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The Truth

about nutrition and neuromuscular disease

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MOVING AHEAD with

Evrysdi® (risdiplam)



1,800+

**people in the US with spinal
muscular atrophy (SMA) are
taking Evrysdi, including
people up to 84 years old****

*Number of people taking Evrysdi as of May 2022. Evrysdi approved in August 2020.

†Clinical studies of Evrysdi did not include people aged 65 and older to determine whether they respond differently from those who are younger

What is Evrysdi?

Evrysdi is a prescription medicine used to treat spinal muscular atrophy (SMA) in children and adults.

Important Safety Information

Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant, as Evrysdi may harm your unborn baby. Ask your healthcare provider for advice before taking this medicine
- are a woman who can become pregnant:
 - Before you start your treatment with Evrysdi, your healthcare provider may test you for pregnancy
 - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping Evrysdi
 - **Pregnancy Registry.** Talk to your healthcare provider right away if you become pregnant while taking Evrysdi. Ask about registering with the Evrysdi Pregnancy Registry, which was created to collect information about your health and your baby's health. Your healthcare provider can enroll you in this registry by calling 1-833-760-1098 or visiting www.evrysidipregnancyregistry.com
- are an adult male. Evrysdi may affect a man's ability to have children (fertility). Ask a healthcare provider for advice before taking this medicine
- are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby

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Studied in the **most inclusive** clinical study program in SMA^{‡§}

- ✓ For newborns to adults with SMA — **later-onset, infantile-onset, and presymptomatic SMA**
- ✓ Designed to help the body **make more SMN protein**
- ✓ Safety profile that has been studied in **more than 490 people** from newborns to adults
- ✓ Oral treatment that **can fit into your day**



[‡]Studies included individuals with a broad range of physical ability, including those with and without the ability to walk, with and without scoliosis (mild to severe), with and without prior disease-modifying treatment (evaluated for safety).

[§]The efficacy and safety of Evrysdi was established in 3 main studies. SUNFISH is a 2-part, placebo-controlled study in 231 adults and children aged 2 to 25 years with Type 2 or 3 SMA. FIREFISH is a 2-part, open-label study in 62 infants aged 2 to 7 months with Type 1 SMA. RAINBOWFISH is an ongoing, open-label study in 26 newborns younger than 6 weeks (at first dose). These newborns were genetically diagnosed with SMA and had not yet shown symptoms (presymptomatic SMA). A fourth study, JEWELFISH, is an ongoing, open-label safety study in 174 people aged 1 to 60 years with Type 1, 2, or 3 SMA that was previously treated with approved or investigational SMA medications.

Important Safety Information (continued)

Tell your healthcare provider about all the medicines you take.

You should receive Evrysdi from the pharmacy as a liquid. If the medicine in the bottle is a powder, **do not use it**. Contact your pharmacist for a replacement.

Avoid getting Evrysdi on your skin or in your eyes. If Evrysdi gets on your skin, wash the area with soap and water. If Evrysdi gets in your eyes, rinse your eyes with water.

The most common side effects of Evrysdi include:

- For later-onset SMA: fever, diarrhea, rash
- For infantile-onset SMA: fever; diarrhea; rash; runny nose, sneezing, and sore throat (upper respiratory infection); lung infection (lower respiratory infection); constipation; vomiting; cough

These are not all of the possible side effects of Evrysdi. For more information on the risk and benefits profile of Evrysdi, ask your healthcare provider or pharmacist.

You may report side effects to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at 1-888-835-2555.

Please see accompanying brief summary for additional Important Safety Information.

If you cannot afford your Evrysdi medication, visit MySMASupport.com for financial assistance information.

Talk with your doctor about Evrysdi
or visit www.Evrysdi.com/Go to learn more



Patient Information
EVRYSDI® [ev-RIZ-dee]
(risdiplam)
for oral solution

What is EVRYSDI?

- EVRYSDI is a prescription medicine used to treat spinal muscular atrophy (SMA) in children and adults.

Before taking EVRYSDI, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. If you are pregnant, or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. EVRYSDI may harm your unborn baby.
- are a woman who can become pregnant:
 - Before you start your treatment with EVRYSDI, your healthcare provider may test you for pregnancy. Because EVRYSDI may harm your unborn baby, you and your healthcare provider will decide if taking EVRYSDI is right for you during this time.
 - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping EVRYSDI.
 - **Pregnancy Registry.** There is a pregnancy registry for women who take EVRYSDI during pregnancy. If you become pregnant while receiving EVRYSDI, tell your healthcare provider right away. Talk to your healthcare provider about registering with the EVRYSDI Pregnancy Registry. The purpose of this registry is to collect information about your health and your baby's health. Your healthcare provider can enroll you in this registry by calling 1-833-760-1098 or visiting <https://www.evrysdipregnancyregistry.com>.
- are an adult male planning to have children: EVRYSDI may affect a man's ability to have children (fertility). If this is of concern to you, make sure to ask a healthcare provider for advice.
- are breastfeeding or plan to breastfeed. It is not known if EVRYSDI passes into breast milk and may harm your baby. If you plan to breastfeed, discuss with your healthcare provider about the best way to feed your baby while on treatment with EVRYSDI.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider, including your pharmacist, when you get a new medicine.

How should I take EVRYSDI?

See the detailed Instructions for Use that comes with EVRYSDI for information on how to take or give EVRYSDI oral solution.

- You should receive EVRYSDI from the pharmacy as a liquid that can be given by mouth or through a feeding tube. The liquid solution is prepared by your pharmacist or other healthcare provider. If the medicine in the bottle is a powder, **do not use it.** Contact your pharmacist for a replacement.
- Avoid getting EVRYSDI on your skin or in your eyes. If EVRYSDI gets on your skin, wash the area with soap and water. If EVRYSDI gets in your eyes, rinse your eyes with water.

Taking EVRYSDI

- Your healthcare provider will tell you how long you or your child needs to take EVRYSDI. Do not stop treatment with EVRYSDI unless your healthcare provider tells you to.
- For infants and children, your healthcare provider will determine the daily dose of EVRYSDI needed based on your child's age and weight. For adults, take 5 mg of EVRYSDI daily.
 - Take EVRYSDI exactly as your healthcare provider tells you to take it. Do not change the dose without talking to your healthcare provider.
- Take EVRYSDI 1 time daily after a meal (or after breastfeeding for a child) at approximately the same time each day. Drink water afterwards to make sure EVRYSDI has been completely swallowed.
- Do not mix EVRYSDI with formula or milk.
- If you are unable to swallow and have a nasogastric or gastrostomy tube, EVRYSDI can be given through the tube.
- If you miss a dose of EVRYSDI:
 - If you remember the missed dose within 6 hours of when you normally take EVRYSDI, then take or give the dose. Continue taking EVRYSDI at your usual time the next day.
 - If you remember the missed dose more than 6 hours after you normally take EVRYSDI, skip the missed dose. Take your next dose at your usual time the next day.
- If you do not fully swallow the dose, or you vomit after taking a dose, **do not take** another dose of EVRYSDI to make up for that dose. Wait until the next day to take the next dose at your usual time.

Reusable Oral Syringes

- Your pharmacist will provide you with the reusable oral syringe(s) that are needed for taking your medicine and explain how to use them. Wash the syringes per instructions after use. Do not throw them away.
- Use the reusable oral syringe(s) provided by your pharmacist (you should receive 1 or 2 identical oral syringes depending on your prescribed daily dose) to measure your or your child's dose of EVRYSDI, as they are designed to protect the medicine from light. Contact your healthcare provider or pharmacist if your oral syringe(s) are lost or damaged.
- When transferred from the bottle to the oral syringe, take EVRYSDI right away. Do not store the EVRYSDI solution in the syringe. If EVRYSDI is not taken within 5 minutes of when it is drawn up, EVRYSDI should be thrown away from the reusable oral syringe, and a new dose should be prepared.

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

- **For later-onset SMA:**
 - fever
 - diarrhea
 - rash
- **For infantile-onset SMA:**
 - fever
 - runny nose, sneezing, and sore throat (upper respiratory infection)
 - constipation
 - diarrhea
 - lung infection (lower respiratory infection)
 - vomiting
 - rash
 - cough

These are not all of the possible side effects of EVRYSDI. For more information, ask your healthcare provider or pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store EVRYSDI?

- Store EVRYSDI in the refrigerator between 36°F to 46°F (2°C to 8°C). Do not freeze.
 - If necessary, EVRYSDI can be kept at room temperature up to 104°F (up to 40°C) for a combined total of 5 days. EVRYSDI can be removed from, and returned to, a refrigerator. The total combined time out of refrigeration should not be more than 5 days.
- Keep EVRYSDI in an upright position in the original amber bottle to protect from light.
- Throw away (discard) any unused portion of EVRYSDI 64 days after it is mixed by the pharmacist (constitution) or if EVRYSDI has been kept at room temperature (below 104°F [40°C]) for more than a total combined time of 5 days. Discard EVRYSDI if it has been kept above 104°F (40°C). Please see the Discard After date written on the bottle label. (See the **Instructions for Use** that comes with EVRYSDI).

Keep EVRYSDI, all medicines and syringes out of the reach of children.

General information about the safe and effective use of EVRYSDI.

Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use EVRYSDI for a condition for which it was not prescribed. Do not give EVRYSDI to other people, even if they have the same symptoms you have. It may harm them. You can ask your pharmacist or healthcare provider for information about EVRYSDI that is written for health professionals.

What are the ingredients in EVRYSDI?

Active ingredient: risdiplam

Inactive ingredients: ascorbic acid, disodium edetate dihydrate, isomalt, mannitol, polyethylene glycol 6000, sodium benzoate, strawberry flavor, sucralose, and tartaric acid.

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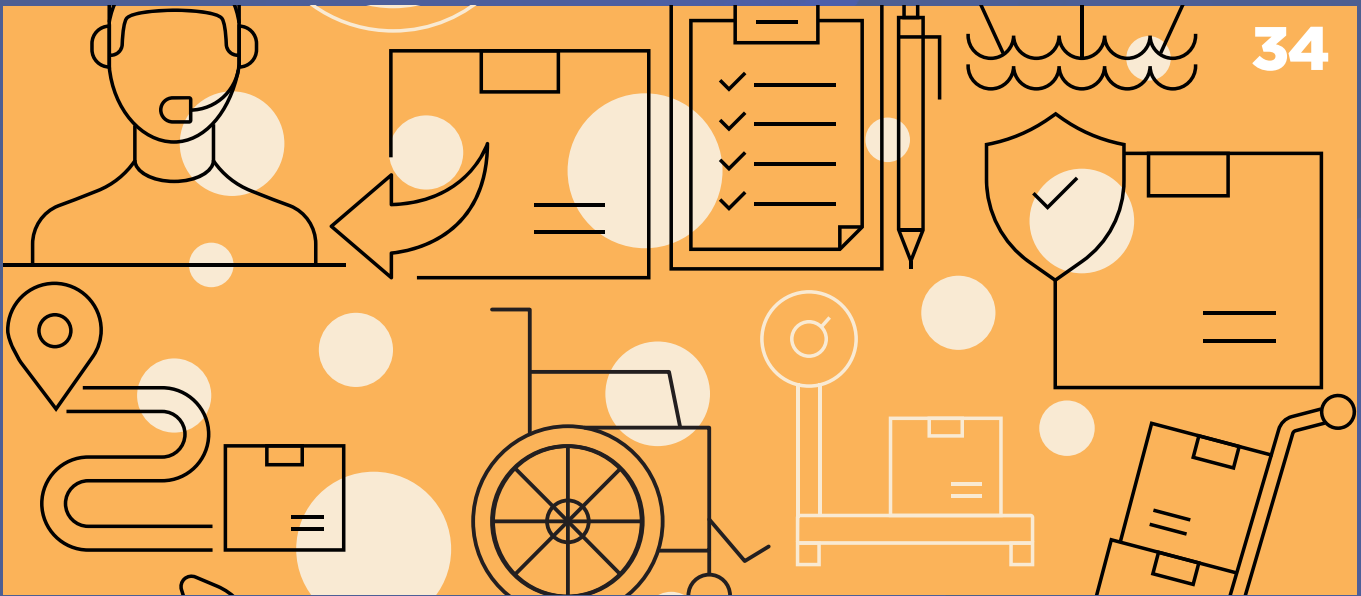
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For more information, go to www.EVRYSDI.com or call 1-833-387-9734.



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Cover image: iStock.com/wildpixel

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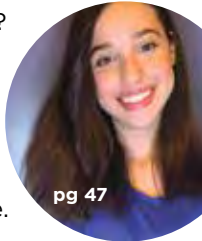


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Volunteers Drive and Inspire Us

MDA has been leading the way as the #1 voluntary health organization for more than 70 years, and families are at the heart of our mission. We couldn't fulfill our mission without the volunteers who work tirelessly to support the neuromuscular disease community in so many ways. That is why we have proclaimed 2023 the Year of the Volunteer.

Volunteering takes many forms. It includes not only donating time and talents to things like MDA Summer Camp and gala events, but also philanthropy — giving strategic gifts to help us solve problems over the long term. The generosity of our volunteers allows us, year after year, to continue to provide the research, care, and programs that are empowering individuals with neuromuscular diseases to live longer, more independent lives.

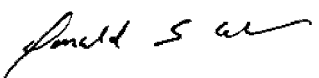
Our volunteers and donors tell us they also experience enormous rewards from taking the time to give back. Volunteering leads to new friendships and lasting relationships. It creates the opportunity for individuals to connect with one another and develop new skills

“The sense of purpose and community our volunteers create has a lasting impact — not just for those we serve, but for the volunteers themselves. Volunteers feel they are getting back more than they are giving when they donate their time and talents to MDA.

— **Wendi Dressen, MDA Senior Director, Volunteer Services**

to support MDA. They are making a profound difference and have our sincere gratitude. Strength in unity. Strength in community.

Sincerely,



Donald S. Wood, PhD
President and CEO
Muscular Dystrophy Association



Donald S. Wood, PhD

and senses of purpose. And whether you are an MDA volunteer or donor, you benefit by knowing that you are strengthening our mission and expanding our reach.

MDA's board of directors is comprised of prominent business and community leaders who volunteer assistance and counsel to support our mission. The MDA community recently mourned the loss of Victor Wright, a longtime board member, major contributor, and tireless champion for the families we serve.

Throughout 2023 we look forward to focusing on recognizing the countless individuals who work tirelessly

+BECOME A VOLUNTEER
Learn about volunteer opportunities at mda.org/volunteer.

Muscular Dystrophy Association is the #1 voluntary health organization in the United States for people living with muscular dystrophy, ALS, and related neuromuscular diseases. For more than 70 years, MDA has led the way in accelerating research, advancing care, and advocating for the support of our families. MDA's mission is to empower the people we serve to live longer, more independent lives.

EDITORIAL

Mindy Henderson

Director, Quest Editor-in-Chief
For editorial inquiries:
quest@mdaUSA.org

ADVERTISING

Elisa N. Beerbohm

Director, Marketing and Ad Sales
For print and digital advertising opportunities:
eebeerbohm@mdaUSA.org
MDAQuest.org/advertise

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2022 Bank of American Chicago Marathon



Volunteer committee members and mentors worked tirelessly to ensure the best experience for runners as they took on 26.2 miles for MDA.



2023: Year of the Volunteer

At MDA, we believe in the power of community and our approach aims for impacts greater than the sum of its parts. Volunteers, new and longstanding, are making a difference by working tirelessly to inspire and move communities at MDA Summer Camp, galas, golf outings, and other local and national events. That is why we have proclaimed 2023 The Year of the Volunteer. Create connections, foster relationships, and deepen MDA's impact within your community by volunteering your time and talents.

“Volunteering with the MDA awarded me the opportunity to work with and support an amazing cause. Knowing that my contributions and support are going to help those facing adversity is such a rewarding feeling.”

— Volunteer Committee Member

Annual Miles for MDA



Taunton High School DECA and Student Council MA members brought the community together to support MDA with food, fun, and disability awareness.

Beaniez Volunteers at MDA Summer Camp



Let's Play Program Manager, Kenny Small AKA “Beaniez,” felt the connection when he volunteered for a week at MDA Summer Camp.

41st Annual Millston Labor Day Softball Tournament



Sue Marcou and Wendi Wright, chair and co-chair, have dedicated their time to raising funds and awareness to support MDA Summer Camp.





What's New in Quest



Welcome to the first 2023 issue of Quest magazine! We have big plans for the year to bring you more of the information, tools, resources, and stories you need to live your life empowered. Starting with this issue, we have added a couple new columns: A Look Inside and Quest for Success.

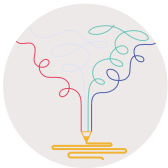
A Look Inside (page 7) is designed to peel back the curtain and give you a peek at what's going on within MDA, and help you understand more about what MDA is doing to serve you. And we have some fantastic personal stories, examples, and information about how individuals in our community have overcome obstacles and solved problems in order to achieve their version of success in our new Quest for Success column (page 10).

Don't worry, we will continue to bring you all the scientific and research information, features, and other articles you have become accustomed to, and we hope these new additions will enhance your toolkit.

