating, and managing neuromuscular disease. “Genetic testing can shorten the time to diagnosis and prevent misdiagnosis of muscular dystrophies,” says Robert Nussbaum, MD, chief medical officer of Invitae, a leader in advanced medical genetics. “An earlier, accurate diagnosis can facilitate earlier interventions, alert physicians about potential complications, allow genetic counseling of family members, and support clinical research into neuromuscular diseases.”

Yet a quarter of those living with neuromuscular disease and a third of adult patients have not had their diagnosis confirmed with genetic testing, according to MDA’s 2018 ONEVoice survey. Of the respondents who had not had genetic testing, the two main reasons cited were lack of access to it or that it was not affordable.





# etic Testing



*MDA Care Centers bring no-cost genetic testing to people suspected of having a muscular dystrophy*

BY DONNA ALBRECHT

Earlier this year, MDA and Invitae announced a joint program that offers no-cost genetic testing and post-test counseling for certain neuromuscular diseases through all MDA Care Centers. Called Detect Muscular Dystrophy, the program was first developed by Invitae in July 2019. Forming a partnership around the program helps both Invitae and MDA further their goals of reducing barriers to genetic testing for people searching for answers.

“We worked with the company to bring this program to MDA Care Center physicians,” says Sharon Hesterlee, MDA executive vice president and chief research officer. “Both MDA and Invitae concluded that understanding the landscape of muscular disease mutations has enough intrinsic value that the cost of testing should be waived. As more people have the genetic cause of their disease identified, it will help lay the groundwork for clinical trials, reimbursement for new therapies, and better services for those living with these disorders.”

### The first step

Accessing the Detect Muscular Dystrophy program usually starts with talking with your doctor, who can refer you to an MDA Care Center in your area. The Detect Muscular Dystrophy program is available through Care Centers to individuals (in the US and Canada) who have experienced progressive muscle weakness, elevated creatine kinase (CK)

levels, a family history of muscular dystrophy, or a positive test result for Duchenne muscular dystrophy (DMD) through newborn screening, among other eligibility criteria.

Tests are done with a sample of blood or saliva. Results are available anywhere from 10 to 21 days after Invitae receives the sample.

“This is a fairly comprehensive panel of tests,” Sharon explains. “It covers up to 123 genes associated with inherited muscle disorders, including muscular dystrophies, myopathies, and congenital myasthenic syndromes. The panel does not currently cover repeat expansion or contraction disorders, like myotonic dystrophy or facioscapulohumeral muscular dystrophy, but these may be added in the future.”

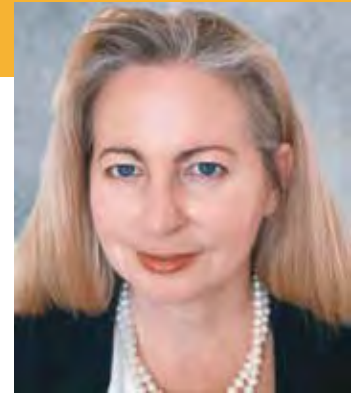
### Getting test results

Once the test results are in, a genetic counselor schedules a follow-up visit with the patient — or their parents, in the

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MDA and Invitae concluded that understanding the landscape of muscular disease mutations has enough intrinsic value that the cost of testing should be waived.

—Sharon Hesterlee



case of a minor — to discuss the diagnostic results. “This visit confirms the diagnosis, establishes a treatment plan, and initiates FDA-approved medication or the availability of a clinical trial,” says Ellen Moran, a

genetic counselor at the MDA Care Center at NYU Langone Health’s Center for Children.

Subsequent visits may be scheduled to discuss the risk of recurrence, family implications, and reproductive options. In some cases, the genetic counselor will recommend genetic testing for family members to identify who is at risk for the disorder in themselves or transmitting the disorder to their offspring.

The amount of information shared during post-test genetic counseling can be immense.

“The information is emotional, and we know that at some point it becomes overload,” Ellen says. “We encourage families to bring support with them, while remaining cognizant of protecting the patient’s and family’s privacy.”

To supplement the genetic counseling, Care Center staff can provide educational materials about diagnosis, treatments, and genetics that families can take home.

