

STAY INFORMED. LIVE EMPOWERED.

Quest

MDAQUEST.ORG ISSUE 4 • 2022



Scan this code for your free Quest subscription!

Advocate for Your Health

Tips for communicating with doctors

READY FOR ANYTHING

Be prepared for natural disasters

SAVVY SEARCHING

How to find reliable research information

ADVERTISEMENT

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
- 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

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.

Genentech

A Member of the Roche Group © 2022 Genentech USA, Inc. All rights reserved. Printed in USA. M-US-00011592(v2.0) 08/2022

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)

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.
- 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 syringes 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 syringes provided by your pharmacist (you should receive 2 identical oral syringes) 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 syringes 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
 - constipation (upper respiratory infection)
 - 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.
- 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). Please see the Discard After date written on the bottle label. (See the **Instructions for Use** that comes with EVRYSDI).

Keep EVRYSDI and all medicines 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.

Genentech

A Member of the Roche Group

EVRYSDI® (risdiplam)

Distributed by:

Genentech, Inc.
A Member of the Roche Group
1 DNA Way
South San Francisco, CA
94080-4990

EVRYSDI is a registered trademark of Genentech, Inc.
M-US-00007143(v3.0)
©2022 Genentech, Inc.
All rights reserved.

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



Muscular Dystrophy Association is the No. 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:
ebeerbohm@mdaUSA.org
MDAQuest.org/advertise

SUBSCRIPTIONS

Sign up for free or change your Quest subscription status at
MDAQuest.org/subscribe.

You also can sign up or change your personal information by calling the MDA Resource Center at **833-ASK-MDA1**.

Quest Volume 29, Issue 4

Published by
Muscular Dystrophy Association
161 N. Clark St., Suite 3550
Chicago, IL 60601
800-572-1717
email: quest@mdaUSA.org
Available on the internet
at MDAQuest.org
ISSN 1087-1578
Postage paid at Bolingbrook, IL
Nonprofit postal permit number 1446.

Postmaster: Treat as Standard
A Mail Only

© 2022, Muscular Dystrophy Association. All rights reserved. MDA and QUEST are registered trademarks of the Muscular Dystrophy Association.

The acceptance of advertising in this magazine does not constitute or imply endorsement by MDA of any product or service. MDA accepts no responsibility for any claims made in any advertisement. Quest reserves the right to refuse to accept any advertisement.

Information contained in Quest may not be reproduced, published, transmitted or distributed in whole or in part without prior written consent of MDA.

Always consult your professional advisers as to how medical, legal or financial information in Quest pertains to you. MDA assumes no liability for any information in Quest.



PUBLICATION MANAGEMENT



GLCDELIVERS.COM

MDA Leading the Way

MDA has long been at the forefront of neuromuscular disease research, leading the way to breakthroughs, advancements, and an impressive number of firsts. Not only are we responsible for a multitude of scientific breakthroughs, but we have accomplished an impressive number of firsts in areas such as care, drug development, advocacy, and even fundraising. For instance:

- MDA was the **1st** organization to focus on all neuromuscular diseases rather than targeting just one disease.
- MDA was the **1st** philanthropic organization to fund research for neuromuscular diseases.
- MDA was the **1st** organization to raise more than \$2 billion for faster breakthroughs.
- The **1st** researcher to discover the gene that causes Duchenne muscular dystrophy (DMD) received an MDA grant.
- The **1st** FDA-approved treatments for spinal muscular atrophy (SMA), amyotrophic lateral sclerosis (ALS), and DMD were funded by MDA.
- MDA was the **1st** to establish a network of multidisciplinary care centers for neuromuscular diseases.
- MDA was the **1st** to populate a neuromuscular disease patient registry with medical records.
- MDA was the **1st** voluntary health organization to hold a free summer camp program for youth with neuromuscular diseases.
- MDA was the **1st** to launch one-of-a-kind fundraising platforms such as a nationally televised telethon.
- MDA was the **1st** neuromuscular disease organization to invest in a drug development venture philanthropy model.
- MDA was the **only** neuromuscular disease organization to serve on the Air Carrier Access Advisory Committee.
- MDA was the **1st** to lead a coalition for MD CARE Act, the historic ACT for ALS, and Newborn Screening Saves Lives Act for neuromuscular disease.
- MDA has been an **innovator** in disability employment and access to care and higher education.