We are looking forward to what lies ahead in 2023 and wish you and your loved ones a happy, healthy, productive, and successful year. Thank you for putting your trust in us to help you do that!

*Mindy Henderson, Director, Quest Editor-in-Chief
Muscular Dystrophy Association*

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DID YOU KNOW?

Have you listened to the Quest Podcast?

Our community is loving the thoughtful conversations podcast host and Quest editor-in-chief Mindy Henderson has with guests about issues affecting those with neuromuscular disease and other disabilities and those who love them. Don't miss the top 5 downloaded podcasts of the past year:

1. The Beginning: Receiving a Diagnosis (Episode 1)
2. Love & Marriage (Episode 12)
3. The ABCs of Accessible Travel (Episode 17)
4. Parenting (Episode 13)
5. Siblings Tell All (Episode 11)

Find Quest Podcast on your favorite podcast app or download these episodes at [MDAQuest.org/podcast](https://mdaquest.org/podcast).

Our library of Access Workshops is growing!

These self-paced online workshops are filled with presentations, activities, and resources to educate the neuromuscular disease community on overcoming barriers.

Don't miss any of our engaging workshops:

- Access To Employment**
- Access to Financial Independence**
- Access to Coverage: Approved Treatments**
- Access to Coverage: Insurance**
- Access to Coverage: Equipment & Assistive Devices**
- Access to Education: K-12**
- Access to Education: Higher Education**

View the complete list of workshops at mda.org/AccessWorkshops.



A Network of Expert Care

A deeper understanding of MDA's National Care Center network

Every year, MDA Care Centers and their expert clinicians serve more than 60,000 people living with neuromuscular diseases. Started in 1953 at a single clinic in New York, the Care Center network has grown to clinics at more than 150 top medical institutions across the country. As the largest network of multidisciplinary neuromuscular disease clinics across the United States, MDA Care Centers provide critical access to therapies and diagnostic testing.

To learn more about the impact and intricacies of Care Centers, Quest spoke with Marydeth Guerin, the senior director of the MDA Care Center network.

Why are the Care Centers a vital part of MDA's mission, and why are they essential to the community?

MDA Care Centers help break down some barriers to accessing care, helping our community locate experts familiar with their rare neuromuscular diagnoses. Your local neurologist may be a fantastic doctor, but they may not specialize in neuromuscular diseases. The Care Center network has helped create



Image: iStock.com/Tetiana Lazunova

a community of providers who are experts in these rare diseases. These providers can also contact one another for information, guidance, and peer-to-peer consultations.

The Care Centers' multidisciplinary approach is important, too. Research shows that a team of different healthcare professionals working together is especially beneficial for people with rare diseases and complex care needs. Bringing together a range of skills and knowledge improves the quality of care and provides patients and their families with more resources and support.

Data from some Care Centers is also part of MDA's neuroMuscular ObserVational Research (MOVR) Data Hub. At designated Care Centers, MOVR gathers data from patients who agree to share information. This large dataset can provide researchers with insights into how drugs and other treatments affect outcomes, how clinical trials could be designed better, and how neuromuscular disease affects people the same or differently. (Turn to page 42 to learn more about MOVR.)

The clinicians we partner with also help MDA progress our organizational mission. We can tap into this expert network to help inform some of our efforts and education, serve

The MDA Care Center network is a partnership with hospitals, medical centers, and healthcare institutions across the country.

+FIND A CARE CENTER

All MDA Care Center locations are listed at mda.org/CareCenters.



as educational speakers and chairs for MDA's Annual Clinical and Scientific Conference, steer advocacy initiatives, and provide other guidance.

Who can receive care at an MDA Care Center?

Anyone can go to a Care Center, but insurance coverage can vary from plan to plan. Each Care Center has a social worker on staff who can help you navigate insurance coverage and answer questions, including whether you may be able to seek in-network coverage due to a rare disease diagnosis from a Care Center that may otherwise be considered out-of-network.

At most Care Centers, you do not need a confirmed neuromuscular disease diagnosis before making an appointment,

and many provide diagnostic testing. However, each Care Center controls its individual operational procedures and may have set protocols within its broader hospital or practice. Some Care Centers may require that you have a confirmed neuromuscular diagnosis before being scheduled for a multidisciplinary clinic visit. Generally, such Care Centers do have clinicians who specialize in diagnosing these rare diseases and may encourage you to schedule an appointment for diagnostic testing separately first before being referred to the full multidisciplinary clinic. Contact the Care Center near you to find out if a confirmed diagnosis is a prerequisite and what their individual process is for scheduling a multidisciplinary Care Center visit. Find a list of locations at mda.org/CareCenters.

Do different Care Centers focus on specific diseases or age groups?

There are pediatric, adult, and ALS-designated Care Centers, among others.

The network also has Care Affiliates that do not meet the full criteria to be grant-funded MDA Care Centers but are still part of the affiliated network and have some neuromuscular expertise without a multidisciplinary team. That could be a single neurologist at a private practice who has neuromuscular expertise, for example.

Can I go to more than one Care Center?

Yes. An example of this may be traveling to see a Charcot-Marie-Tooth disease (CMT) expert every other year while also seeing a local provider at another Care Center in the interim. If you want other opinions or more convenient care — whatever the case may be — you can be seen at more than one Care Center.

Do you encounter any misconceptions about the Care Centers?

Yes, one misconception we sometimes hear is that MDA owns or operates all of the Care Center locations and that MDA employs the Care Center staff. Actually, the MDA Care Center network is a partnership with hospitals, medical centers, and healthcare institutions across the country.

MDA provides grant funding and designation awards to the sites that meet and stay in compliance with MDA Care Center criteria. The grant funding that we

MDA CARE CENTERS BY THE NUMBERS

MDA's National Care Center network:

Comprises clinics at **150+** of the nation's top medical institutions across the US



Offers **200+** appointment locations across the US

Conducts **90,000+** medical visits annually for individuals living with muscular dystrophy, ALS, and related neuromuscular diseases

Provides expert care for **60,000+** individuals annually who are living with neuromuscular diseases

Includes **2,400+** clinical providers across the network

Serves as a hub for neuromuscular research activity with **20,000+** individuals participating in clinical trials and natural history studies in 2019 alone

98% of MDA Care Centers provide access to therapies recently approved by the FDA, and more than **80%** of MDA Care Centers provide access to diagnostic testing.

THE MULTIDISCIPLINARY TEAM

Multidisciplinary care teams at MDA Care Centers can include a variety of healthcare professionals with expertise in neuromuscular diseases. At a minimum, each Care Center must have a core multidisciplinary team that includes:

- A director who is a physician
- A clinic coordinator (typically a registered nurse or nurse practitioner)
- A physical therapist
- A social worker

give the Care Centers does not cover the institution's total cost to host the Care Center, but it supports the multidisciplinary team and services to make specialized care available to patients.

Another misconception is that MDA can directly use grant funds for individual patient expenses. In order to maximize benefits and resources for the patient community as a whole, the grants help fund institutions that host MDA Care Centers and the multidisciplinary services available there. Our goal in standardizing how grant funding can be used is to help ensure that there is consistent access to high-quality, specialized care across the Care Center network, regardless of where you may live. Specific laws and regulations, such as the Stark Law and anti-kickback statute, factor into why we can't use the money for individual families' co-pays, deductibles, or other fees.

Do Care Centers offer telehealth options?

Yes. Telehealth options are becoming increasingly prominent, and many sites have adopted hybrid

telehealth models so individuals don't have to come in person for every visit. They might come in once a year to see the full team and receive all the services they need. But if they need to be seen every quarter, for example, they can use telehealth for other appointments and alleviate the travel and scheduling burdens. You can contact your MDA Care Center to find out more about telehealth options for your care. We also encourage you to speak with your provider about whether telehealth is an appropriate option for you, as this may vary depending on your unique healthcare needs and disease progression.

In some instances, a Care Center expert may be able to partner with your local healthcare provider to offer you telehealth services, particularly if travel or transportation are a barrier to your ability to access care. In some cases, the local provider can complete some of the physical assessments with the guidance of a neuromuscular disease expert from a Care Center. MDA Care Center providers are used to collaborating with a patient's outside medical team. They are dedicated to providing the highest quality of care and treatment for people with neuromuscular conditions. Talk with your Care Center provider to determine what approaches are most appropriate for you and what they are able to accommodate. [Q](#)



+GET SUPPORT

For help finding an MDA Care Center and other support and resources, contact the MDA Resource Center at **833-ASK-MDA1** or ResourceCenter@mdaUSA.org.



Words to Live By

Community members share tips and advice

Success looks different for everyone, and there are many different paths to independence and accomplishment. In our 2022 blog series Quest for Success, we profiled individuals from our community and shared how they got where they are today. We love sharing these success stories so much that we've decided to keep the series going in Quest magazine. Here, we've collected some of the best tips and advice shared in these blog posts over the past year.

Education and career

Pursuing education or a career can be a daunting journey for anyone. While there may be additional obstacles when you have a disability, there are also accommodations, modifications, and resources that make it possible for every person to use and share his or her valuable skillsets. One of the key things the individuals we interviewed shared was the power in identifying and believing in your own abilities.



REAL LIVES

Read all the Quest for Success stories at MDAQuest.org/tag/quest-for-success.

“I believe that it’s so important to take what you can do and really bring 110%. ... Be honest about what you need help with and go from there with what you can do.”

— *Jax Cowles, 34, merchandising coordinator with spinal muscular atrophy (SMA)*



“Each person’s story is a reminder that you can achieve whatever goal you set for yourself — you are able.”

— *Corinne Grgas, 31, nurse practitioner with GNE myopathy*



“Don’t allow someone to tell you that you can’t live a productive, value-added life. ... If you know that you want to be independent, to work, to get married, to have a house ... find ways. It may not be easy, but it is possible.”

— *Ira Walker, 37, HR professional with SMA*



Community support

Community is an incredible resource — both the neuro-muscular disease community and the broader disability community can offer support, guidance, resources, tangible advice, and a sense of belonging.

“There is a national community of peers and advocates who long for the same thing. ... There are a lot of us in our MDA community and beyond who are sitting alongside you and will help to show you the way.”

— *Chris Rosa, 55, nonprofit president and CEO with limb-girdle muscular dystrophy (LGMD)*



“Connecting with others, either in person or on disability advocacy social media platforms, and learning from their lived experiences is an invaluable resource. We are all unique individuals with unique experiences, but it’s important to realize people have done this before and there are ways to figure it out.”

— *Brian Chao, 32, nonprofit CFO with Ulrich congenital muscular dystrophy (CMD)*



Words of advice

Each interview in our Quest for Success series elicited heartfelt words of advice. These individuals shared their tips for thinking beyond the obstacles life throws at us.

“It’s hard in the beginning to realize that you can overcome your disability because it just seems like that is what your life is — but your disability doesn’t define you at all. ... Think about what you want to do in life and go for it. Go out there and do it and make your own story. You can become an inspiration for other people.”

— *Maanav Gupta, 18, college student, podcast host, and aspiring sportscaster with Duchenne muscular dystrophy (DMD)*



“Every one of us is able to help others, whether we are mobile or otherwise equipped, through our words, our actions, [and] living our best life with hope and optimism.”

— *Jason Morgan, 33, county commissioner with Becker muscular dystrophy (BMD)*

“Don’t get too caught up in what the world tells you about yourself. That can be disillusioning to a person with a disability. Know yourself well and don’t allow anyone else to tell you who you are.”

— *Tana Zwart, 37, media manager with facioscapulohumeral muscular dystrophy (FSHD)*





VYVGART[®]
(efgartigimod alfa-fcab)

Injection for Intravenous Use
400 mg/20 mL vial

Picture your life in motion

GENERALIZED MYASTHENIA GRAVIS

*doesn't get to steal
this moment*

What is VYVGART[®] (efgartigimod alfa-fcab)?

VYVGART is a prescription medicine used to treat a condition called generalized myasthenia gravis, which causes muscles to tire and weaken easily throughout the body, in adults who are positive for antibodies directed toward a protein called acetylcholine receptor (anti-AChR antibody positive).

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about VYVGART?

VYVGART may cause serious side effects, including:

- **Infection.** VYVGART may increase the risk of infection. In a clinical study, the most common infections were urinary tract and respiratory tract infections. More patients on VYVGART vs placebo had below normal levels for white blood cell counts, lymphocyte counts, and neutrophil counts. The majority of infections and blood side effects were mild to moderate in severity. Your health care provider should check you for infections before starting treatment, during treatment, and after treatment with VYVGART. Tell your health care provider if you have any history of infections. Tell your health care provider right away if you have signs or symptoms of an infection during

treatment with VYVGART such as fever, chills, frequent and/or painful urination, cough, pain and blockage of nasal passages/sinus, wheezing, shortness of breath, fatigue, sore throat, excess phlegm, nasal discharge, back pain, and/or chest pain.

- **Undesirable immune reactions (hypersensitivity reactions).**

VYVGART can cause the immune system to have undesirable reactions such as rashes, swelling under the skin, and shortness of breath. In clinical studies, the reactions were mild or moderate and occurred within 1 hour to 3 weeks of administration, and the reactions did not lead to VYVGART discontinuation. Your health

VYVGART is a first-of-its-kind, FDA-approved treatment for adults with anti-AChR antibody positive generalized myasthenia gravis (gMG)

AChR=acetylcholine receptor
Visit [VYVGART.com/glossary](https://www.vyvgart.com/glossary) for a glossary of terms.

When added to their current gMG treatment, VYVGART helped clinical trial participants with anti-AChR antibody positive gMG achieve:

Improved daily abilities

68% (44 of 65) of participants on VYVGART achieved significant improvement in their ability to perform daily activities*

Reduced muscle weakness

63% (41 of 65) of participants on VYVGART achieved a significant reduction in muscle weakness†

*Improvement maintained for 4 or more weeks was measured by a decrease of 2 or more points on the Myasthenia Gravis Activities of Daily Living (MG-ADL) scale, with the first reduction occurring no later than 1 week after the last infusion of treatment cycle 1. The MG-ADL scale assesses the impact of gMG on daily functions by measuring 8 signs or symptoms that are commonly affected in gMG. Each item is measured on a 4-point scale, where a score of 0 represents normal function and a score of 3 represents the loss of ability to perform that function. Total scores range from 0 to 24 points, with a higher score showing more severe gMG.

†Improvement maintained for 4 or more weeks was measured by a decrease of 3 or more points on the Quantitative Myasthenia Gravis (QMG) scale, with the first reduction occurring no later than 1 week after the last infusion of treatment cycle 1. The QMG scale assesses muscle weakness in gMG based on 13 items. Each item is assessed on a 4-point scale, where a score of 0 represents no muscle weakness and a score of 3 represents severe muscle weakness. Total scores range from 0 to 39, with a higher score meaning muscle weakness is more severe.

➔ Talk to your neurologist and visit [VYVGART.com](https://www.vyvgart.com) or call **1-833-VYVGART (1-833-898-4278)**

care provider should monitor you during and after treatment and discontinue VYVGART if needed. Tell your health care provider immediately about any undesirable reactions.

Before taking VYVGART, tell your health care provider about all of your medical conditions, including if you:

- Have a history of infection or you think you have an infection
- Have received or are scheduled to receive a vaccine (immunization). Discuss with your health care provider whether you need to receive age-appropriate immunizations before initiation of a new treatment cycle with VYVGART. The use of vaccines

during VYVGART treatment has not been studied, and the safety with live or live-attenuated vaccines is unknown. Administration of live or live-attenuated vaccines is not recommended during treatment with VYVGART.

- Are pregnant or plan to become pregnant and are breastfeeding or plan to breastfeed.

Tell your health care provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

What are the common side effects of VYVGART?

The most common side effects of VYVGART are respiratory tract

infection, headache, and urinary tract infection.

These are not all the possible side effects of VYVGART. Call your doctor for medical advice about side effects. You may report side effects to the US Food and Drug Administration at 1-800-FDA-1088.

Please see the full Prescribing Information for VYVGART and talk to your doctor.