### Common questions

Genetic testing and post-test genetic counseling are offered at no cost to patients through the Detect Muscular Dystrophy program. The costs of the program are paid by Invitae and its sponsors.

Beyond the cost of testing, many families have questions about the impact of test results on their children and family members and the potential for insurance discrimination. Some may be familiar with direct-to-consumer genetic tests that may make health information available to others. Results through the Detect Muscular Dystrophy program are private, Dr. Nussbaum assures.



“As a medical genetics company, Invitae is covered by HIPAA [the Health Insurance Portability and Accountability Act of 1996], which means patient information is protected with us just like it is at a doctor’s office,” he says. “We believe patients should own and control their data, and we never share identifiable data without consent; for example, for research.”

“  
We believe patients should own and control their data, and we never share identifiable data without consent; for example, for research.”  
—Robert Nussbaum, MD



**Testing from home**  
The COVID-19 pandemic and subsequent stay-at-home measures broadened healthcare providers’ abilities to utilize telehealth, making consultations — including those for genetic testing and counseling — more convenient.

Many primary care providers offer phone or videoconferencing appointments. For those who prefer not to leave

home, this can be a good way to ask your provider about the Detect Muscular Dystrophy program and whether you qualify.

Doctors can contact Detect Muscular Dystrophy and request that a saliva test be sent to a patient by mail. You can follow the directions for providing a sample of saliva and mail it back to Invitae. When the results are in, a genetic counselor may be able to provide your diagnostic results and post-test counseling by videoconference.

Research has shown that no-charge testing programs help increase utilization of genetic testing, which can shorten the time to diagnosis by as much as two years for some conditions. MDA and Invitae’s goal with the Detect Muscular Dystrophy program is to extend the benefits of genetic testing to more people who might have muscular dystrophies, so they can start treatment earlier; understand how their disease is likely to progress, and get the best possible care.

“This new partnership between Invitae and MDA will provide people with neuromuscular disease unprecedented access to genetic testing,” Sharon says.

Donna Albrecht is the author of “Raising a Child Who Has a Physical Disability” (Wiley). She lives with her husband and border collie near San Francisco.

**+OUR COMMITMENT**  
For 70 years, MDA has been committed to transforming the lives of individuals living with neuromuscular diseases through innovations in science and care. To learn more, visit [mda.org](https://mda.org) or call 1-833-ASK-MDA1.

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>40% of patients taking  
SPINRAZA are adults\*

3-80  
DAYS YEARS

Has treated SMA in patients  
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90%

>90% of patients who started  
SPINRAZA remain on treatment<sup>‡</sup>

\*Based on commercial patients, early access patients, and clinical trial participants through December 2019.

<sup>†</sup>Includes clinical trial patients.

<sup>‡</sup>Based on commercial patients in the US (including Puerto Rico) through December 2019.

<sup>§</sup>Clinical studies of SPINRAZA did not include sufficient numbers of subjects aged 65 and over to determine whether they respond differently from younger patients.

#### INDICATION

SPINRAZA® (nusinersen) 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 respiratory infection, fever, constipation, headache, vomiting, 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.

**Before taking SPINRAZA**, tell your healthcare provider if you are pregnant or plan to become pregnant.

**Please see full Prescribing Information on SPINRAZA.com.**

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#### IMPORTANT FACTS ABOUT SPINRAZA<sup>®</sup> (nusinersen)

##### USES

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

##### WARNINGS

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##### COMMON SIDE EFFECTS

- **The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, 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.

**Before taking SPINRAZA**, tell your healthcare provider if you are pregnant or plan to become pregnant.

##### 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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# MDA Fundraising Goes Virtual



The virtual gala included a live auction with auctioneer Fred Northup, Jr.

Despite social distancing requirements, MDA has gotten creative when it comes to some of our most popular fundraising events. Quick, innovative thinking has turned galas, Muscle Walks, and Fill the Boot events into virtual opportunities, generating crucial funds for MDA to serve its mission during these challenging times. These events also allow the community to join together to celebrate an important milestone — MDA's 70th anniversary.

## Online gala raises nearly \$1 million

What do you do when your popular galas can't be held in person? You replicate them online. MDA led the industry by hosting a virtual gala on May 1, gathering sponsors and supporters from more than eight events that would have been held live in the spring.