It is thanks to our extremely talented research scientists, clinicians, advocates, and staff, along with our generous supporters and partners, that we have been able to achieve these milestones. We pledge to continue leading the way forward on behalf of the people we serve.

Sincerely,

Donald S. Wood, PhD

President and CEO

Muscular Dystrophy Association



Donald S. Wood, PhD

THANK YOU, READERS

We got incredible feedback from all of you who read Quest in print and online, and listen to the Quest podcast in our 2022 reader survey. See some highlights below.

We'd like to offer special congratulations to Gerry Rhodes of New Albany, Ohio, who won the survey drawing for a \$100 Visa gift card. Readers like Gerry help us make Quest a valuable resource for the MDA community.

99% of readers consider Quest a valuable resource for learning about neuromuscular diseases.

92% live with a neuromuscular disease or have a family member living with one.

75% read or listen to Quest content at least once a month.

Creating a Welcoming Environment

MDA partner Acosta prioritizes inclusivity

During this Year of Independence, MDA is celebrating all those who have overcome challenges to achieve independence, as well as the partners who are empowering people to reach their goals.

MDA's work for the muscular dystrophy community would not be possible without the support of dedicated partners and sponsors. One valuable partner is the sales and marketing company Acosta, which has raised over \$82 million for MDA since 1985 through events like Aisles of Smiles. Acosta's support for the disability community extends far beyond MDA fundraisers; it's evident in the company's dedication to creating empowering employment opportunities for people with disabilities.

Acosta has raised over \$82 million for MDA since 1985.

In 2020, Brian Wynne became Acosta's CEO. A business leader and the father of a child with a disability, Wynne is passionate about furthering Acosta's priorities to foster a welcoming work environment. Learning many lessons from his son, Wynne says he and Acosta are dedicated to increasing opportunities for people with disabilities and fighting stigmas.

"I think it's essential for companies to include people with disabilities for several reasons," he says. "To have a successful business, in any industry, you need the best talent available. And if you want to have the best talent, you have

“To have a successful business, in any industry, you need the best talent available. And if you want to have the best talent, you have to make sure you're including everyone in the talent pool. — Brian Wynne



to make sure you're including everyone in the talent pool — all of the people who have wonderful talents, skills, and abilities, not excluding people with disabilities. I genuinely believe there's no bigger factor to a successful business than having the most diverse and inclusive workplace."

Acosta works to achieve an empowering environment through inclusive hiring practices and consistent team education, such as workshops about disabilities in the workforce, and advocacy and awareness. Wynne advises people with disabilities to initiate conversations about creating inclusive environments.

"If you're a person with a disability, have confidence and talk about your abilities, the challenges you will face, and the minor accommodations that would help you overcome those challenges and help you deliver on the company's missions," Wynne says. "Having those conversations upfront, in a very transparent way, helps make that person successful in their role and helps prevent problems in the future."

Along with being proactive and transparent, Wynne encourages continued education, advocacy, and awareness.

"That's why I think the work that MDA does is so critical," Wynne says. "The more you can introduce people to the talents and gifts of people with disabilities, the better it is for companies, associates, and the person with disabilities. Everyone benefits when we are more inclusive." [Q](#)



Brian Wynne, CEO of Acosta

Closing a Meaningful Year



Mindy Henderson

For our final 2022 issue of *Quest*, we wanted to fill the pages of this magazine with as much useful information, resources, and strategies as we could to help you finish the year empowered.

With articles about advocating for yourself in the doctor's office, staying informed about research and clinical studies, and being prepared for natural disasters, I hope this issue will prove valuable to you.

The holidays are my personal favorite time of the year, but for many, they can also be stressful. In these pages, you'll find updates on accessible air travel and some helpful holiday travel tips. On our *Quest Media* website ([MDAQuest.org](https://www.MDAQuest.org)), we've included an online exclusive about combating holiday stress. As an additional treat, visit the website to check out the first ever MDA Gift and Giving Guide, full of product picks from myself and our community of MDA Ambassadors.

This first year I have spent working for MDA, delivering content to you across **Quest Media — the magazine, the blog, and the podcast** — has been one of the most rewarding and meaningful of my life.

So many of you provided incredible feedback in our annual reader survey this year, and when you speak, we listen! The *Quest* team and I have read every bit of feedback and are hard at work planning bigger, better things for you via *Quest* in 2023.

Wishing you and your families all the best this holiday season!