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US-VYV-22-00075 V2 06/2022

<p>Important Information about VYVGART® (efgartigimod alfa-fcab); Rx only.</p> <p>The risk information provided here is not comprehensive. To learn more, talk about VYVGART with your health care provider. The US Food and Drug Administration (FDA)-approved product labeling can be found by visiting www.vyvgart.com/pi or calling 1-833-VYVGART (1-833-898-4278).</p>	<p>infections. Tell your health care provider right away if you have signs or symptoms of an infection during treatment with VYVGART such as fever, chills, frequent and/or painful urination, cough, pain and blockage of nasal passages/sinus, wheezing, shortness of breath, fatigue, sore throat, excess phlegm, nasal discharge, back pain, and/or chest pain.</p>	<p>What are the common side effects of VYVGART?</p> <p>The most common side effects of VYVGART are respiratory tract infection, headache, and urinary tract infection. Other side effects included a tingling (pins and needles) sensation and muscle pain.</p> <p>These are not all the possible side effects of VYVGART. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.</p>
<p>What is VYVGART?</p> <p>VYVGART is a prescription medicine used to treat a condition called generalized myasthenia gravis, which causes muscles to tire and weaken easily throughout the body, in adults who are positive for antibodies directed toward a protein called acetylcholine receptor (anti-AChR antibody positive).</p>	<p>• Undesirable immune reactions (hypersensitivity reactions). VYVGART can cause the immune system to have undesirable reactions such as rashes, swelling under the skin, and shortness of breath. In clinical studies, the reactions were mild or moderate and occurred within 1 hour to 3 weeks of administration, and the reactions did not lead to VYVGART discontinuation. Your health care provider should monitor you during and after treatment and discontinue VYVGART if needed. Tell your health care provider immediately about any undesirable reactions.</p>	<p>What are the effects of VYVGART on other drugs?</p> <p>The use of VYVGART with medications that bind to a receptor called the human neonatal Fc receptor (FcRn) may reduce the effectiveness of these medications. Tell your health care provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.</p>
<p>What is the most important information I should know about VYVGART?</p> <p>VYVGART may cause serious side effects, including:</p> <ul style="list-style-type: none"> • Infection. VYVGART may increase the risk of infection. In a clinical study, the most common infections were urinary tract and respiratory tract infections. Patients on VYVGART vs placebo had below normal levels for white blood cell counts, lymphocyte counts, and neutrophil counts. The majority of infections and blood side effects were mild to moderate in severity. Your health care provider should check you for infections before starting treatment, during treatment, and after treatment with VYVGART. Tell your health care provider if you have any history of 	<p>Immunization</p> <p>Discuss with your health care provider if you have received or are scheduled to receive a vaccine (immunization) and if you need to receive age-appropriate immunizations before initiation of a new treatment cycle with VYVGART. The use of vaccines during VYVGART treatment has not been studied, and the safety with live or live-attenuated vaccines is unknown. Administration of live or live-attenuated vaccines is not recommended during treatment with VYVGART.</p>	<p>What information should I know about VYVGART and pregnancy and breastfeeding?</p> <p>There are no available data on the use of VYVGART during pregnancy and breastfeeding. Talk to your doctor if you are pregnant or plan to become pregnant and are breastfeeding or plan to breastfeed.</p>
		<p>Can VYVGART be used in children?</p> <p>The safety and efficacy in children (pediatric patients) have not been established.</p>



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Duchenne muscular dystrophy (DMD)

Brain Development Study

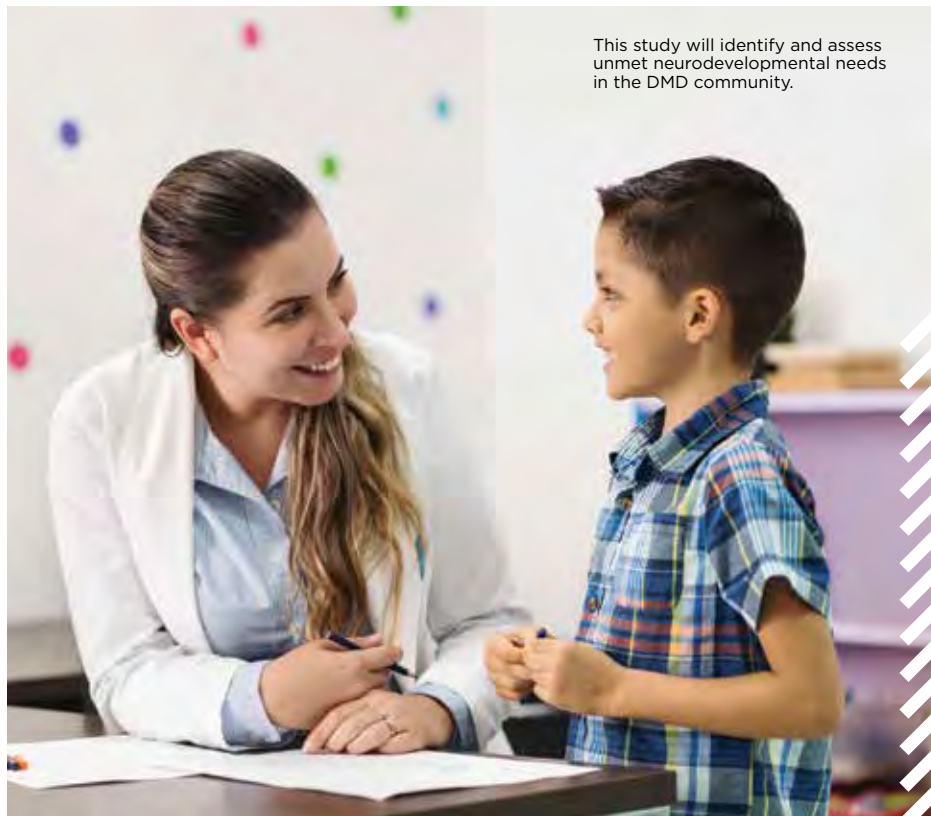
Researchers at Virginia Commonwealth University are seeking boys living with DMD and their biological mothers to participate in a study of neurodevelopmental (brain development) needs resulting from DMD.

This study does not involve a new intervention. Enrolled participants will be asked to undergo baseline neurological assessments and attend three clinic visits (at two to four weeks, one year, and 18 months). At these visits, participants may be asked to complete the NIH Toolbox Cognition Measures testing, take part in other cognitive assessments and behavior questionnaires, and undergo an optional MRI.

All personally identifiable information in the data will be removed to protect identities. The total duration of the study for participants will be 18 months.

To be eligible, boys must have a confirmed diagnosis of DMD and meet additional criteria. Mothers must be 18 years or older and the biological mother of the child with DMD. Individuals may not be eligible to participate if they are unable to use an iPad or their caregiver is unable to give consent. Travel support may be available.

To view more information about this trial, visit ClinicalTrials.gov and enter NCT05280730 in the “Other terms” search box.



This study will identify and assess unmet neurodevelopmental needs in the DMD community.

CLINICAL TRIAL TERMS TO KNOW

Double-blind: Neither researchers nor participants know which participants are taking the drug or placebo.

Multiarm: Comparing several different experimental treatments against a common control group within a single study.

Multicenter: The trial is completed at more than one site.

Randomized: Participants are assigned at random to groups taking the drug or placebo.

Drug Trial Enrolling Children

Biopharmaceutical company Edgewise Therapeutics has started the phase 2 LYNX clinical trial of its experimental drug EDG-5506 in children with DMD. The placebo-controlled trial will examine the drug's safety, pharmacokinetics (how a drug is absorbed, distributed, and metabolized in the body), and effect on biomarkers of muscle damage. The study will also explore changes in functional measures, such as the North Star Ambulatory Assessment (NSAA) and self-reported and caregiver-reported outcomes.

EDG-5506 is an investigational oral drug designed to limit the muscle damage caused by the absence or loss of functional dystrophin

protein in diseases such as DMD and Becker muscular dystrophy (BMD).

Participants will take three doses of EDG-5506 over 12 weeks. Afterward, LYNX participants will continue taking open-label EDG-5506 for an additional nine months to gain more insights into safety and functional measures.

Approximately 27 children with DMD ages 4 to 9 on a stable corticosteroid regimen are expected to participate. A research center in Atlanta is the first site to begin enrolling. Up to 12 investigative sites across the United States are expected to participate in the trial.



To learn more about the LYNX trial, including inclusion and exclusion criteria, visit ClinicalTrials.gov and enter NCT05540860 in the "Other terms" search box.

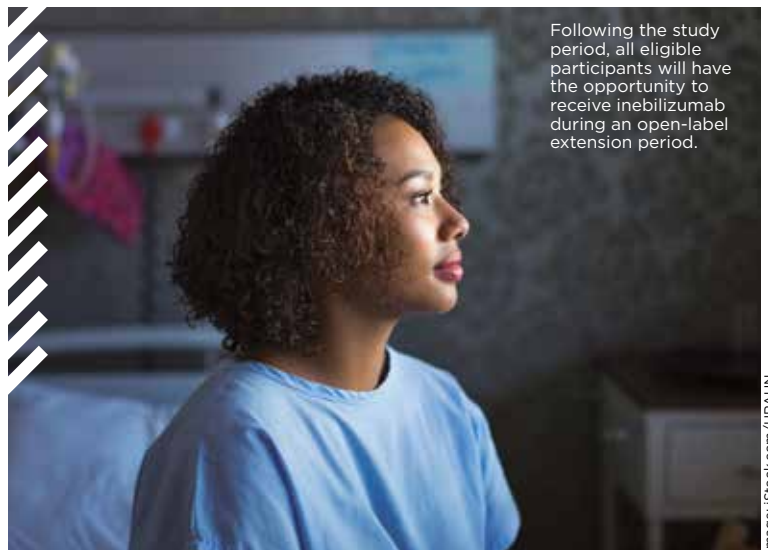
Myasthenia gravis (MG)

Phase 3 Study Seeks Participants

Researchers at Horizon Therapeutics are seeking adults living with MG to participate in a phase 3 clinical trial to evaluate inebilizumab's safety and efficacy for treating MG. Inebilizumab is being assessed for its ability to reduce the autoimmune response that causes MG, thereby potentially reducing disease symptoms and improving function in people with the disease.

Participants will be randomly assigned to take either inebilizumab or an inactive placebo control over the course of the study. The total trial duration for each participant will be about 2 years, with 15 to 19 clinic visits depending on their MG subtype (anti-MuSK Ab+ or anti-AChR Ab+).

The drug/placebo will be administered by intravenous (in the vein) infusion. The effects of inebilizumab will be evaluated via multiple blood tests and disease assessments, including quality-of-life questionnaires.



Following the study period, all eligible participants will have the opportunity to receive inebilizumab during an open-label extension period.

Image: iStock.com/HRAUN

To be eligible, individuals must be at least 18 years old, have multiple muscle groups affected by MG, and have an MG diagnosis with positive test for anti-AChR or anti-MuSK antibodies, which can be tested at screening. Travel support may be available.

To learn more about the study or how to participate, visit MyastheniaGravisMintStudy.com or contact Horizon Therapeutics at 866-479-6742 or ClinicalTrials@HorizonTherapeutics.com.



The drug sponsor formally asks the FDA to approve a drug for marketing in the United States by submitting an NDA.

FDA Accepts Application for Zilucoplan

The US Food and Drug Administration (FDA) has accepted biopharmaceutical company UCB's new drug application (NDA) for zilucoplan. UCB seeks approval of zilucoplan for the treatment of generalized myasthenia gravis (gMG) in adult patients who are acetylcholine receptor antibody positive (anti-AChR Ab+).

Zilucoplan is a subcutaneous (under the skin), self-administered drug that may improve muscle function by blocking the autoimmune reaction that destroys the junction between nerves and muscles in people living with gMG.

The FDA's approval is based on results from the pivotal phase 3 RAISE study, a multicenter, randomized, double-blind, placebo-controlled study to confirm the efficacy, safety, and tolerability of zilucoplan in patients with anti-AChR Ab+ gMG. In the study, 174 adult participants were randomly assigned to receive daily doses of zilucoplan or placebo. Results showed that treatment with zilucoplan was generally safe, well-tolerated, and resulted in significant improvements in key MG-specific outcomes compared to placebo. The most common adverse events were injection site bruising, headache, and diarrhea.

For more on the RAISE study, visit [ClinicalTrials.gov](https://www.clinicaltrials.gov) and enter NCT04115293 in the "Other terms" search box.

Drug's New Form Gets Priority Review

The FDA has accepted for priority review Argenx's Biologics License Application (BLA) for a subcutaneously (under the skin) administered form of efgartigimod, called SC efgartigimod, for the treatment of adults with gMG. A BLA is one way to obtain full FDA approval and marketing authorization for a biological product. The application has been granted a Prescription Drug User Fee Act (PDUFA) target action date (FDA decision date) of March 20, 2023.

Efgartigimod is designed to reduce levels of harmful immunoglobulin G (IgG) antibodies circulating in the body by binding to the neonatal Fc receptor.

The BLA submission is based on data from the phase 3 ADAPT-SC study that showed similar IgG reduction after 29 days

of treatment with SC efgartigimod compared to intravenous (in the vein) administration of efgartigimod (brand name Vyvgart) in adult patients with gMG. The majority of enrolled participants were positive for acetylcholine receptor antibodies (anti-AChR Ab+), but the trial also included patients with no detectible AChR antibodies.

SC efgartigimod was generally well-tolerated; the most frequent adverse event was injection site reactions. After completing ADAPT-SC, 95% of participants entered ADAPT-SC+, a three-year extension study evaluating its long-term safety and tolerability.

Visit [argenx.com](https://www.argenx.com) for more information.

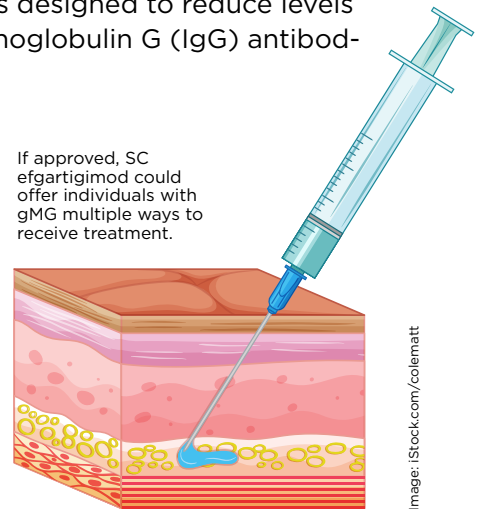
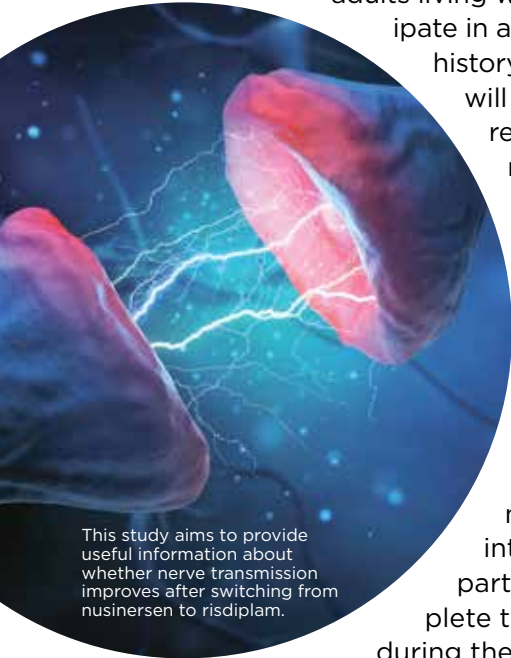


Image: iStock.com/colehatt

Spinal muscular atrophy (SMA)

Natural History Study Seeks Participants

Researchers at Ohio State University are seeking adults living with SMA to participate in a 12-month natural history study. The study will look at changes/



This study aims to provide useful information about whether nerve transmission improves after switching from nusinersen to risdiplam.

responses to communication between the nerves and muscles in individuals who received long-term treatment with nusinersen and then transitioned to treatment with risdiplam.

This study does not involve a new intervention. Enrolled participants must complete three doctor visits during the study. Participants

will be evaluated using repetitive nerve stimulation (RNS) of the spinal accessory nerve, nerve transmission assessment and testing, and various clinical function tests and measures.

To be eligible, individuals must:

- Sign an informed consent form
- Be aged 18–65 at the time of signing informed consent form
- Have documented genetic confirmation of 5q SMA
- Meet additional criteria

Travel support may be available for study participants.

To learn more, visit ClinicalTrials.gov and enter NCT05219487 in the “Other terms” search box. To inquire about participation, contact study coordinator Shemikka Young at **614-685-8661** or shemikka.young@OSUMC.edu.

MDA research in action

MDA Awards 70 New Grants

In September, MDA awarded 70 new grants totaling over \$17 million for neuromuscular disease research. The newly funded projects aim to advance research discoveries and new therapy development in numerous diseases, including amyotrophic lateral sclerosis (ALS), Charcot-Marie-Tooth disease (CMT), Duchenne muscular dystrophy (DMD), facioscapulohumeral muscular dystrophy (FSHD), limb-girdle muscular dystrophy (LGMD), spinal muscular atrophy (SMA), myasthenia gravis (MG), and myotonic dystrophy (DM).

“MDA continues to fund the most innovative research that will lead to cures for a range of

neuromuscular diseases,” says Sharon Hesterlee, PhD, MDA’s chief research officer. “We have already seen our investment pay off with the first effective neuromuscular disease therapies, and these grantees are pushing the envelope even further in diseases once thought incurable.”

MDA’s model of funding research across many neuromuscular diseases means that findings from



MDA has invested more than \$1 billion in research over the last 70 years, advancing treatments for neuromuscular diseases and helping individuals live longer, more independent lives.

Image: iStock.com/K_E_N_sorbetto

one disease often enable progress in others, maximizing the speed of progress.