More than 700 attendees tuned in at [YouTube.com/mda](https://www.youtube.com/mda), and the gala, which included an auction, raised more than \$960,000. Thanks to the success of the spring format, MDA plans to hold four more virtual galas this fall: Toast to Life, Black-N-Blue Ball, Muscle Team, and Night of Hope. Each will gather groups from around the nation to support MDA's mission and celebrate our sponsors and supporters.

Check [mda.org](https://mda.org) for announcements of upcoming galas.

## Tracking steps for Muscle Walk

Live walks have been affected by the limits on large gatherings. Moving our in-person Muscle Walks to a virtual format this summer gives supporters the opportunity to walk, run, dance, or move any way they'd like to raise funds for MDA.

Through a partnership with the Charity Miles app, individuals can track their activity and share with their friends and community to sponsor their movement. The app also connects directly to a fitness tracker — which allows for wheelchair activity tracking as well — through the user's phone. The virtual Muscle Walk ends Labor Day weekend.

To participate, visit [mda.donordrive.com/event/virtualmusclewalk](https://mda.donordrive.com/event/virtualmusclewalk).

## Fill the (virtual) Boot

Normally, fire fighters would be hitting the streets this summer, asking their communities to Fill the Boot for MDA. This year, the International Association of Fire Fighters (IAFF) and MDA worked together to find a safer alternative to rally for the mission.

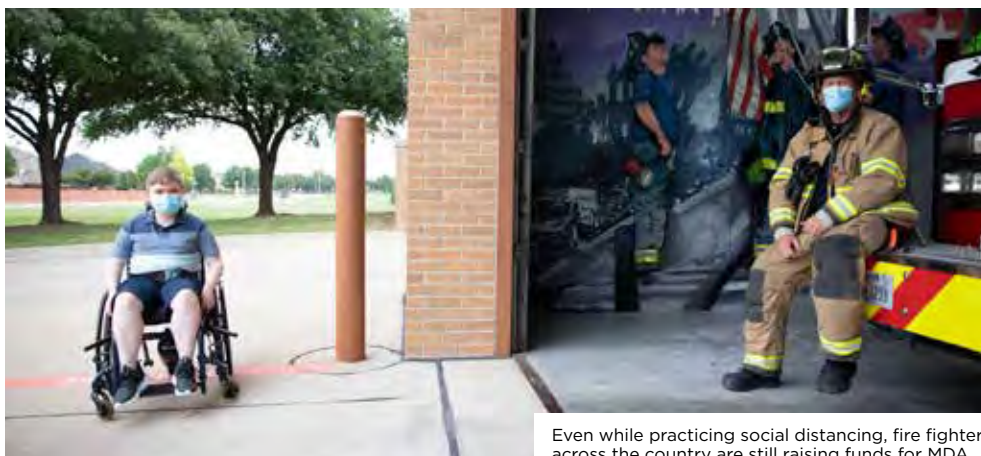
Fire fighters and community members can join virtual Fire House Teams to Fill the Boot for MDA. This virtual Fill the Boot drive features friendly state-by-state fundraising challenges, social media recognition, and the chance to access special fire house recipes with every donation.

Virtual Fill the Boot starts in August.

To join a Fire House Team and Fill the Boot for MDA, visit [filltheboot.donordrive.com](https://filltheboot.donordrive.com).

## +SUPPORT QUEST

Make a donation to MDA that will help *Quest* continue to educate and engage families and the people who help them. To make a tax-deductible donation to MDA, visit [mda.org/questsupport](https://mda.org/questsupport).



Even while practicing social distancing, fire fighters across the country are still raising funds for MDA.



## The CHAMPION ALS Study

The CHAMPION ALS study is a clinical trial seeking to enroll people living with ALS. The purpose of the study is to assess the safety and efficacy of ravulizumab-cwvz compared to placebo in adults with ALS.

You may be eligible to participate if you have been diagnosed with ALS, are at least 18 years of age, and have had ALS symptoms for up to 3 years.

For more information about participating in the CHAMPION ALS study, visit [alschampion.com](http://alschampion.com) and talk to your doctor.