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



Hillrom™

ADVERTISEMENT



BREATHE EASIER WITH HOSPITAL-LEVEL THERAPY AT HOME

The Volara™ System offers 3-in-1 therapy versatility with Continuous Positive Expiratory Pressure (CPEP), Continuous High Frequency Oscillation (CHFO), and integrated aerosol delivery of medication. Now with the Volara System the same OLE therapy delivered in the hospital is accessible to patients at home.

hillrom.com/volara-patient

Volara™ is a trademark of Hill-Rom Services PTE Ltd.

© 2021 Hill-Rom Services, Inc. ALL RIGHTS RESERVED.
APR279402 rev 1 17-SEP-2021 ENG – US

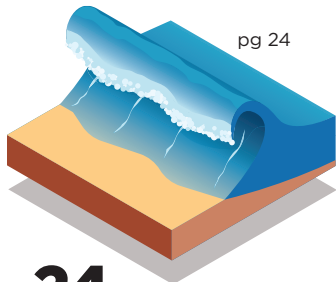


CONTENTS

ISSUE 4 2022

FEATURES

18
Advocate for Your Healthcare Needs
 Be confident speaking up in the doctor's office or hospital.



pg 24

24
Acts of Nature
 Learn how to prepare for your safety during climate-related disasters.

31
Well-Informed
 Follow these guidelines to be a savvy online researcher.

Cover image: iStock.com/DrAfter123

DEPARTMENTS

8
A LOOK INSIDE
 Get an inside view of how we are building back Summer Camp.

10
PROGRESS NOW
 Read about recent medical research, scientific advances, and clinical trials.



pg 10

14
SPOTLIGHT
 Carsten Bönemann, MD, discusses Bethlem myopathy.

16
THRIVE 365
 We're making progress on improving air travel for wheelchair users.



pg 39

38
ACCESS MDA
 Get updates on advocacy, education, and more.

42
FROM WHERE I SIT
 Tomeca Goodwin meets accessibility challenges with creativity.

44
LASTING IMPRESSION
 Our reader photo contest winner took a sensational selfie.

MORE ONLINE

QUEST GIFT & GIVING GUIDE
 Kick off your holiday shopping with the first-ever Quest Gift and Giving Guide, which features MDA Ambassadors' favorite product picks. Find it at MDAQuest.org.



HOLIDAY CHEER
 Anyone can feel overwhelmed by the hustle and bustle of the winter holiday season, but for people living with neuromuscular diseases, managing the additional activities and expenses is crucial to maintaining mental, physical, and financial health. Read helpful tips to minimize stress and maximize the spirit of the season in a Quest online exclusive article at MDAQuest.org/holiday-cheer.

MDA-116

Do You Want to Contribute to Research in Duchenne Muscular Dystrophy? Join the Lynx Trial

The LYNX Trial: LYNX

A year long, two-part trial to study the safety of an investigational therapy called EDG-5506 in children with Duchenne, and to identify EDG-5506 doses for further study that have the potential to best reduce muscle damage. Part A is 12-weeks long and is placebo-controlled. Part B is open label and will be offered to all participants who were involved in Part A, and will allow further study of safety and functional outcomes.



The Investigational Therapy

EDG-5506 is administered as a daily oral dose of one or more tablets, designed to protect injury-susceptible fast skeletal muscle fibers in dystrophinopathies such as DMD and certain other neuromuscular disorders.

Who Can Participate?

- Aged 4 to less than 10 years of age with a documented mutation in the DMD gene and a phenotype consistent with 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 at the start of the study
- Willing and able to travel to one of 12 clinical sites available in the US
- Able to meet other criteria as specified

Travel expenses will be paid for by the Study for eligible participants

Up to 12 sites will be enrolling across the US. For more information please go to www.clinicaltrials.gov NCT05540860 or contact studies@edgewisetx.com

The Return of ‘the Best Week of the Year’

Looking backward to move ahead



MDA Summer Camp in Iowa brought campers together for lifelong memories.

There is no better way to acknowledge the Year of Independence than by looking back at our first in-person MDA Summer Camp sessions in two years. While the pandemic continued to impact organizations around the globe, we were thrilled to safely return to in-person camp, while exploring new adventures through virtual avenues.

Let's take a look back at this incredible journey.

A different approach

Every organization had to adapt due to the pandemic, and MDA was no different. In 2022, our Summer Camps returned, but with a smaller footprint and altered programs intended to keep our campers, amazing volunteers, and dedicated staff safe.