Here are some of the grant highlights:

- **Jeffrey Statland**, of the University of Kansas Medical Center; **Nicholas Johnson**, of Virginia Commonwealth University; and **Rabi Tawil** and **Charles Thornton**, of the University of Rochester, were jointly awarded the Clinical Research Network Grant of \$1.5M to support their clinical trial network for FSHD, LGMD, and DM. The funding will support the consolidation of project management and oversight from 15 core centers in the United States under a single umbrella of the MD Clinical Trial Research Network, aimed at clinical trial readiness for these diseases.
- **Michael Shy**, of the University of Iowa, was awarded the Research Infrastructure Grant of \$439,250 to support the Inherited Neuropathy Consortium (INC) to develop the infrastructure (advanced tools) necessary to evaluate therapies for patients with CMT.
- **Forum Kamdar**, of the Regents of the University of Minnesota, was awarded a Clinical Research Network Grant of \$198,000 for her project to combine data from large muscular dystrophy and advanced heart failure centers to assess cardiac care of patients and address gaps in the current cardiac care of patients.
- Other grants will fund pivotal research to support the development of genetic therapies in DMD, mitochondrial diseases, and LAMA2 muscular dystrophy. These projects include: “Utrophin Genome Editing for Duchenne’s Muscular Dystrophy (DMD) Therapy,” by Tejvir Khurana, MD, PhD, at the University of Pennsylvania; “Developing Gene Therapy Approaches for mtDNA Deletions,” by Carlos Moraes, PhD, at the University of Miami; and “Linker-based Gene Therapy of LAMA2-related Muscular Dystrophy Using AAVMYO,” by Dr. Markus Ruegg at the University of Basel.

For a complete list of individual awards for this grant cycle, visit mda.org/gaag.

NEW

Introducing the Quest Media Adaptive Lifestyle Website

Quest Media

MDAQuest.org is MDA’s newly launched adaptive website offering the latest in magazine, blog and podcast content.



Understanding Congenital Muscular Dystrophy

A Q&A with Dean Burkin, PhD



Congenital muscular dystrophy (CMD) is a group of progressive neuromuscular diseases that affect individuals at or shortly after birth.

Image: iStock.com/Narongrit-Sritana

Congenital muscular dystrophy (CMD) is a group of progressive neuromuscular diseases that affect individuals at or shortly after birth. Sometimes referred to as having “floppy baby” or “floppy infant syndrome,” newborns with CMD have muscle weakness. In addition, those with CMD often develop joint contractures — a stiffening of the joints. As the disease progresses, people living with CMD often experience reduced mobility, breathing difficulty, respiratory infections, and shorter lifespans.

To learn more about CMD, we spoke with Dean Burkin, PhD, professor of pharmacology and pediatrics at the University of Nevada, Reno, School of Medicine.



Dean Burkin, PhD

What conditions fall within the CMD category?

So far, about 30 different CMDs have been identified. They are caused by mutations in genes that encode proteins located in the extracellular matrix (a structure of connective tissues that plays an important role in cell growth, cell movement, and other functions), receptors on the surface of muscle cells, or proteins within cells including the nucleus (the component within a cell that contains the chromosomes). CMDs include defects in:

- Extracellular matrix proteins, including laminin (laminin- α 2-related CMD) or collagen (collagen VI-related CMD or collagen XII-related CMD)

- Cell receptors integrin $\alpha 7$ and $\alpha 9$ (ITGA7-related CMD or ITGA9-related CMD), and α -dystroglycan (dystroglycanopathies and forms of limb-girdle muscular dystrophy)
- Intracellular proteins (SEPN1-related myopathies and Titin myopathy)
- Nuclear proteins lamin A/C (laminopathy or LMNA-related CMD)

What does this group of diseases have in common?

Advances in whole genome DNA sequencing, which reveals an individual's entire genetic code, have led to the identification of numerous genes that, when defective, cause CMD. Although disease presentation in individuals can be very different, the CMDs can be grouped broadly according to the location of the defective protein.

How does CMD affect the people who live with it?

Children with CMD often fail to meet expected developmental milestones. Individuals with CMD frequently lose or never develop the ability to walk and require mobility assistance at a young age. They often experience weakened respiratory muscles and may require ventilator assistance to breathe. Respiratory infections are common in people affected by CMD. Some forms of CMD negatively impact heart function. CMD often causes difficulty swallowing and weight gain, which can lead to the need for special diets. CMD may also affect neurological, gastrointestinal, cardiovascular, pulmonary, and musculoskeletal systems.

What is the current standard of care?

The standard of clinical care is dependent on the type of CMD and often requires a team of caregivers and providers. Care is currently aimed at managing symptoms. This may include the use of ventilators for breathing problems, antibiotics to treat respiratory infections, mobility devices, monitoring heart function, physical therapy, speech therapy, and/or surgical interventions to correct severe spinal curvature.

Have there been any recent advances in treatment?

A phase 1 clinical trial (the CALLISTO trial) with a small-molecule drug called Omigapil from Santhera Pharmaceuticals was completed for LAMA2-related CMD and collagen VI-related CMD. Omigapil acts to prevent muscle loss, and the study indicated it was safe and well tolerated. The drug has yet to move to phase 2 clinical trials, but this trial has provided information on the design of future clinical trials and the selection of potential outcome measures for these CMDs.

Are there any other promising therapies on the horizon?

There are many therapies being studied that may have applications across the CMDs. Most treatment strategies for the CMDs are still in the pre-clinical (animal model) testing stage.

Exciting potential therapies for the CMDs include gene therapy, in which the target gene is small and can be packaged into current viral delivery systems.

Another is gene editing, using a strategy called CRISPR-Cas9. This holds the promise of personalized medicine to correct individual genetic defects and restore gene function. Stem cell therapies can be used to promote muscle repair or for use in gene therapy/editing. Exon-skipping and antisense technology could inhibit defective gene expression and is an exciting approach for many CMDs. Protein replacement therapy has shown advances for LAMA2-related CMD and aims to deliver the missing or defective protein to affected tissues. Designer linker proteins, which are hybrid molecules designed to stabilize muscle cells, are also under development. Finally, small molecules and FDA-approved medicines developed for other diseases could prove to slow disease progression or prevent muscle cell damage for many CMDs. [Q](#)

There are many therapies being studied that may have applications across the CMDs.



DISEASES A-Z

Browse our library of disease-specific content at [MDAQuest.org/diseases-a-z](https://mdaquest.org/diseases-a-z).

Recent treatment research advances include a clinical trial for Omigapil, which acts to prevent muscle loss in some types of CMD.



Image: iStock.com/Altoclassic

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ALS=amyotrophic lateral sclerosis.

INDICATION

RADICAVA (edaravone) and RADICAVA ORS (edaravone) are indicated for the treatment of amyotrophic lateral sclerosis (ALS).

IMPORTANT SAFETY INFORMATION

Do not receive RADICAVA or RADICAVA ORS if you are allergic to edaravone or any of the ingredients in RADICAVA and RADICAVA ORS.

Before you take RADICAVA or RADICAVA ORS, tell your healthcare provider about all of your medical conditions, including if you:

- have asthma.
- are allergic to other medicines.



Not actual product size.

- are pregnant or plan to become pregnant. It is not known if RADICAVA or RADICAVA ORS will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if RADICAVA or RADICAVA ORS passes into your breastmilk. You and your healthcare provider should decide if you will receive RADICAVA or RADICAVA ORS or breastfeed.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

RADICAVA ORS[®]: Oral Formulation of a Proven Treatment for ALS



RADICAVA ORS[®] helps **slow the loss of physical function** in patients with ALS. Results may vary.



The 5 mL dose may take **only a few minutes to administer** on treatment days.^a

- RADICAVA ORS[®] should be taken in the morning on an empty stomach after overnight fasting. No food or drink should be consumed (except water) for 1 hour after administration. If you have any dietary concerns or are unable to fast for 8 hours, talk to your doctor

The most common side effects of RADICAVA[®] (edaravone) and RADICAVA ORS[®] include bruising (contusion), problems walking (gait disturbance), and headache. These are not all the possible side effects of RADICAVA[®] and RADICAVA ORS[®]. Fatigue was also reported for RADICAVA ORS[®].

- 5.9% of patients discontinued RADICAVA ORS[®] because of side effects in a 24-week safety study in 185 patients with ALS

^aThe initial treatment cycle starts with daily dosing of RADICAVA ORS[®] for 14 days followed by a 14-day drug-free period. Subsequent treatment cycles include daily dosing 10 out of 14 days followed by a 14-day drug-free period.



Talk to your doctor today about RADICAVA ORS[®] and view informative videos at [RadicavaORSVideos.com](https://www.RadicavaORSVideos.com).

Contact a **Resource Specialist** who is a go-to resource in the **JourneyMate Support Program[™]** for general information about ALS and RADICAVA ORS[®]. Call toll-free 1-855-4-JRNY-M8 (1-855-457-6968).

IMPORTANT SAFETY INFORMATION

(continued)

What are the possible side effects of RADICAVA and RADICAVA ORS?

RADICAVA and RADICAVA ORS may cause serious side effects, including hypersensitivity (allergic) reactions and sulfite allergic reactions.

- Hypersensitivity reactions have happened in people receiving RADICAVA or taking RADICAVA ORS and can happen after your medicine has been given.
- RADICAVA and RADICAVA ORS contain sodium bisulfite, a sulfite that may cause a type of allergic reaction that can be serious and life-threatening. Sodium bisulfite can also cause less severe asthma episodes in certain people. Sulfite sensitivity can happen more often in people who have asthma than in people who do not have asthma.

- Tell your healthcare provider right away or go to the nearest emergency room if you have any of the following symptoms: hives; swelling of the lips, tongue, or face; fainting; breathing problems; wheezing; trouble swallowing; dizziness; itching; or an asthma attack (in people with asthma).

Your healthcare provider will monitor you during treatment to watch for signs and symptoms of all the serious side effects and allergic reactions.

The most common side effects include bruising (contusion), problems walking (gait disturbance), and headache.

These are not all the possible side effects of RADICAVA or RADICAVA ORS. **Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088. You may also report side effects to www.fda.gov/medwatch or Mitsubishi Tanabe Pharma America, Inc. at 1-888-292-0058.**

Please see accompanying Patient Information and full Prescribing Information available at [RadicavaORS.com](https://www.RadicavaORS.com).



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Radicava ORS[®]
(edaravone) Oral Suspension
105mg/5mL

PATIENT INFORMATION

RADICAVA (ra di kā vah)
(edaravone) injection
for intravenous use
RADICAVA ORS (ra di kā vah o r s)
(edaravone) oral suspension

What are RADICAVA and RADICAVA ORS?

RADICAVA and RADICAVA ORS are prescription medicines used to treat people with amyotrophic lateral sclerosis (ALS). It is not known if RADICAVA or RADICAVA ORS are safe and effective in children.

Do not receive RADICAVA or RADICAVA ORS if you are allergic to edaravone or any of the ingredients in RADICAVA and RADICAVA ORS. See the end of this leaflet for a complete list of ingredients in RADICAVA and RADICAVA ORS.

Before you take RADICAVA or RADICAVA ORS, tell your healthcare provider about all of your medical conditions, including if you:

- have asthma.
- are allergic to other medicines.
- are pregnant or plan to become pregnant. It is not known if RADICAVA or RADICAVA ORS will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if RADICAVA or RADICAVA ORS passes into your breastmilk. You and your healthcare provider should decide if you will receive RADICAVA or RADICAVA ORS or breastfeed.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

How will I receive RADICAVA?

- You will be prescribed RADICAVA by a healthcare provider and told how often you will receive RADICAVA.
- RADICAVA will be given by intravenous (IV) infusion into your vein.
- It takes about 1 hour to receive the full dose of RADICAVA.
- Your healthcare provider will monitor you closely during your treatment with RADICAVA.

How will I take RADICAVA ORS?

- See the detailed **Instructions for Use** on how to take RADICAVA ORS at the end of this Patient Information leaflet.
- You will be prescribed RADICAVA ORS by a healthcare provider and told how often you will take RADICAVA ORS.
- RADICAVA ORS is to be taken by mouth or by using a feeding tube.
- RADICAVA ORS should be taken in the morning on an empty stomach. You should stop eating at bedtime.
 - **Do not** eat or drink anything 8 hours before each dose of RADICAVA ORS if you eat a high-fat meal.
 - **Do not** eat or drink anything 4 hours before each dose of RADICAVA ORS if you eat a low-fat meal.
 - **Do not** eat or drink anything 2 hours before each dose of RADICAVA ORS if you take a calorie supplement.
- You should wait at least 1 hour after taking your medicine before eating or drinking anything except water.

What are the possible side effects of RADICAVA and RADICAVA ORS?

RADICAVA and RADICAVA ORS may cause serious side effects including:

1. Hypersensitivity (allergic) reactions. Hypersensitivity reactions have happened in people receiving RADICAVA or taking RADICAVA ORS and can happen after your medicine has been given. Tell your healthcare provider right away or go to the nearest emergency room if you have any of the following symptoms:

- hives
- breathing problems
- itching
- swelling of the lips, tongue, face
- dizziness
- wheezing
- fainting

2. Sulfite allergic reactions. RADICAVA and RADICAVA ORS contain sodium bisulfite, a sulfite that may cause a type of allergic reaction that can be serious and life-threatening. Sodium bisulfite can also cause less severe allergic reactions, for example, asthma episodes, in certain people. Sulfite sensitivity can happen more often in people who have asthma than in people who do not have asthma. Tell your healthcare provider right away or go to the nearest emergency room if you have any of the following symptoms:

- hives
- trouble breathing or swallowing
- itching
- swelling of the lips, tongue, face
- dizziness
- asthma attack (in people with asthma)
- wheezing
- fainting

Your healthcare provider will monitor you during treatment to watch for signs and symptoms of all the serious side effects and allergic reactions.

The most common side effects of RADICAVA and RADICAVA ORS include bruising (contusion), problems walking (gait disturbance), and headache.

These are not all the possible side effects of RADICAVA and RADICAVA ORS. For more information, ask your healthcare provider or pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

You may also report side effects to www.fda.gov/medwatch or Mitsubishi Tanabe Pharma America, Inc. at 1-888-292-0058.

What are the ingredients in RADICAVA and RADICAVA ORS?

Active ingredient: edaravone

RADICAVA Inactive ingredients: L-cysteine hydrochloride hydrate, sodium bisulfite, sodium chloride, phosphoric acid, and sodium hydroxide.

RADICAVA ORS Inactive ingredients: L-cysteine hydrochloride hydrate, polyvinyl alcohol, simethicone emulsion, sodium bisulfite, sorbitol, and xanthan gum. Phosphoric acid and sodium hydroxide are added to adjust to pH 4.

Marketed and distributed by: Mitsubishi Tanabe Pharma America, Inc., a US subsidiary of Mitsubishi Tanabe Pharma Corporation, 525 Washington Blvd., Suite 400, Jersey City, NJ 07310

For more information, go to www.Radicava.com or call 1-888-292-0058.



In Transit We Trust?

For people with disabilities, public transit can be a ticket to independence — or an exercise in inequality

BY MATT ALDERTON

Image: iStock.com/masha_tace



Elizabeth Serrano, who lives with spinal muscular atrophy (SMA), gets around well in her power wheelchair. But when she needs to go farther than her wheelchair can take her, it can be a struggle.

Her parents used to drive her to work and social activities in her accessible van. “I still have a van, but my father passed away and my mother can’t drive anymore,” says Elizabeth, 62, of Levittown, Toa Baja, Puerto Rico. “Now I have to pay someone to drive me around.”

But private chauffeurs are expensive. And since the start of the COVID-19 pandemic in 2020, they’ve been difficult to find. Increasingly, Elizabeth relies on public transit. In doing so, she’s discovered an inconsistent system that expands mobility in some respects but limits it in others.

“It’s OK,” she says. “But it could be a lot better.”

Like Elizabeth, most people with limited mobility would prefer to get around in their own accessible vehicle. But these aren’t available to everyone. If you don’t have one, it’s possible to get where you want to go, when you want to go there by familiarizing yourself with public transit options — and by lobbying for system improvements where they are needed.

Options and obstacles

Title II of the Americans with Disabilities Act (ADA) is supposed to protect people with disabilities from discrimination by guaranteeing equal access to public accommodations, including public transit systems.



Elizabeth Serrano



TRANSIT RESOURCES

The following organizations can help you learn more about ADA requirements in public transportation and how to advocate for accessibility improvements.

↗ **ADA National Network:** Provides information, guidance, and training on how to implement the ADA. ADAta.org

↗ **Federal Transit Administration (FTA):** Provides financial and technical assistance to local public transit systems, oversees public transit safety measures, and supports research of public transit technology. transit.dot.gov

↗ **National Aging and Disability Transportation Center (NADTC):** Promotes transportation options that meet the needs of older adults, people with disabilities, and caregivers. NADTC.org

↗ **Shared-Use Mobility Center (SUMC):** Is dedicated to achieving equitable, affordable, and environmentally sound mobility across the United States. SharedUseMobilityCenter.org

↗ **Transportation for America:** Promotes safe, affordable, and convenient transportation for people of all means and abilities. T4America.org



QUEST PODCAST

Listen to a conversation with a disability policy advisor at the US Department of Transportation at MDAQuest.org/podcast/accessibility-DOT.



But the law isn't well understood or enforced, which means people with disabilities must be their own advocates, suggests Stephen Coleman Kenny, a policy associate at Transportation for America, a nonprofit that advocates for safe, affordable, and accessible transportation systems.

That starts with understanding what accessible transportation looks like.