This information is intended as educational information for patients. It does not replace a doctor's judgment or clinical diagnosis. The medication being studied in the CHAMPION ALS study is not FDA approved for use in ALS.

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



## Drink One for Dane Raises Nearly \$1.4 Million

Dutch Bros Coffee and its customers joined together to raise \$1.39 million for MDA on May 8, the 14th annual Drink One For Dane day. More than 390 drive-through shops in seven states participated to support amyotrophic lateral sclerosis (ALS) research, patient care, and advocacy.

"We're so grateful to everyone who went online or came out to support Drink One for Dane this year," says Travis Boersma, CEO and co-founder of Dutch Bros Coffee. "There's a lot going on in the world right now, but that doesn't change the need to end ALS. Our customers and 'bro-istas' really are amazing and are truly making a massive difference in the lives of so many."

Dutch Bros donated a portion of proceeds from the day's shop sales and offered a specialty mug and sticker package online, which raised an additional \$50,000 for MDA.



Supporters show their love for Dutch Bros and MDA at the 14th annual Drink One for Dane.

MDA has partnered with Dutch Bros since the company held the first Drink One for Dane fundraiser in 2007. The partnership began after Dutch Bros co-founder Dane Boersma was diagnosed with ALS. Since then, Dutch Bros has raised more than \$8.4 million to help support ALS patients and find a cause and cure for the disease.

"This is a testament to the purpose-driven, caring culture of Dutch Bros. Their commitment to unlocking a cure for ALS is remarkable," says Lynn O'Connor Vos, president and CEO of MDA. "Our longstanding partnership with Dutch Bros carries on Dane's legacy. We have a shared mission to protect and care for ALS patients as we work to discover a cure."

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To purchase a specialty mug and join the fight against ALS, visit [dutchbros.com/endsals](http://dutchbros.com/endsals).

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For more than 25 years, *Quest* has been telling the stories of MDA's community. With your donation to MDA, we can continue to educate and engage families and the physicians and researchers who help them with the latest news on neuromuscular disease research, health and wellness, mobility, travel, advocacy, and everyday thriving through *Quest* and our complementary educational channels.

THANK YOU FOR HELPING TO KEEP OUR COMMUNITY INFORMED AT THIS MOST CRUCIAL TIME.



## ADVERTISEMENT

### DO YOU KNOW A BOY WITH DUCHENNE MUSCULAR DYSTROPHY?

If so, he could be eligible for CIFFREO—a clinical study that will assess if a gene therapy (the study drug) is safe and the effect it has on muscles in boys with Duchenne.

#### Can we treat Duchenne differently?

Duchenne is a neuromuscular disease that causes muscle degeneration. Although current treatments for the disease, such as steroids, can be beneficial for some patients, we hope that the study drug will provide an option for the wider Duchenne population. We also hope that it will offer them more protection against muscle degeneration.

#### About the study drug

Dystrophin is an important protein that helps keep muscles strong and healthy. But in people with Duchenne, the gene that codes for the making of dystrophin contains a mistake, which causes muscles to break down and get weaker.

The study drug is designed to produce what we call a “mini-dystrophin.” The goal of this investigational mini-dystrophin treatment is to help your child’s muscles work better. This approach is known as gene therapy.

For this study, the investigational gene therapy works by using a modified virus to deliver a shortened dystrophin gene to targets within the body. The virus is changed so that it cannot cause an infection.

Once the new gene is within the body, signals direct it to produce the shortened dystrophin protein in the heart and skeletal muscles. The hope for the study drug is to help keep muscles strong in participants with Duchenne, which could improve and stabilize their walking (if the boy is still walking) as well as help with other daily activities.