Naturally, we faced some challenges. The ongoing vaccination debate was pivotal in our planning, which was guided by our Medical Advisory Board, who gave the immediate and unanimous recommendation that all camp participants be vaccinated. Our closed-door policy limited some of our favorite activities from previous years, such as the carnival and visits from fire fighters and the media. While we missed these activities, we created new memories.

We also felt the shortage of medical professional volunteers, with many unable to participate, putting their much-needed expertise to work in hospitals around the country while dealing with unprecedented levels of burnout.

The pandemic also impacted our nonmedical volunteers, but the strength of our relationships with colleges and universities, who put the call out for qualified volunteers across their student and teacher populations, firefighters, and veteran volunteers, made it possible to staff and hold 19 of the 24 planned sessions across the country.

Our volunteers are truly special people and are the heart and soul of the program. They readily give their time, focus, and emotions to provide a great experience for our campers.

For the first time in two years, our campers were able to put down their phones, set aside screens, and interact with their peers. Campers spent time outside — they swam, fished, zip lined, learned archery, and rode horses. They indulged their creativity with arts and crafts and talent shows. They shared details of their adventures around the campfire. The dance on the final night is a rite of passage that many campers enjoyed.

+ CAMPERS AND VOLUNTEERS

MDA Summer Camp 2023 registration will open in January at mda.org/summer-camp.

Listening to our community

Hearing feedback from families is always gratifying. A parent shared her daughter's experience, stating: "MDA camp has changed her life. She desperately needed some time away from her everyday life to gain independence and confidence in herself and her capabilities, and this camp has given her that."

Canceling five of the 24 planned sessions was truly painful. However, it was a decision made with the best intentions to keep everyone safe and uphold the integrity of the program.

We faced some behind-the-scenes challenges that could have been show-stoppers, including unknown risk factors, changes in session dates and locations, working with new teams, and adapting to different ways of managing the program. However, knowing how much Summer Camp means to our community, we knew we had to find a way to innovate and make what should have been impossible, possible.

And we did. The protocols we put into place worked. COVID testing upon arrival, use of cohorts for cabins, frequent handwashing, and other thoughtful protocols kept the camp community safe and allowed campers to have fun throughout the summer.

Virtual connections

Virtual Summer Camp continued with 141 virtual campers receiving activity boxes filled with arts and crafts materials, T-shirts, sunglasses, backpacks, and more. Daily Cabin Chat video calls kept our campers engaged with each other, and fun was had by all on Family Game Nights.

While many organizations have suspended virtual options as the pandemic recedes, MDA will continue the virtual camp option. Virtual camp expands our borders and

Our volunteers are truly special people and are the heart and soul of the program. They readily give their time, focus, and emotions to provide a great experience for our campers.

provides a fun, interactive space for those who are not quite ready to go away for a week and those who medically cannot make it to camp but deserve the opportunity to build independence, confidence, and foster lifelong relationships.

New opportunities

We used this challenging time to launch some innovative programs. In partnership with General Motors, MDA introduced STEM Connections, a two-part program focused on STEM concepts. Virtual Summer Campers designed and built their own cars, created creatures with adaptations to live in the wild, made styluses that work with cell phones, and engineered boats.

The other component was a four-week mentoring program designed to help young people with neuromuscular diseases explore STEM disciplines such as mechanical engineering, medicine, and computer science.

STEM careers are growing exponentially, and this program is critical for our young adults looking to their next chapter. Diversity drives innovation, and members of our community have a powerful contribution to make. This program is intended to continue MDA's tradition of making what once seemed impossible, possible and attainable.

Onward!

It was truly rewarding to provide positive experiences while keeping our campers and community safe. We navigated some big challenges and through it all maintained open, honest, and transparent lines of communication with our community through phone calls, emails, and our dedicated staff at the MDA Resource Center.

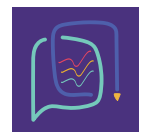
We remain committed to hosting "the Best Week of the Year." [Q](#)



Swimming is a favorite camp activity.



Campers and counselors enjoyed evening activities at MDA Summer Camp in Texas.



MORE ONLINE

Read an expanded article about Summer Camp at [MDAQuest.org/the-return-of-the-best-week-of-summer](https://mdaquest.org/the-return-of-the-best-week-of-summer).