Depending on where they live, citizens with disabilities may have any of three options: rail, bus, and paratransit. To be accessible to people who use wheelchairs and other mobility aids, train stations, trains, and buses must have features like working elevators and ramps, and designated priority seating and wheelchair securement areas on vehicles.

But that's just the start. Unfortunately, disabled riders in many places can't reach accessible trains and buses due to impassable roads and sidewalks.

"It's great if you have an ADA-compliant transit station a mile from your house, but how are you going to get there if the sidewalks along the way lack curb cuts; are narrow, sloped, obstructed, or poorly managed; or if you get stranded in an intersection because the pedestrian signals are timed for able-bodied people?" Stephen asks.

That's where paratransit comes in. Mandated by the ADA, it provides door-to-door service for riders with disabilities who are unable to access fixed-route trains and buses. But it, too, has limitations, according to Elizabeth, who is a regular user of *Llame y Viaje* (Spanish for "Call and Ride") a paratransit service operated by the Puerto Rico Metropolitan Bus Authority. For example, the service only operates within three-quarters of a mile of the regular bus routes in eight of Puerto Rico's municipalities. What's more, it doesn't operate on Sundays or in the evenings past 8 p.m., it requires reservations at least 24 hours in advance, and it often runs hours behind schedule. To top it off, the fare is higher than regular public transit.

"Paratransit is required for every transit provider in the country — but what's not required is to provide good or affordable service," Stephen says. "These systems are often bare-bones and expensive for users. We see a lot of issues with them."

Issues can be especially apparent in rural communities. Although bus and paratransit service are available in many of them, routes and schedules tend to be limited, with fewer and less frequent stops.

Among Americans ages 5 and older, **25.5 million people** have a travel-limiting disability, according to the Bureau of Transportation Statistics.

“Paratransit is required for every transit provider in the country — but what’s not required is to provide good or affordable service.”

— *Stephen Coleman Kenny*

“Some of the lowest rates of car ownership are in rural parts of the country,” Stephen continues. “There are hundreds of rural transit agencies across the country doing great if sometimes expensive work to provide service, but there are still a lot of people in these communities that are isolated from society because they don’t have access to good transit service. And that’s a real problem.”

Some rural and urban transit agencies are partnering with private companies like Uber and Lyft to fill gaps in their service. But those companies say they’re exempt from the ADA and, as a result, don’t always have accessible vehicles available for passengers with disabilities.

Advocating for change

Despite deficiencies in public transit systems, improvements could be on the horizon thanks to President Biden’s Bipartisan Infrastructure Law, which authorizes up to \$109 billion for public transportation — the largest federal investment in



Stephen
Coleman
Kenny

public transportation in the nation’s history. As transit agencies determine how to spend that money, now is a unique opportunity for Americans with disabilities to advocate for investments that would improve accessibility.

“The first step is to find out who represents you at the federal, state, and local levels,” advises Mark Fisher, director of advocacy engagement at MDA. “Next, I’d call the representative’s office, introduce yourself and ask them to have a meeting. The good news is many of these meetings can be done virtually or in person, so connecting with your lawmakers is easier than ever. During the meeting, share your experience and challenges when trying to access public transportation and urge them to take action to fix this problem.”

While state and federal lawmakers can be powerful allies, when it comes to addressing specific problems, Stephen encourages people to contact their local policymakers, who are typically in the best position to help.

Progress may be slow, but even small victories can make a big difference. [Q](#)

Matt Alderton is a Chicago-based freelance writer who frequently covers health and transportation.

ACCESSIBILITY CHECK

Many transit systems were designed before the Americans with Disabilities Act (ADA) was signed into law, and they may have “legacy” rail stations and vehicles that have not been updated. In fact, the Federal Transit Administration reports that about 25% of rail stations in the United States are not fully accessible.

Before taking a train or bus, find out if the accessibility features you need are available along your route. Go to your transportation system’s website or call their customer service line to find out if they have these features.

Trains:

- Working elevators between street level and train platforms
- Gap fillers, bridge plates, or ramps to span the gap between platforms and trains
- Priority seating areas, with emergency call buttons within this area

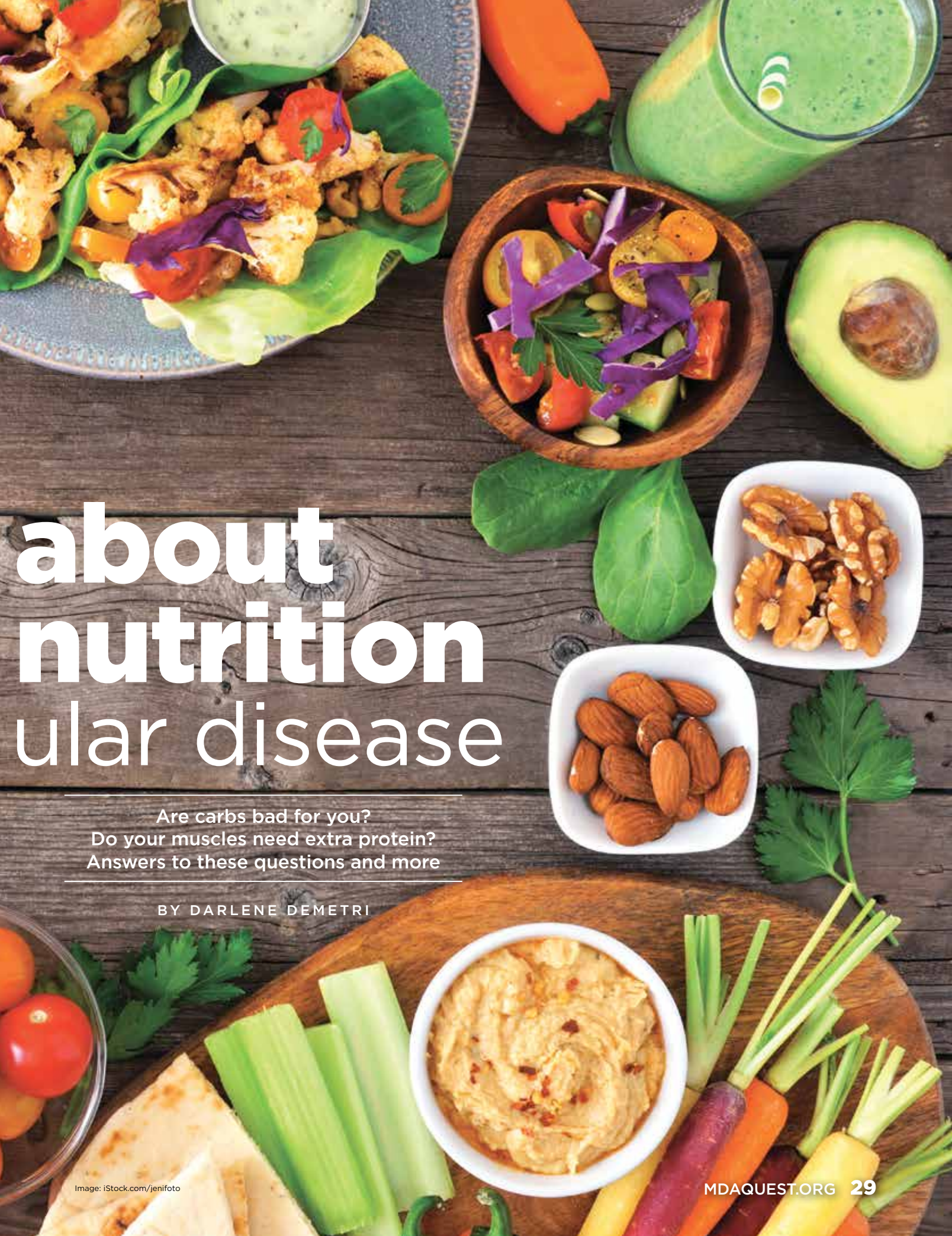
Buses:

- Ramps for wheelchair entry
- Low-entry design with step-free access to the seating area
- Priority seating and wheelchair securement areas



The Truth

+ neuromusc



about nutrition ular disease

Are carbs bad for you?
Do your muscles need extra protein?
Answers to these questions and more

BY DARLENE DEMETRI

What is a healthy diet? That’s not a simple question to answer because it depends on a lot of factors, including if you’re living with a neuromuscular disease. For example, if you have low muscle mass due to muscular dystrophy, high-protein in-between-meal snacks might be a healthy choice. If you have amyotrophic lateral sclerosis (ALS) and can only eat a little bit every day, thickened ice cream could be a healthy part of your diet.

“It really depends. When it comes to nutrition, there’s always a ‘Yes, but. . .,’” says Tad Campbell, an assistant professor with the Department of Clinical Nutrition at UT Southwestern Medical Center in Dallas, Texas. To cut through the confusion, we asked experts about some often-misunderstood areas of nutrition and how they affect people with neuromuscular diseases.

WHAT YOU SHOULD KNOW ABOUT:

Carbohydrates

Many fad diets have fueled the mistaken belief that all carbs are bad for you. In fact, they are an essential energy source.

“Some of the healthiest food groups have carbs,” says Evelyn Gold, a clinical dietician with the Complex Healthcare Program at Nationwide Children’s Hospital in Columbus, Ohio. “Carbs are essential for a well-balanced diet full of variety so the body and brain can conduct the processes to live.”

Complex carbs are the best type, because the body assimilates and expends them slowly. Found in fruits, vegetables, and whole grains, complex carbs provide energy over a long period of

time in manageable increments, are less likely to be stored as fat tissue, and keep you full longer.

Complex carbs high in fiber, like whole grains, are great for the gastrointestinal tract, keeping bowel movements more regular.

Simple carbs, found in table sugar and any type of added sugar in foods and drinks, are short acting. “Like gasoline in your car, you will burn through them quickly and once gone, you have an empty tank,” says Evelyn. Consuming them often can make you feel tired and sluggish. Plus, the body converts an overload of simple carbs into fat tissue.

Fruits, because they contain natural sugars, are often mistaken for simple carbs. But the complex sugars in fruits take the body more time to break down. Complex sugars also exist in natural plant fiber, such as the skin of blueberries and apples. Like vegetables and whole grains, fruits are healthy and provide lasting energy.

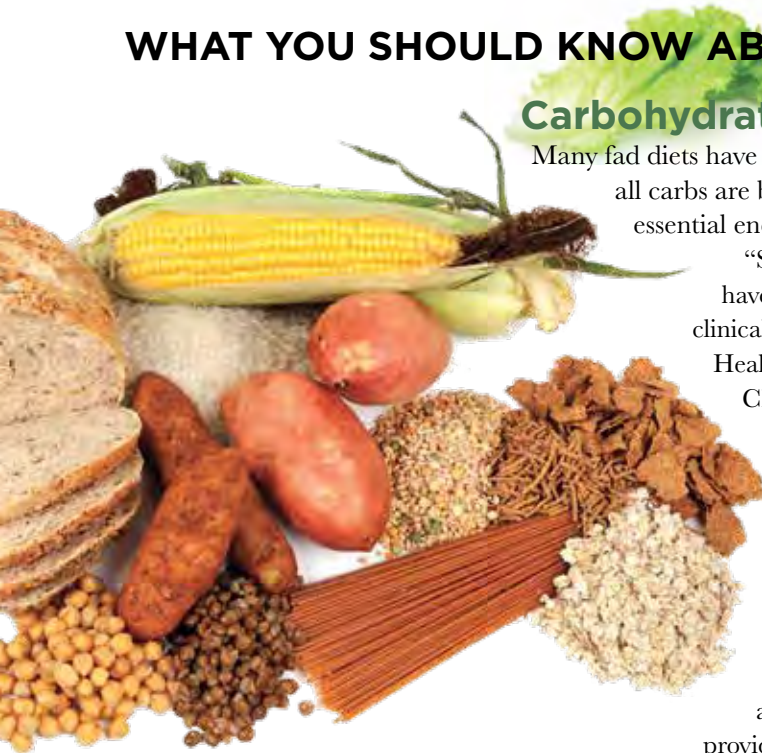


Starchy and non-starchy carbs may be part of a healthy diet, with caveats. Starchy carbs usually have a soft, pasty texture on the inside when cooked, such as peas and potatoes. Starchy carbs make a bigger impact on blood sugar, so portion control may be necessary if you have diabetes or are on corticosteroid treatment.

Some non-starchy vegetables can be a problem if you have difficulty swallowing. “Many people with dysphasia choke on lettuce,” says Tad. “I tell them, ‘Don’t bother with it; it’s dangerous and there’s not enough nutrition in it.’” This can be true for any leafy green.

He recommends cooking vegetables and fruits until they are soft enough to chew and swallow easily.

Expert recommendation: Try to get 5 to 6 servings of a variety of fruits, vegetables, and whole grains a day.



HEALTHY FOOD CHOICES

Complex carbs

Whole grains

Whole wheat breads
Whole wheat pastas
Brown rice
Oatmeal
Quinoa *
Buckwheat *

* Good source of protein.

Non-starchy

Broccoli ♦
Cabbage
Carrots
Leafy greens ♦■
-kale
-lettuce
-spinach
Mushrooms
Onions
Summer squash
-yellow
-zucchini

♦ Good source of calcium.

■ Avoid if you have trouble swallowing.

Starchy

Beans ❖
Corn
Peas
Winter squash ❖
-acorn
-butternut
-pumpkin
Potatoes (unpeeled)

❖ Good source of protein and fiber.

❖ Avoid if you have trouble swallowing.

Natural Sugar

Apples ♦
Berries ○
-blackberries
-blueberries ♦
-raspberries
-strawberries
Grapes ♦
Mangos
Melons
Oranges
Peppers ♦

♦ Skin provides extra fiber.

○ High in antioxidants that fight inflammation.

Proteins

Lean or healthy fats

Eggs †
Fish, especially salmon †
Shellfish
Nuts
Nut butters
Pumpkin seeds
Soybeans
Soy-based products
-tempeh
-tofu

† Good source of vitamin D.

Dairy ♦

Cottage cheese
Greek yogurt ❖
Milk ❖

♦ Good source of calcium.

❖ Look for options fortified with vitamin D.

Fats

It's not a four-letter word. We all need healthy fats for brain function and other key processes in the body.

Unsaturated fat is the healthiest type. It's liquid at room temperature and is a blend of monounsaturated and polyunsaturated fats. Seed and plant-based oils high in monounsaturated fat, such as avocado and olive, are easier to digest and helpful to the cardiovascular system when consumed in moderation, according to the American Heart Association.

Foods high in healthy unsaturated fats include avocados, fish, nuts, nut butters, and seeds.

Saturated fat is solid at room temperature and is found in higher amounts in red meat and whole dairy products, such as butter and lard. Plant-based sources include tropical oils such as coconut, palm, and palm kernel oil.

Trans fat is manufactured and may be present in packaged pastries, pie crusts, and some fast foods like French fries. If the amount in a serving size is small enough, it doesn't need to be listed on the nutrition label. "Always check the ingredients list," advises Tad. "Look for the words 'partially hydrogenated' or 'hydrogenated'. That tells you there are trans fats in the product."

Saturated and trans fats are not easily absorbed by the body. They end up in the circulatory system, where they can raise cholesterol and blood fat levels, clog arteries, cause inflammation in the body, and increase the risk for cardiovascular disease.

Expert recommendation: Focus on consuming unsaturated fat and limit saturated fat to 7% of your daily calories. Avoid trans fat entirely.



Protein

Protein helps maintain muscle mass and other processes in the body. Some people with neuromuscular conditions benefit from making protein a focus in their diet. Unlike carbs and fats, proteins are not stored in the body.

“Protein obtained through food is floating in the bloodstream where it’s needed,” Tad says. “If there is no protein available in the bloodstream, it’s drawn from another protein source in the body—the muscles. That’s how you lose muscle when you lose weight.”

With neuromuscular diseases that result in fatigue, decreased appetite, difficulty feeding oneself, and difficulty chewing and swallowing, protein intake is often shortchanged, so it’s important to seek out healthy high-protein foods with these tips:

- Eat mostly lean protein, which has a lower animal fat or fat content in general.
- Limit fatty protein, which has a high animal fat content. Except for fish, the fats you find in animal protein are saturated.
- Fish is high in monounsaturated and polyunsaturated fats. Dieticians consider all fish healthy to eat, including shellfish.
- Among meats, chicken breast is the leanest option, with little to no saturated fat. Steak has a higher saturated fat content than any edible part of a chicken.
- If you have trouble swallowing, pureed meats, mashed beans, scrambled eggs, tofu, cottage cheese, and Greek yogurt are good options.
- Plant-based proteins include beans, nuts, nut butters, seeds, and soy. If you are considering becoming a vegetarian or vegan, talk with a dietician to make sure you get the optimal amount of protein.

“Although we can’t stop the progression of, say Duchenne muscular dystrophy, we can help an individual preserve lean muscle mass as long as possible by making sure their diet is well-balanced and they are getting in some good sources of usable proteins as opposed to those higher fat proteins,” Evelyn says.

Expert recommendation: Most people need between 0.8 and 1.2 grams of protein per kilogram of body weight. With such a wide range, it’s best to discuss your specific needs with a dietician. In most cases, lean meat and fish are the best options.