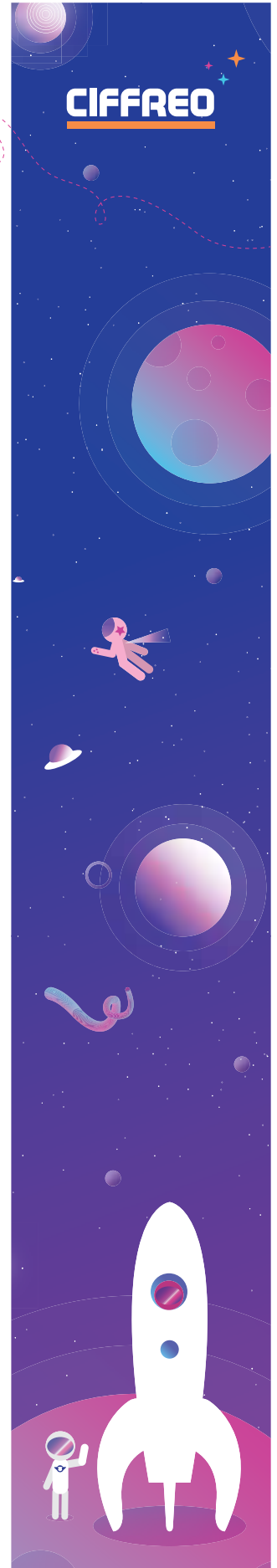
#### Who can join the CIFFREO study?

This study is looking for approximately 100 boys to take part. Among other criteria, each boy must be between 4 and 7 years old (up to his 8th birthday), have a prior genetic diagnosis of Duchenne (all mutations), have been taking daily steroids for at least three months, and be able to walk short distances on his own.

Learn more at [CIFFREODuchenneTrial.com](https://www.CIFFREODuchenneTrial.com)



**CIFFREO**



# Smashing Stereotypes

*Making sorority life more inclusive, one chapter at a time*

BY JESSICA HETZEL

W e all know sororities have a stereotype: skinny girls with blonde hair and blue eyes. I have never seen a disabled sorority girl.

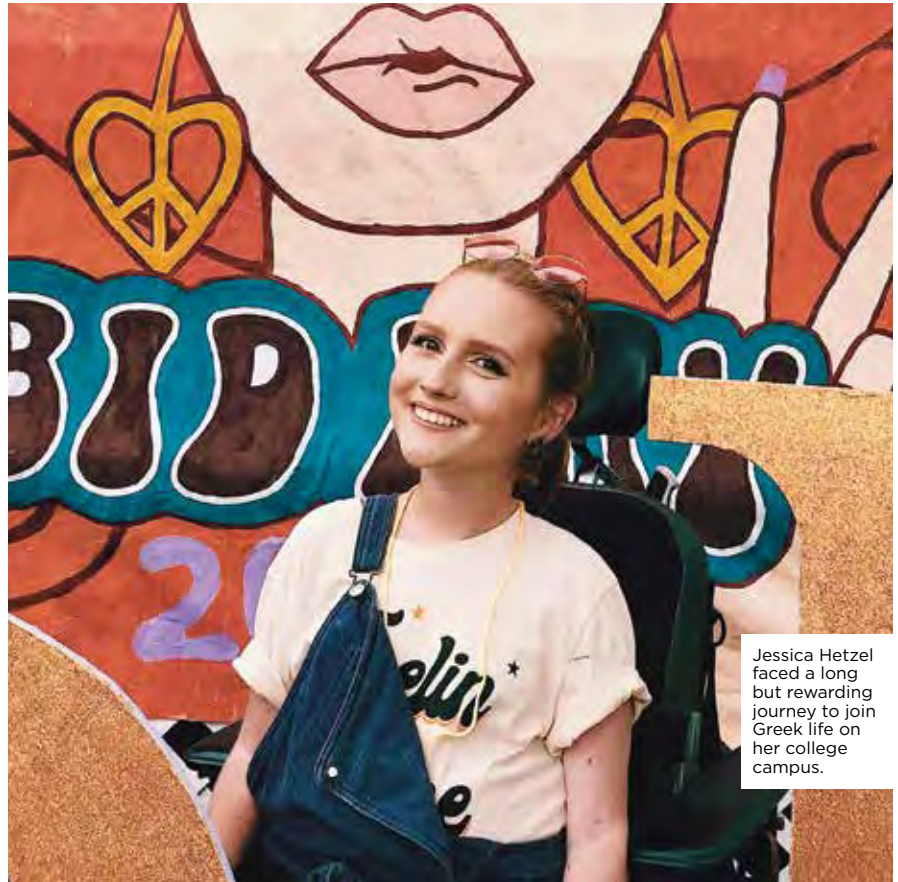
Despite that stereotype, I knew I wanted to be a part of Greek life when I started my freshman year at Central Michigan University (CMU) in fall 2019. I signed up for formal recruitment as soon as registration opened.

Sorority recruitment is scary enough for an able-bodied woman, but as a woman with spinal muscular atrophy (SMA) who uses a power wheelchair, it was 10 times scarier. Throughout the process, I had worries in the back of my mind: Would the chapters discriminate against me? Would the girls ignore or pity me? Were the sorority houses accessible? If I couldn't get into the house, would they not want me?

## Finding a home

In September 2019, I was thrilled to find my home at the Phi Mu-Rho Delta chapter. On bid day, it was fun meeting my sisters, but I couldn't get into our sorority house or take pictures on our porch. On that day, I felt excluded because of the staircase I watched my sisters walk up and down.

About a month later, CMU's Office of Civil Rights and Institutional Equity (OCRIE) requested to meet with me regarding an investigation into discrimination. They said that during recruitment, two girls in a chapter decided to eliminate me because of my disability, and other members had reported it. My worst fear had come true. I had a knot in the pit of my stomach for months and wondered: Did I do something wrong? How could someone see me as



Jessica Hetzel faced a long but rewarding journey to join Greek life on her college campus.

unworthy because I'm disabled? Why would someone think less of me for something I can't change? That feeling of discrimination is so deep and painful.

OCRIE gave me three options: take legal action, further the investigation, or handle it on my own. I decided to handle it by holding an educational meeting with the girls who did the discriminatory act. That meeting is still in the works, but other aspects of Greek life made me realize that they weren't the only ones who would benefit from education on disability and inclusion.

## Greek life

As much as I loved Phi Mu, I still felt excluded many times. When my sisters planned events, they did their best to make them accessible, but there were often hiccups. For example,

## +TELL YOUR STORY

Sharing your journey can be daunting, but it can make a world of difference to someone going through similar struggles. If you'd like to share your story with *Quest*, email us at [quest@mdausa.org](mailto:quest@mdausa.org).

we held an overnight retreat at an accessible venue, but there were only bunk beds, and it is extremely difficult for me to get on a bunk bed.

My sisters would attend events hosted at other Greek houses that were inaccessible. I would be in group chats watching my sisters plan a sleepover, and I knew I couldn't attend because it was at an inaccessible apartment, and I use medical equipment at night.

The more I saw my sisters attending parties and events that I couldn't go to, the sadder I got. I considered dropping out of the sorority. What was the point of being in a sorority if I had to constantly say no to activities?

### Eye-opener

My life changed when I took "History of Disabilities in America." This class talked about experiences I've had my whole life and showed me that I wasn't alone. My professor, JoDell Heroux, PhD, inspired me so much that I decided to be the change I wanted to see in Greek life. I was not going to let one more woman be discriminated against during recruitment. I was not going to let one more woman feel unworthy because she was disabled.

In January, I hosted a chapter development meeting for Phi Mu focused on disability, inclusion, and service animals. I asked Dr. Heroux to present with me, and our one-hour-and-15-minute inclusion training seminar touched on service animal dos and don'ts, stereotypes of disabilities, different models of disabilities, ableism around us, inspiration porn, and the importance of inclusive language. We also led an activity on ableism to help our audience practice active learning and increase their information retention.

That day was a turning point. Since the meeting, our sisterhood has been stronger and richer. For the first time, I truly feel connected and included in my sorority. I feel almost as if my sisters can finally see the real me. And I hope they'll use their knowledge to educate other people.

### Bright future

Becoming a part of Greek life has been a long journey, and it's only my first year. Even though I have faced challenges and difficulties, I wouldn't trade any of my experiences

“

Sorority recruitment is scary enough for an able-bodied woman, but as a woman with spinal muscular atrophy (SMA) who uses a power wheelchair, it was 10 times scarier. —Jessica Hetzel



because then I wouldn't be able to help others. The feeling of educating people is so empowering, and I will continue to do it my whole life.

I plan to present the training seminar to all of the sororities and fraternities on campus. My goal is to make sure every sorority and fraternity house is accessible by the time I graduate. I want to make CMU's Greek life the most accessible and inclusive in the country, because everyone deserves to find their home.