AFFORDABLE FOODS

Good nutrition doesn’t have to break the bank. Read “Nutrition on a Budget” at [MDAQuest.org/nutrition-on-a-budget](https://www.mdaquest.org/nutrition-on-a-budget).





Calcium

Critical for healthy bones and teeth, calcium is a mineral found in dairy products, leafy green vegetables, and soy.

“Often, bone density is low in patients who can’t walk, because they’re not putting enough pressure on certain bones to maintain density and strength,” says Evelyn. Individuals with swallowing issues also are likely to consume fewer calcium-rich foods.

This is where dairy can help. It’s high in calcium, a good source of protein, and may contain other nutrients such as vitamin D and B vitamins. “Cottage cheese alone provides all the macro and micronutrients,” Tad says.

Vitamin D helps the body absorb calcium and is important for regulating emotions.

“Americans don’t get enough calcium from their diets, and it is difficult to get vitamin D through food, so these micronutrients are usually fortified in milk and juices,” Evelyn says.

Expert recommendation: Look for milk, juice, cereal, and bread fortified with calcium and vitamin D.



WHOLE FOODS VS PROCESSED FOODS

Processing food means changing it from its natural state. A whole food looks as it did when it came from the earth, such as an apple or a carrot. Processed foods come in many forms, from canned to frozen to packaged.

When food is processed, preservatives, including salt, are usually added, making many convenience foods high in sodium, which contributes to high blood pressure and water retention. Processed foods also typically have added fat and sugar, and important nutrients, such as fiber, may be removed.

“The rule of thumb we go by is you want your foods to be as close to nature as possible as opposed to foods that had a lot of handling and things done to them before you eat them,” Tad says.

But dietitians also acknowledge that processed foods are often easier to prepare and eat.

“With a neuromuscular disease are you able to stand there for several minutes prepping and cooking an entire meal that requires a lot of whole foods? Or are you better off finding something more convenient for yourself?” Tad asks.

There might be a place for some processed foods in your diet to make life easier. For example, for someone with weakness in the arms, a high-protein, easy-to-prepare finger food like frozen chicken tenders can be a good choice. Look for a brand that uses chicken breast meat and has at least 12 grams of protein per serving.

With all packaged foods, look for options that contain less than 160 milligrams of sodium per serving.

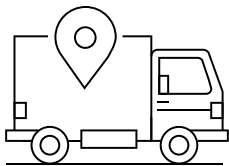
Ultimately, a healthy diet for you is one that is sustainable and produces the best outcomes. Working with a dietitian who is familiar with neuromuscular diseases, which can be found at many MDA Care Centers, can help you get there. [Q](#)

Darlene Demetri is a Connecticut-based freelance writer living with facioscapulohumeral muscular dystrophy (FSHD).



+WHAT TO EAT?

Watch the Nutrition and Neuromuscular Disease MDA Engage webinar to learn more about healthy eating at mda.org/care/mda-engage/community-webinars.



Navigating the World of DME

[DURABLE MEDICAL EQUIPMENT]

*Tips for avoiding frustration
with shortages and delays*

BY SUSAN JOHNSTON TAYLOR

If you've tried to order a new power wheelchair or other mobility device in the past two years, you may have experienced delays, product shortages, or other frustrations. "We're still experiencing the very same issues that troubled us in 2021 and 2022 as it relates to the supply chain and labor market," says Katie Stevens, CEO of Reliable Medical, a medical equipment provider in the Midwest.

Some of these challenges began to improve later in 2022, but the year also brought rising costs in every industry, including durable medical equipment (DME). Fortunately, professionals across the DME field — from manufacturers to distributors — are working to reduce delays and keep up with demand amid challenging circumstances.

Quest talked with industry insiders to find out what's happening in this space, where it might be heading in the future, and how you can navigate this changing landscape.

image: iStock.com/cnythzl



Professionals across the DME field are working to reduce delays and keep up with demand amid challenging circumstances.



A Reliable Medical client tests a manual wheelchair.

3 factors impacting lead times and equipment availability

Going on our third year of the pandemic, we've all become used to hearing about shortages affecting consumer products from baby formula to Sriracha hot sauce. The forces that lead to sparse supermarket shelves also affect DME.

1. Supply chain

Supply chain refers to the series of processes that are required to manufacture and distribute goods. "The complex rehab technology, or CRT, supply chain is already an intricate system that involves organizations, people, activities, information, and resources, so when something changes in one of those areas, it affects the rest," says David Pietrzak, ATP, vice president of supply chain for National Seating and Mobility, a national CRT supplier.

David adds that shortages of aluminum and microchips have directly impacted orders. "Intense workforce shutdowns teamed with rising energy costs have slowed production of these items around the globe," he says.



David Pietrzak, ATP

Even when parts are available, other logistical issues can slow things down. "Moving raw materials, components, and products between locations is a major part of the CRT supply chain, and transporting goods continues to be a challenge," he says.

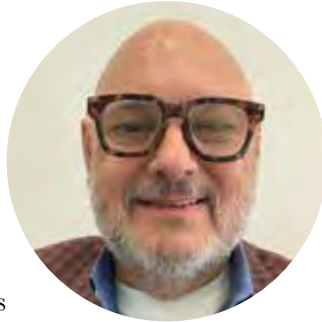
Often, though, challenges can lead to innovation. "The current environment forced us to take a hard look at the process and ask the questions 'Why is it this way?' and 'How can we improve it and provide more visibility to what's going on?'," David says. For example, his company is now more involved in the procurement process to cut down long lead times and provide greater transparency to consumers about delayed components. As other companies increase transparency, this could help reduce bottlenecks.



2. Staffing shortages

When people left the workforce or changed careers during the pandemic, staffing shortages hit almost every industry, DME included. Mike Seidel, vice president of new accounts for Numotion, a national CRT supplier, says some supply issues have improved recently, but labor shortages persist.

“It’s challenging to fill orders when you don’t have the staff to assemble the equipment and get it out the door, but one of the largest obstacles Numotion is having right now is on the service and repair side,” he says. “Our demand has never been higher, and our biggest challenge is having enough certified service technicians in the field.” Numotion is recruiting new technicians, but it takes time to train them.



Mike Seidel, Numotion

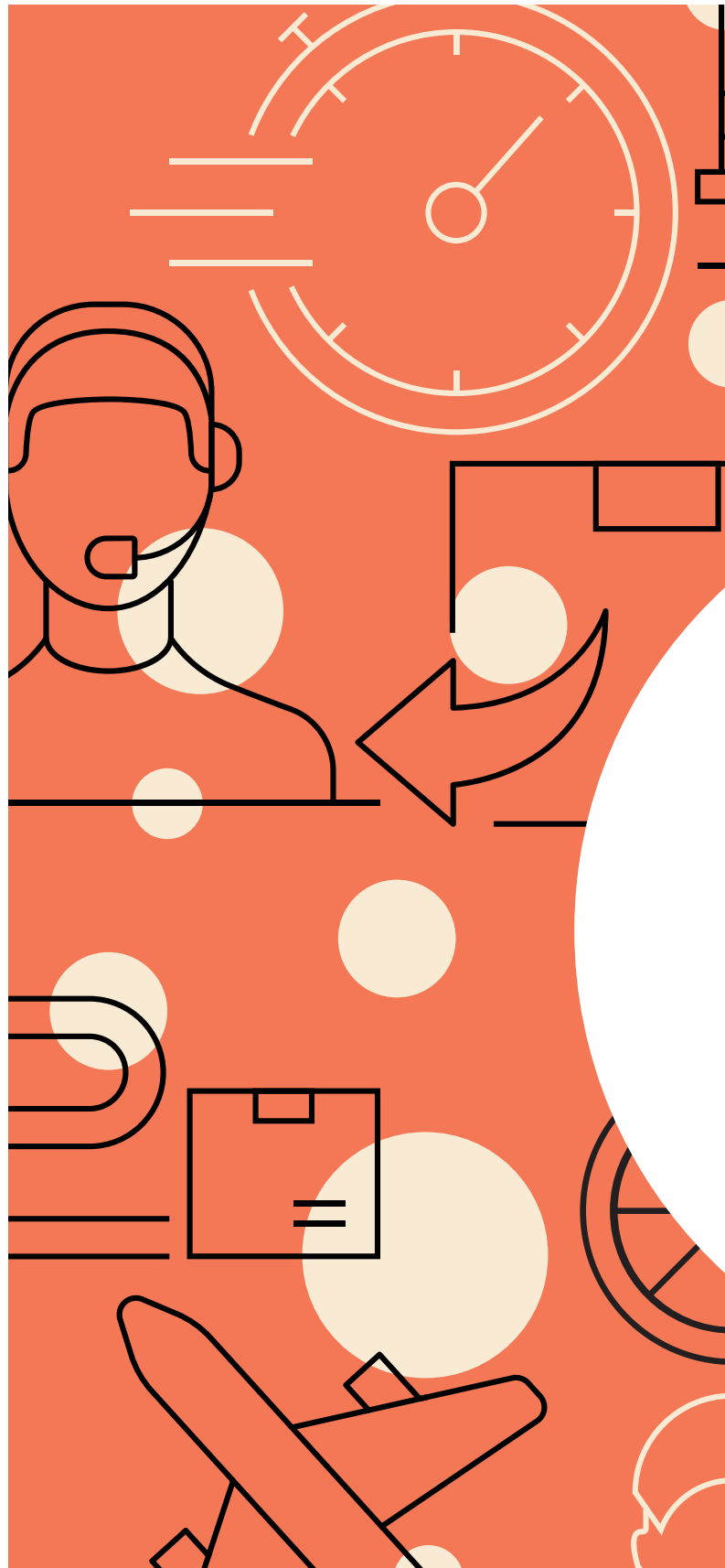
FINDING RESOURCES FOR EQUIPMENT

If you have questions about where and how to get durable medical equipment (DME), the MDA Resource Center can help. The MDA Resource Center is a collective of individuals with personal and professional experience who strive to empower our diverse neuromuscular community with resources supporting a world of independence and possibility. This team is available Monday through Friday, 9 a.m.-5 p.m. CT, to take your calls and answer your emails.

“We’ll walk people through explaining the process of obtaining durable medical equipment, and we can provide resources if they get a denial,” says Brooke Smith, LMSW, MEd, associate director at the MDA Resource Center. The MDA Resource Center can also help you locate an organization in your community that loans out DME or accepts equipment donations.

With nearly 20 years of experience in resource navigation, Brooke and her fellow social workers and specialists in the MDA Resource Center are committed to providing quality, compassionate care.

Call the MDA Resource Center at **833-ASK-MDA1** or email **ResourceCenter@mdaUSA.org**.



Virtual service options became popular during the pandemic, and they're still available.

A Reliable Medical client tests a power wheelchair.



3. Inflation

Costs of all goods and services increased in 2022. But, unlike companies that can raise their prices and pass those increases to customers to preserve their profit margin, DME companies must set their prices based on the approved amounts set by Medicare, Medicaid, and other insurers.

“DME providers are put in a place of either accepting very poor reimbursement from certain payers or not getting the contract and not having the ability to serve customers in that market,” says Katie of Reliable Medical.



Katie Stevens,
Reliable Medical

Insurance reimbursement amounts have not kept pace with inflation. That’s caused some companies to narrow their product offerings. “While not a tactic at Reliable, certain providers are negotiating preferred partnerships with one manufacturer or partner. It may be a good business strategy, but it eliminates patient and clinician choice,” she says. The industry is pushing for higher reimbursement rates, which could help bring back more options.

Reimbursements are especially low for repairs, so some DME companies have stopped servicing equipment they didn’t sell or have completely halted service and repair. In addition, some insurers require prior approval for repairs, which can slow things down. “We’ve been working diligently with many private insurance companies to allow repairs up to an agreed upon amount with no authorization,” Mike says. “They’ve approved the wheelchair for medical necessity, so why do we have to go back through establishing medical necessity for the repair parts needed to keep it going? It seems redundant in many cases.” If that policy becomes more widespread in the future, it could help speed up repairs.

Advocate for yourself

The global supply chain and economic policies may be out of your control, but industry experts shared a few tips for getting what you need in this unpredictable market.

Talk to your clinician. As some DME companies narrow their product offerings, Katie recommends “working with your clinicians to know what your product options are, and really fighting for the most appropriate clinical outcome.”

Use virtual service options when available. Virtual service options, where you meet with a technician on Zoom instead of in person, became popular during the pandemic, and they’re still available. For simple fixes, this

can get your chair working again quickly and efficiently. “Did something come unplugged?” Mike asks. “Is it a simple component that’s not very expensive? Or is it your whole wiring harness and electronics?” A technician may be able to answer those questions with a virtual service visit.

Ask about in-shop repairs. When there’s a shortage of technicians, it can take a long time to schedule in-home service visits. Companies like Numotion offer in-shop repair services at their locations. “It can help you get served faster,” Mike says. It’s more efficient for technicians because they have a wider variety of parts and equipment on-site, not to mention they’re not losing time driving from appointment to appointment.

Delays and shortages are frustrating when you rely on DME, but as we move into 2023, there are encouraging signs that the industry will be able to provide equipment and services to the people who need them more quickly and efficiently. [Q](#)

Susan Johnston Taylor writes about health and general interest topics for print and online publications.

“[Work] with your clinicians to know what your product options are, and really fight for the most appropriate clinical outcome.”

—Katie Stevens



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EMFLAZA® has been shown to preserve muscle strength and function

In a clinical trial of 196 boys aged 5 to 15 with Duchenne muscular dystrophy, the effectiveness and safety of EMFLAZA was compared with placebo (sugar pills) and prednisone. EMFLAZA improved muscle strength at 12 weeks compared with placebo (0.15 change in strength score vs -0.10 change in strength score)

*These findings were not considered statistically significant. This means that because the two groups studied were not large enough, the results could have occurred by chance.



Mikey, Age 9

Reid, Age 7

Actual EMFLAZA patients.

STUDY INFORMATION

Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center.

Objective: To assess outcomes among patients with DMD receiving deflazacort or prednisone in real-world practice.

Methods: Clinical data for 435 boys with DMD from Cincinnati Children's Hospital Medical Center were studied retrospectively using time-to-event and regression analyses.

Results: Median ages at loss of ambulation were 15.6 and 13.5 years among deflazacort- and prednisone-initiated patients, respectively. Deflazacort was also associated with a lower risk of scoliosis, improved ambulatory function, greater % lean body mass, shorter stature, and lower weight, after adjusting for age and steroid duration. No differences were observed in whole body bone mineral density or left ventricular ejection fraction.

Marden JR, Freimark J, Yao Z, Signorovitch J, Tian C, Wong BL. Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center. J Comp Eff Res. 2020;9(3):177-189. doi:10.2217/ cer-2019-0170.

Delayed onset of scoliosis (curved spine)

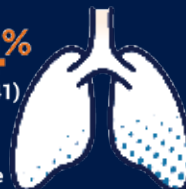
LOWERED RISK OF SCOLIOSIS compared to prednisone



Over 11.9 years, 17.9% of patients developed scoliosis taking prednisone vs 7.9% taking deflazacort.

Preserved lung function

9.24% (CI: 3.06 - 15.41) higher forced vital capacity %-predicted vs prednisone



*Forced vital capacity is a type of test that measures the amount of air your son can inhale and exhale.

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(deflazacort)

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



Summary of Information for EMFLAZA®

What is EMFLAZA® (deflazacort) used for?

Emflaza is a prescription medicine used to treat Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

When should I not take EMFLAZA?

Do not use if you have had hypersensitivity, including allergic reactions, to deflazacort or any of the inactive ingredients.

What warnings should I know about EMFLAZA?

- EMFLAZA can cause changes in endocrine function. 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.
- There is an increased risk of infection when taking EMFLAZA. Tell the healthcare provider if the patient has had recent or ongoing infections or if they have recently received a vaccine. Medical advice should be sought immediately if the patient develops fever or other signs of infection. Patients and/or caregivers should be made aware that some infections can potentially be severe and fatal. Warn patients who are on corticosteroids to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.
- EMFLAZA can cause an increase in blood pressure and water retention. If this occurs, dietary salt restriction and potassium supplementation may be needed.
- There is an increased risk of developing a hole in the stomach or intestines in patients with certain stomach or intestine disorders when taking corticosteroids like EMFLAZA.
- EMFLAZA can cause severe behavioral and mood changes. Seek medical attention from the health care provider if any behavioral or mood changes develop.
- There is a risk of osteoporosis with prolonged use of EMFLAZA, which can lead to vertebral and long bone fractures.

- EMFLAZA may cause cataracts or glaucoma and a health care provider should monitor for these conditions if corticosteroid therapy is continued for more than 6 weeks.
- Immunizations should be up-to-date according to immunization guidelines prior to starting therapy with EMFLAZA. Live-attenuated or live vaccines should be administered at least 4 to 6 weeks prior to starting EMFLAZA. Live-attenuated or live vaccines should not be used in patients taking EMFLAZA.
- EMFLAZA can cause serious skin rashes. Seek medical attention at the first sign of a rash.
- Rare instances of anaphylaxis have occurred in patients receiving corticosteroid therapy, including EMFLAZA.

What should I tell my health care provider?

Tell the health care provider about all medical conditions, including if the patient:

- is pregnant or planning to become pregnant. EMFLAZA® (deflazacort) can harm your unborn baby.
- is breastfeeding or planning to breastfeed. EMFLAZA may appear in breastmilk and could affect a nursing child.

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.

What are the side effects of EMFLAZA?

The most common side effects of 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. These are not all of the possible side effects of EMFLAZA. Call your doctor for medical advice about side effects.

To report an adverse event, please call 1-866-562-4620 or email at usmedinfo@ptcbio.com. You may also report side effects to FDA at 1-800-FDA-1088 or at www.fda.gov/medwatch.

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US-EMF-0292 11/22

What Is MOVR

Neuromuscular diseases are a diverse group of rare, mostly genetic conditions. This has made developing treatments challenging. However, investments in rare disease research by private and public agencies, including MDA, have led to some breakthroughs. Fourteen new therapies for neuromuscular diseases were approved in the last 10 years, and more than 180 therapies are currently in development.

Funding is not the only thing that advances research. Equally important is data that helps clinicians, drug developers, research funders, and regulators make decisions about, for example, how to design a clinical trial or whether a drug is safe and effective.

That's where MDA's neuroMuscular ObserVational Research (MOVR) Data Hub comes in. It is the first data hub to collect clinic-entered data over extended periods of time from participants living with certain neuromuscular diseases. The geographic range of MOVR sites combined with the breadth of data it collects make MOVR a powerful resource for understanding genes that cause neuromuscular diseases, studying disease progression, and improving clinical trials.

How it works

MOVR works with the MDA Care Center Network to gather data on individuals living with neuromuscular diseases, with their consent, at their clinic visits. Currently, more than 50 Care Centers are collecting data on seven diseases: amyotrophic lateral sclerosis (ALS), Becker muscular dystrophy (BMD), Duchenne muscular dystrophy (DMD),

facioscapulohumeral muscular dystrophy (FSHD), limb-girdle muscular dystrophy (LGMD), Pompe disease, and spinal muscular atrophy (SMA).

"Our goal is to extend MOVR to the majority of the more than 150 MDA Care Centers, which collectively see more than 60,000 individuals annually for regular care and participation in clinical trials," says Elisabeth Kilroy, PhD, director of MOVR at MDA. She also set a five-year goal to add Charcot-Marie-Tooth disease (CMT), myotonic dystrophy (DM), myasthenia gravis (MG), and some ultra-rare neuromuscular diseases for which very little data is available.

For individuals who opt to participate in MOVR, after providing consent, there's nothing they need to do other than attend their regularly scheduled Care Center appointments. The data captured in MOVR is information already collected at these visits, including their age at symptom onset, age at diagnosis, first symptoms, family history, genetic testing results, clinical trial participation, surgeries, medications, and assistive devices. All information is added to the MOVR Data Hub without personally identifiable information.

"We're efficiently capturing clinical data from visits that are happening already," Dr. Kilroy says. This provides clinicians and researchers with access to valuable knowledge on disease progression and how well therapies and other interventions are working.

MOVR aims to serve the entire neuromuscular disease community

Researchers

can better understand the relationship between health outcomes and genes, and between health outcomes and medical interventions (such as drugs, surgeries, and medical devices).

Clinicians can quickly match their patients to clinical trials, approved therapies, and personalized care.

Individuals at all ages and stages of neuromuscular disease gain a platform to be "seen and counted" by the medical and scientific communities.

MOVR?

MDA's innovative data hub aims to improve research and care

BY AMY BERNSTEIN

Access to therapies

As the search for safe and effective neuromuscular disease therapies increasingly looks to gene-targeted therapies, it's more important than ever for researchers to make data-driven decisions. Even after a therapy is approved, collecting data is still vital. "MOVR serves as a platform for collecting data beyond the clinical trials and in a diverse participant population," Dr. Kilroy says.

Therapies for rare disease are more likely than non-rare disease therapies to receive provisional approvals that require further evaluating the drug while it is on the market, as was the case for all four exon-skipping therapies for DMD. In addition, government regulators may require drug developers to follow recipients of novel gene therapies for years following treatment.

In addition to meeting regulatory requirements, data collection allows doctors at participating MOVR sites to look at the national data to gain a broader understanding of how current treatments affect disease progression. Tracking trends can be especially crucial for diseases such as ALS, where there are many variables affecting disease progression. "It is important that we understand how therapies work in the real-world setting, and how they change the course of disease progression," explains Dr. Kilroy.

Researchers from academia, biopharmaceutical, or government entities can request specific data or get access to a sophisticated dashboard that allows them to gather MOVR data and run analyses. The

<MOVR FAQ>

How can I participate?

Individuals clinically diagnosed with an eligible disease who receive care at a participating MDA Care Center, or legal guardians, can sign a consent form to participate in MOVR.

Does it take much time?

No. Data is collected during the course of regular MDA Care Center visits.

How is my privacy protected?

Only authorized personnel at a MOVR site have access to the identified data collected from participants at their site. Data is stripped of personally identifiable information before being added to the MOVR Data Hub.

Who uses this data?

Representatives from healthcare, academia, the biopharmaceutical industry, government, or nonprofit organizations may request MOVR data. Each request follows a formal review process to ensure the proposed project will produce meaningful scientific findings.

What if I decide I don't want to participate?

A participant may withdraw their consent at any time.

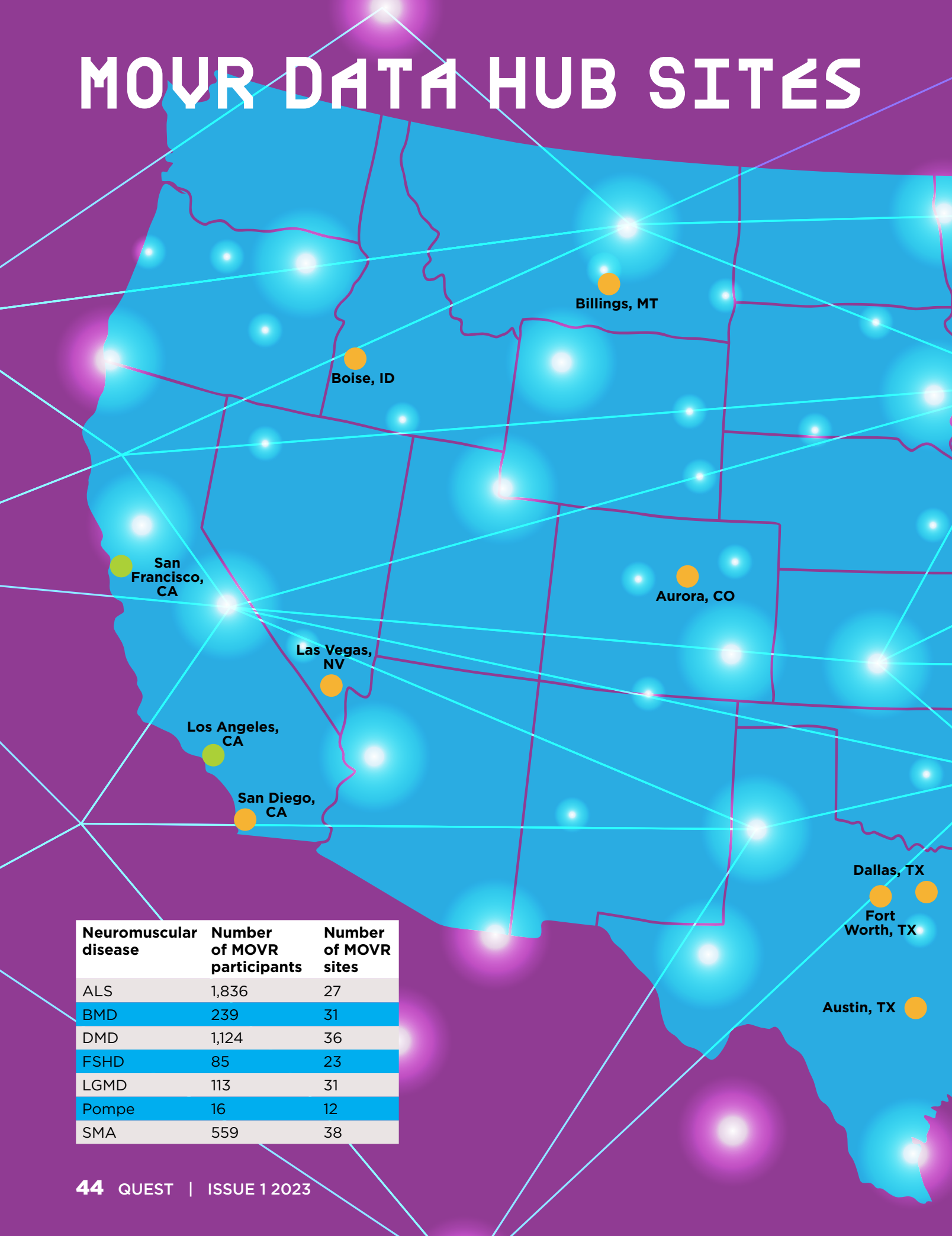
MOVR team evaluates each request and shares data only when a proposed project has the potential to produce meaningful scientific findings.

With its capacity for collecting and analyzing a vast amount of clinical data for neuromuscular diseases before, during, and after treatment, MOVR will be instrumental in uncovering the most effective therapies and ensuring they remain available to the neuromuscular disease community in the coming years. [Q](#)

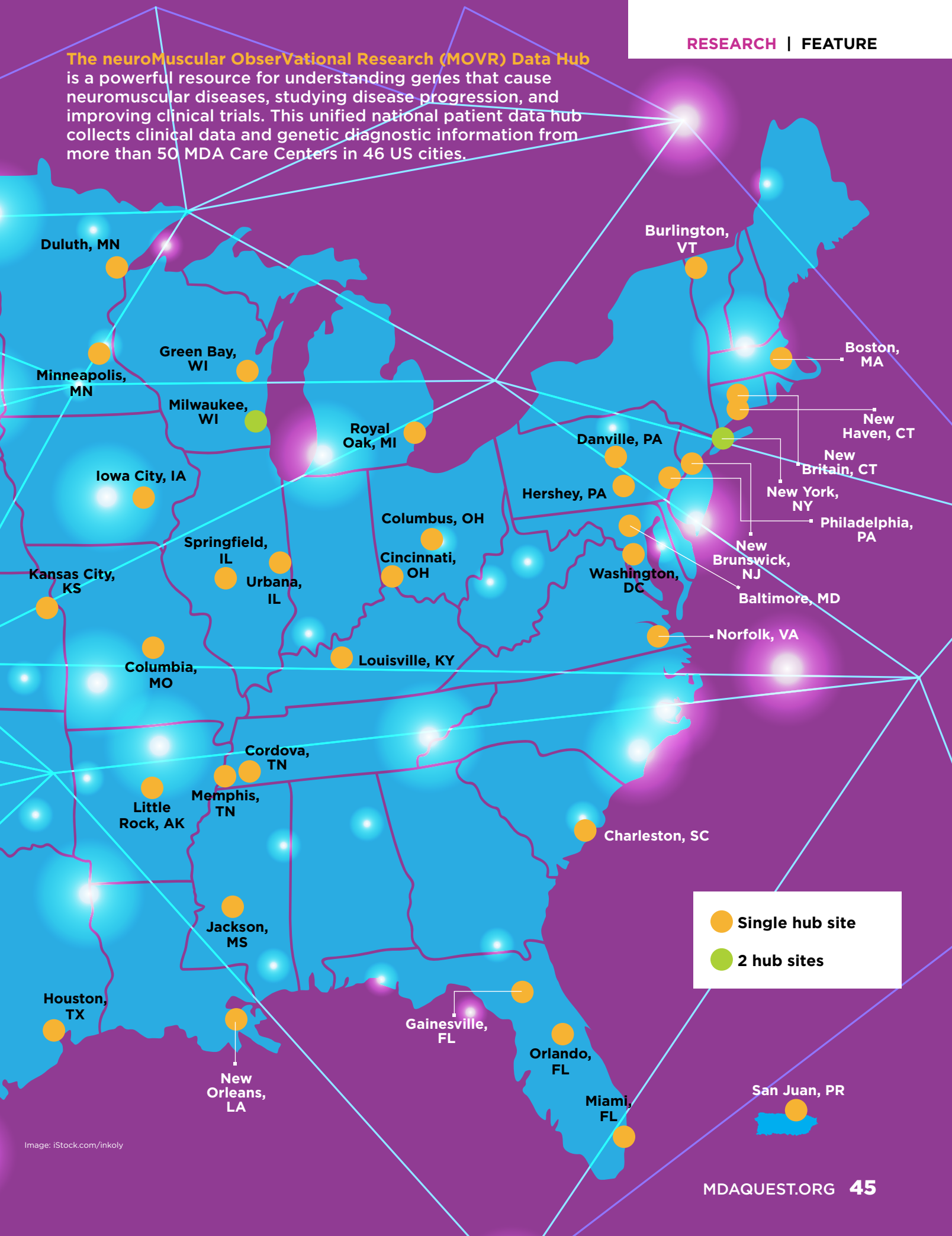
Amy Bernstein is a writer and editor for Quest.

MOVR DATA HUB SITES

Neuromuscular disease	Number of MOVR participants	Number of MOVR sites
ALS	1,836	27
BMD	239	31
DMD	1,124	36
FSHD	85	23
LGMD	113	31
Pompe	16	12
SMA	559	38



The neuroMuscular ObserVational Research (MOVR) Data Hub is a powerful resource for understanding genes that cause neuromuscular diseases, studying disease progression, and improving clinical trials. This unified national patient data hub collects clinical data and genetic diagnostic information from more than 50 MDA Care Centers in 46 US cities.



- Single hub site
- 2 hub sites

Image: iStock.com/inkoly



2022's Advocacy Victories

Thanks to the work of the MDA advocacy team and volunteers, 2022 was an exceptional year for legislative milestones for the neuromuscular disease community. Here are some of the achievements MDA is celebrating.

Increasing access to care

- More states now screen for Pompe disease and spinal muscular atrophy (SMA) at birth. Now, at least 32 states screen for Pompe, and 47 screen for SMA.
- MDA co-sponsored the nomination to add Duchenne muscular dystrophy (DMD) to the Recommended Uniform Screening Panel (RUSP) for newborns.
- Congress increased funding by \$3 million for newborn screening programs at the Centers for Disease Control and Prevention (CDC) and by \$2 million at the Health Resources and Services Administration. Lawmakers also funded \$1 million for a study to improve and modernize the newborn screening program.
- Lawmakers extended important Affordable Care Act subsidies through the Inflation Reduction Act, which will make buying insurance more affordable.
- Starting in 2025, there will be a \$2,000 per year out-of-pocket cost-sharing cap on Medicare prescription drugs.
- Policies that increase access to telehealth have been extended by two years.
- Congress will invest \$450 million over three years in programs that will make it easier for people to access home and community-based healthcare services.

Accelerating therapeutic development

- New diagnostic codes for limb-girdle muscular dystrophies (LGMD) were implemented. These will potentially shorten the diagnosis timeline, help clinicians deliver more precise medical care, improve clinical trials, and increase future access to targeted treatments.
- Congress passed crucial new programs and initiatives for the US Food and Drug Administration (FDA), including some that will help create innovative and beneficial clinical trials for rare diseases, speed up regulatory review, and

invest in gene and cell therapies. In addition, clinical trials will be more diverse, accelerated pathways for new therapies

were improved, and critical rare disease programs were renewed.

- Legislators increased funding for the National Institutes of Health (NIH) by \$2.5 billion and by \$226 million for the FDA.
- We continue to implement the ACT for ALS (amyotrophic lateral sclerosis), which speeds up therapies for those living with ALS and other neuromuscular diseases. Overall, in 2022, MDA advocates secured \$100 million in funding for the NIH expanded access grants program and \$10 million for the FDA rare neurodegenerative disease research grants program.

Empowering independence

- The US Department of Transportation (DOT) released an Airline Passengers with Disabilities Bill of Rights.
- MDA advocates contributed first-person testimonials about their experiences traveling by air.
- Hundreds of advocates urged the DOT to make lavatories more accessible on airplanes.
- Congress passed the ABLE Age Adjustment Act, which will increase the age by which an individual must have established a qualifying disability from 26 to 46.

Returning to Capitol Hill (virtually)

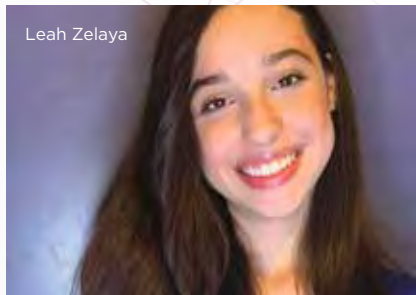
Finally, after a three-year hiatus, MDA advocates went back to Capitol Hill, but this time virtually. On Sept. 28 and 29, 2022, families from across the country urged lawmakers to take action on crucial issues impacting the neuromuscular community during the Virtual Summit & Hill Day. Together, we had 100 legislative meetings. [Q](#)

We could not have accomplished these milestones without our community advocates. If you are not an MDA grassroots advocate, join us at mda.org/advocacy.