Discrimination stems from lack of knowledge, fear, and stereotypes. Once you remove those, you'd be surprised how accepting people are. Recently, CMU's Greek life community gave me the New Member of the Year award.

I am very proud, and I'm excited for what my future holds.

Grow through what you go through, and always be kind. [@](#)

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Jessica Hetzel, 18, lives in Michigan with her service dog, Emerson. She is a college student, disability advocate, and public speaker. Follow her journey on instagram: [@jessshetzel](#).

# Great Moments in MDA History

AS MDA CELEBRATES its 70th anniversary, we look back at some of the milestones in our mission to transform the lives of people with muscular dystrophy, ALS, and related neuromuscular diseases.



## 1954

Fire fighters began standing on street corners with boots in hand after MDA was named

the International Association of Fire Fighters' (IAFF) "charity of choice." Through the iconic Fill the Boot campaign, IAFF has raised \$670 million.

## 1952

The National Association of Letter Carriers (NALC) became the first official national MDA sponsor. Fundraising efforts through raffles, pancake suppers, golf tournaments, backyard carnivals, and more have helped bring in \$107 million.



## 1955

The first MDA Summer Camp debuted with 16 campers. Now, MDA hosts camps across the country in which youngsters ages 8-17 with neuromuscular diseases can experience a fun-filled week where anything is possible.



## 1966

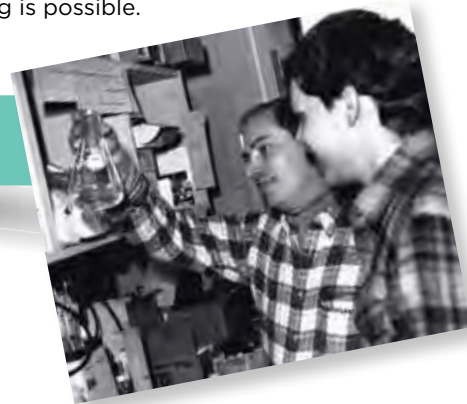
The MDA Telethon was first televised on a single station in New York. The Telethon became

a Labor Day tradition, viewed nationally and hosted by Jerry Lewis for more than 50 years. Over the decades, these star-studded events raised more than \$2.45 billion.



## 1986

Louis Kunkel, PhD, and his team of researchers, supported by MDA, identified the gene that, when flawed, causes Duchenne muscular dystrophy (DMD). This discovery opened the door to gene therapy for neuromuscular diseases.



## 2001

Jerry Lewis led a delegation of MDA scientists and clients to testify before a subcommittee of the US Senate, resulting in the introduction of the MD-CARE Act, which secured essential federal funding for neuromuscular disease research.



### +ENTER THE LASTING IMPRESSION PHOTO CONTEST

We want to recognize you, our readers, and the extraordinary moments in your lives. Share a photo of a meaningful moment for you or a loved one with a neuromuscular disease, and it could be selected to appear in Lasting Impression in a future issue of *Quest*. All photo entries must be submitted by Sept. 13, 2020, at [surveymonkey.com/r/questphoto](https://www.surveymonkey.com/r/questphoto).

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## The National ALS Registry

is a program that allows  
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We are working towards a better  
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**Helping** researchers find possible risk factors

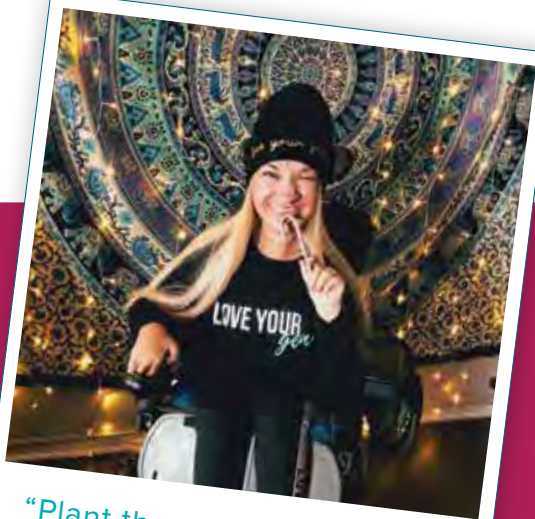
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