Clockwise from top: MDA advocates Jaime, Leah, and Bevis Zelaya; Senator Chuck Schumer staff member Grace Magaletta; and MDA staff member Paul Meimeyer

Meet our 2023 Ambassadors



MDA is excited to welcome our new National Ambassador for 2023, Leah Zelaya, 15, of Brooklyn, New York. Leah lives with scapulothoracic spinal muscular atrophy, and she and her family have long been enthusiastic advocates for MDA, participating in events like Hill Day. Leah says she is excited to continue her advocacy work and be a source of encouragement for the MDA community in 2023.

“By sharing my story, I hope to give a different perspective on how the world views us,” Leah says. “We are human beings who dream, hope, and feel — just like everyone else does. I hope to raise enough awareness so that others in our community will also join in our mission of ending neuromuscular disease.”

Leah joins our other National Ambassador, Amy Shinneman, who will be serving her second year in this role. Amy, of Indianapolis, lives with Bethlem myopathy and previously served as a local MDA Ambassador after her diagnosis in 2018. [@](#)



Learn more about our 2023 ambassadors at MDAQuest.org/mda-welcomes-its-2023-national-ambassadors.

Thank You, Volunteers!

As we begin MDA’s Year of the Volunteer, we are recognizing the many MDA volunteers who helped in the past year. We couldn’t fulfill our mission without our volunteers, who contributed so many hours to inspire and support our communities. “The sense of purpose and community our volunteers create has a lasting impact — not just for those we serve, but for the volunteers themselves,” says Wendi Dressen, MDA’s senior director of volunteer services. “Volunteers feel they are getting back more than they are giving when they donate their time and talents to MDA.”

Here are just some of the ways our volunteers make a difference:

- At MDA Summer Camp
- Through MDA volunteer committees and leadership roles
- Through MDA general administrative volunteer roles



2023 MDA Year of the Volunteer

- Through MDA advocacy
- At MDA fundraising events
- Through MDA care initiatives
- Through MDA virtual volunteering [@](#)

Interested in becoming a volunteer? Learn more at mda.org/volunteer.



Making Strides in DEI

Disability is diversity

MDA believes that true diversity cannot be achieved without the presence of those who live with disabilities. So we are making progress in furthering diversity, equity, and inclusion (DEI) so people with neuromuscular diseases can access all life's milestones. In 2022, we continued to lead the charge in advocating for DEI in a variety of ways:



- **The formation** of a DEI coalition committed to leveraging its voice and platform to create a more inclusive world. Our coalition members are Mindy Henderson, editor-in-chief of Quest Media; Michael Lewis, director of Disability Policy & Advocacy; and Marissa Lozano, M.Ed., director of Community Education. Our DEI Coalition works with other like-minded advocates and organizations to ensure everyone has the same access to personal freedoms, financial independence, ability and mobility, and access to the world.

- **Continued advocacy** to provide better access to health care, higher education, disability employment, and more accessible travel. (Read MDA's 2022 advocacy recap on page 46.)
- **Fostering collaborations** to bridge the gap between people living with disabilities and organizations.
- **Hosting DEI** roundtables and workshops to understand the interests, needs, and personal journeys of the disability community, as well as available opportunities.

MDA also has lasting partnerships aimed at creating a more inclusive culture and helping connect organizations with the talent the disability community has to offer. One successful partnership is with Pfizer, which found immense value in the collaboration. According to Pfizer: “[Our] volunteer DMD Equity Task Force was proud to partner with the MDA on the development and delivery of the Disability is Diversity: Through the Lens of Duchenne Muscular Dystrophy initiative. It served as a powerful call to action for us to better understand the needs of patients and caregivers everywhere and to enhance our investment in Pfizer’s patient-centric efforts.”

MDA will continue this work in 2023 to elevate the conversation that disability is diversity.

Tech for workplace inclusion

Using technology in creative ways can be one of the keys to unlocking workplace inclusivity and independence. MDA is committed to finding ways to use technology to achieve these goals.

“Technology is the great enabler, and for people living with a disability, it is also the greatest

Images: iStock.com/Yulia Sutryagina

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equalizer,” says Kristine Welker, MDA’s chief of staff. “As employers begin to embrace that remote work is here to stay, they realize that the workplace is now infinitely more accessible to people with disabilities. Furthermore, showing a commitment to a diverse and accessible workplace is a selling point that can attract even more talent.”

Through partnerships with technology platforms like Inclusively and FinancialForce, MDA is changing common mindsets around disability and helping create opportunities that are inclusive by design — so that every individual can participate in all aspects of life.

These tech partnerships reinforce MDA’s connection to our community and empower job seekers to seize their employment opportunities. [Q](#)



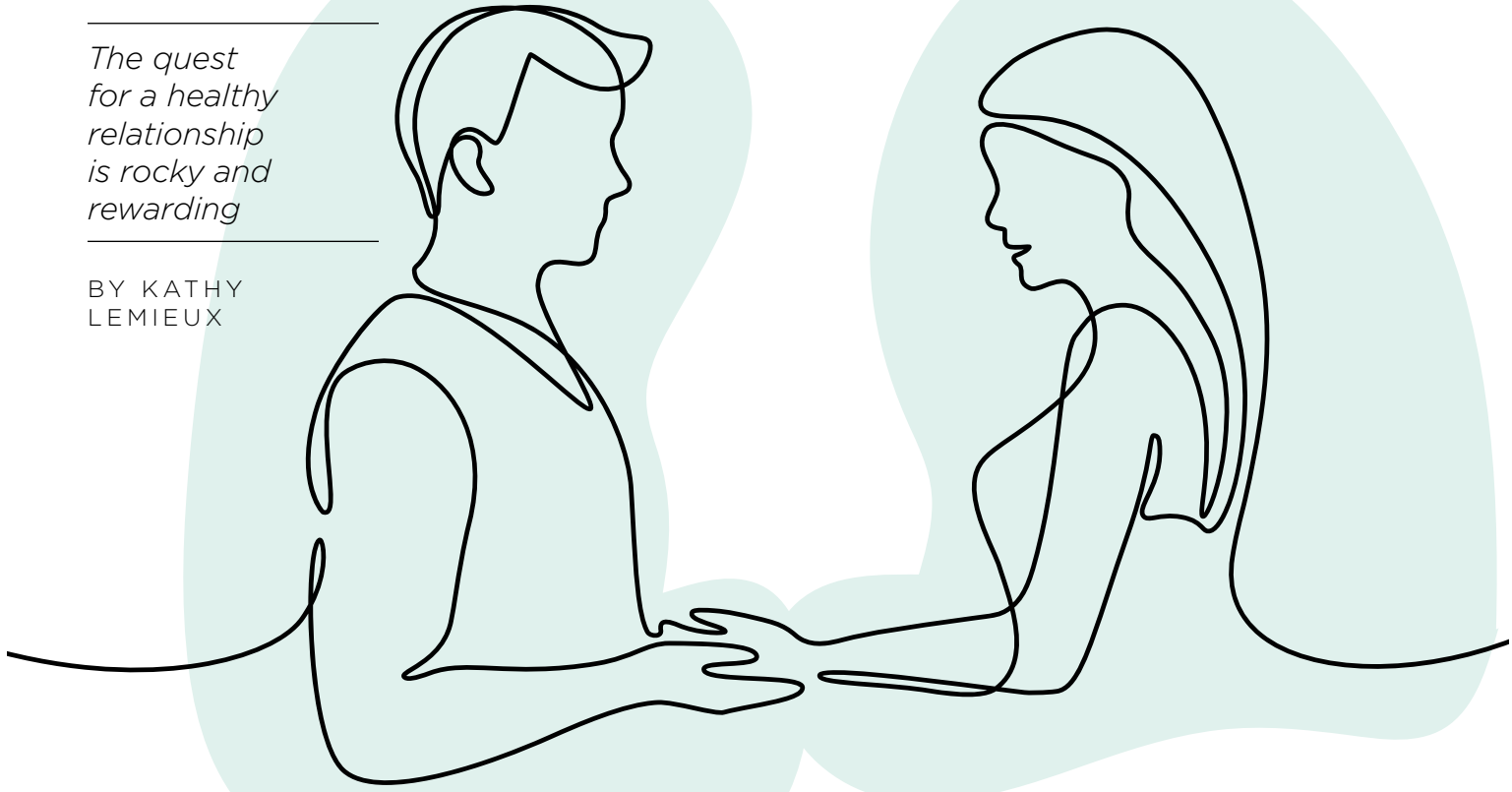
LISTEN to MDA’s Quest Podcast Episode 22, “Creating an Inclusive Work Environment” to hear Sarah Bernard, co-founder and chief operating officer of Inclusively, discuss the organization’s mission and how inclusivity benefits everyone. Find it at MDAQuest.org/podcast.



Real Love

The quest for a healthy relationship is rocky and rewarding

BY KATHY LEMIEUX



Finding love can be difficult for almost anybody. Finding real love when you are someone with a disability can feel like trying to hit the lottery, playing your numbers with all the hopes of a perfect match. Finding real love may feel difficult and risky. My story is a perfect example of the gamble needed to hit beautiful, mutually fulfilling, joyous love. It's finding real love.

Finding my path

As a woman born in the 1970s with spinal muscular atrophy (SMA), finding romantic love was presented to me by society as an unreachable fantasy. Barbie and Ken could have a wildly romantic relationship, but not me. The kind of deep, heartfelt love I saw in movies was unlikely for someone needing such complex physical care as me.

As I grew up, I became confident, outspoken, and a great self-advocate. It was easy for me to mature this way; I was surrounded by strong-willed women. My sisters, two of whom were also born with SMA, taught me every day what's necessary to be seen, heard, and understood.

When I was in college, with lots of friends and social outlets, it seemed like I would have plenty of opportunities to find a special someone. However, if those were the "fish in the sea," even with the best Shimano fishing rod I was

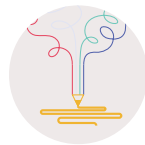
catching bottom feeders too often. I threw back the fish and focused on my education and career, my friends and family, and my own personal happiness. I was certain that was the best path to finding real love.

But in the years that followed, my path was not smooth. Those years were full of beautiful promises ending in broken roads and difficult decisions.

Rousing and rocky

My first marriage lasted almost 10 years. It's difficult to see the path you vowed to walk together forever be rerouted in separate directions. Was he the one who changed so much, or was it me, too?

After a bit of "on again, off again," we met at the courthouse and signed away our promises of forever. That day we got along like best friends.



MORE ONLINE

Dating isn't easy. Read tips for starting relationships and tackling intimacy in the Quest online exclusive "Muscular Dystrophy and Dating," at MDAQuest.org/MD-and-dating.

Image: iStock.com/Tetiama Garkusha

Sometime later I met someone who seemed like my mirror-image in personality, intellect, and character ... or so I thought. We were caught up in a whirlwind. It was so fast that I failed to notice what were surely red flags, until one very hot fourth of July.

There was an air show, and he was at the airport because of his military status. I showed up to say hello. He seemed embarrassed and annoyed. I was confused. I reached over to touch his hand, and he pulled away, bent over, and said something like, "Don't ever wear flip flops in public because your feet don't look right."

Ouch! That was the end of that whirlwind. I may have felt weak in some ways, as I was still healing from a broken marriage, but there was no way I needed his advice about footwear. Shoes were my thing!

These broken relationships and their broken promises left me less interested in finding someone to be with and even more interested in investing in myself.

Rebirth and real love

I tried so hard to keep it all together, but it seemed like the difficult times multiplied after I chose to be single again. I was struggling with finances, health issues, caregivers, and more. I was overwhelmed. On my way to work one day, the weight of it brought me to tears. I was driving and couldn't see through the tears, so I pulled over. I started praying and asking for God's help in my life. By His grace and through my work on myself, I experienced a journey from submission to spiritual rebirth. Wow! This is an incredible, undeserving love, a divine love, and a real love.

My perspective changed, my priorities shifted, and my heart was new. I could approach life from a brand-new angle.

Kathy LeMieux found the right partner in her husband, Dave.



Don't get me wrong, I was still interested in marriage, but I continued with my life, my family, my service dog, my job, and myself, all the while looking for him in my peripherals.


One evening, I was home alone, and I started daydreaming about being in a great relationship. I saw myself leaving work, stopping at the market, and coming in the door with fresh ingredients to prepare a gorgeous, healthy dish that he savored. I quickly snapped out of it when I realized my head was tipped too far forward and I couldn't lift it. Having been in this predicament before, I knew not to panic as that makes it even more difficult to breathe. "My bedtime caregiver should be here soon," I told myself. Thankfully, my wait was short.

This incident made me realize that I can be in a healthy relationship, but I will require help with pretty much all of life's daily tasks, household duties, pet care, career expectations, and self-care. Yes, I was aware of the level of care I require, and I knew a successful relationship would take an understanding partner.

Finally, I met him on a blind date. We had only spoken by phone, and I arrived at the restaurant first. I looked up and saw a tall, chiseled, blonde, tan, sweet-looking man. "Are you Dave?" I asked. "Well, are you Kathy?" he replied. I tilted my head and smiled, amused because I was the only woman in a wheelchair in the restaurant. We ordered lunch, but never ate. We talked, shared stories, asked questions about each other's faith and future hopes, and we laughed a lot. I smiled until my face hurt. I love that pain.

It was a fit. We pursued each other as friends, life partners, and husband and wife.

Eighteen years later, we are still best friends, and we still love to laugh. As my husband, he accepts the many challenges that come with living with SMA. As his wife, I understand his life with cerebral palsy.

Put simply, this is real love. 

Kathy LeMieux has victoriously celebrated 52 birthdays. Based in South Carolina, she is a life coach at Grace with a Glitch (GraceWithaGlitch.com), where she helps guide people with disabilities to define new norms. She also enjoys writing blog posts, letters, and poems.

My perspective changed, my priorities shifted, and my heart was new. I could approach life from a brand-new angle.



A Wealth of Creativity

MDA's Art Collection celebrates the artistic talents of the MDA community

Living with a disability is no barrier to creativity. Proving this is the MDA Art Collection, which includes the artwork on this page. Showcasing the amazing talent of the MDA community, the current collection was established in 1992 and comprises 400 original works by artists living with neuromuscular diseases. The collection showcases the versatility and imagination of artists ages 2 to 84 and spans media from traditional paintings and drawings to digital designs, a collage with corn, and paint applied with wheelchair wheels and artists' feet. Selected art from the collection has been exhibited at prestigious institutes such as the Dallas Museum of Art; Cork Gallery at Lincoln Center in New York; Tucson Museum of Art; Bishop Museum in Honolulu; Chicago Public Library; Fort Lauderdale Museum of Art; Los Angeles Children's Museum; JFK Center at Vanderbilt University in Nashville; and the Capital Children's Museum in Washington, DC.

MDA is excited to continue this legacy and culture of inclusion by bringing the art collection back on the road. Find details and view the art gallery online at mda.org/art.



Brave Men and Women by Jack MacColeman. This artwork was painted to celebrate the 50th anniversary of the partnership between fire fighters and MDA.



Fishing Buddies by Samuel Williams. Samuel, who created this artwork at age 10, served as the 1998 and 1999 MDA State Ambassador for North Carolina.



Forth Right by Cato Givan. This is a portrait of Cato's service dog, Faust.



Breath of Spirit by Carlos Serbia. In this piece, Carlos uses bright colors to represent positive energy, hope, and the passion he puts into everything he does.

Trial opportunity for children living with **Duchenne Muscular Dystrophy** Join the LYNX Trial

LYNX Trial:

Edgewise Therapeutics is currently enrolling in the LYNX trial, a Phase 2 study of EDG-5506, an investigational therapy to treat children with Duchenne. The year long, two-part trial will study the effect of EDG-5506 on safety, biomarkers of muscle damage and functional measures in children living with DMD. There is an initial 12-week placebo-controlled period, followed by a 40-week open-label period during which all participants will receive EDG-5506.

Participants and their caregiver will visit the site approximately 10 times. In the first 16 weeks, site visits occur every 2 to 4 weeks; after that, visits occur every 2 to 3 months.



Investigational Therapy:

EDG-5506 is designed to reduce the contraction-induced muscle damage that occurs in individuals with DMD, with the goal of protecting and preserving muscle function.

Who Can Participate:

- Aged 4 to less than 10 years of age with a documented genetic diagnosis of DMD
- Able to rise from the floor in less than 10 seconds & climb 4 stairs in less than 10 seconds at the start of the study
- On a stable dose of corticosteroids for at least 6 months prior to first dose. May also be on stable (at least 1 year prior to Screening) approved exon-skipping therapy
- Able to meet other criteria as specified

Travel and other resources will be coordinated and provided for eligible participants and their families.

Sites across the United States will enroll for the LYNX trial. For more information, please go to www.clinicaltrials.gov NCT05540860 or contact studies@edgewisetx.com.





Thank You to Our Partners

Together with our generous partners we are raising funds and awareness for families with muscular dystrophy and related diseases. Since 1950, MDA has been proud to team up with caring corporations, organizations, and brands that generate tens of millions of dollars each year through creative campaigns, cause marketing, and year-round special events.