PROGRESS NOW

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

New approvals

FDA Approves Relyvrio for ALS

The US Food and Drug Administration (FDA) approved sodium phenylbutyrate/taursodiol (Relyvrio) for the treatment of amyotrophic lateral sclerosis (ALS) on Sept. 29. Relyvrio (formerly AMX0035) is the third drug approved in the United States for the treatment of ALS.

Relyvrio is a fixed-dose therapy that can be taken orally or by feeding tube and is designed to slow the loss of motor

neurons by blocking cell death pathways.

The FDA approved Relyvrio after the positive results of CENTAUR, a 24-week, multicenter, randomized, double-blind, placebo-controlled phase 2 study of

137 adults with ALS. The people treated with Relyvrio experienced a slower rate of decline compared to those receiving the placebo, and a long-term analysis showed they had longer overall survival.

Relyvrio was generally well-tolerated, with the most common side effects being diarrhea, abdominal pain, nausea, and upper respiratory tract infection.



Learn more at amylyx.com.

CLINICAL TRIAL TERMS TO KNOW

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

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

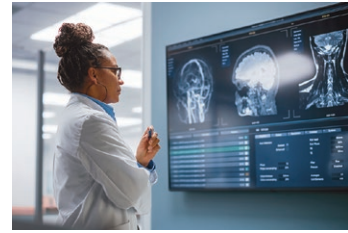
Randomized: The trial is completed at more than one site.

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



Amyotrophic lateral sclerosis (ALS)

Tegoprubart Promising in Phase 2 Trial



Eledon Pharmaceuticals reported positive results from its phase 2a clinical trial of the investigational therapy tegoprubart (formerly AT-1501) for the treatment of ALS. Findings indicated tegoprubart was generally well-tolerated in trial participants, with no drug-related serious adverse events. The drug engaged its molecular target, dampening immune system activation and reducing markers of inflammation.

Tegoprubart is a type of molecule known as a humanized monoclonal antibody. It is designed to restrain the abnormal immune response in ALS by inhibiting CD40 Ligand (CD40L), a protein linked to increased immune response and neuroinflammation in ALS.

The trial evaluated the safety, tolerability, and dose of tegoprubart in 54 adults living with ALS. Participants at 13 sites in the United States and Canada received one of four doses (1, 2, 4, or 8 mg/kg) of the experimental drug via intravenous (into the vein) infusion. Each participant received six infusions of tegoprubart during the 12-week study.

Researchers found higher doses of the drug were associated with greater reductions in immune response. Assessments also indicated a slowing of disease progression measured by changes in a functional rating scale known as the ALSFRS-R.

MDA funding of the ALS Therapy Development Institute (ALS TDI) helped to support foundational research underlying the development of targeted therapies including tegoprubart.

For more information about tegoprubart, visit ir.eledon.com/news-releases.

Image: iStock.com/gorodenkoff

Becker muscular dystrophy (BMD)

Participants Needed for Vamorolone Trial

Researchers are seeking men with BMD, ages 18 to 64, to participate in a phase 2 trial to evaluate the safety, tolerability, and efficacy of vamorolone for treating BMD.

Vamorolone is an anti-inflammatory steroid medication designed to provide the same benefits as traditional corticosteroids, without the unwanted side effects like stunted growth, weight gain, and high blood pressure.

partially functional in BMD, by suppressing certain inflammatory molecules.

Trial participants will be randomly assigned to receive vamorolone or a placebo for 24 weeks. Vamorolone will be given as an oral suspension at a dose of 500 mg daily, or 250 mg if the patient weighs less than 110 lbs. The study plans to enroll individuals at sites in Pittsburgh, Pennsylvania, and Italy.

Researchers will evaluate the therapy's movement into, through, and out of the body, as well as its biochemical effects on the body. Motor function will be evaluated using the Time to Run/Walk Test and the North Star Ambulatory Assessment. The NeuroQOL scales will be used to assess health-related quality of life.

+RECRUITING CLINICAL TRIALS

Find an updated list of trials that are actively recruiting individuals to help advance research and treatment development at mda.org/clinical-trial-updates

Preclinical data have indicated that vamorolone can increase levels of dystrophin, a protein that is only

For information, visit ClinicalTrials.gov and enter **NCT05166109** in the "Other terms" search box.

Duchenne muscular dystrophy (DMD)

MOMENTUM Trial Resumes



Image: iStock.com/JHVEPhoto

The protocol for Part B of the MOMENTUM clinical trial includes magnesium supplementation and monitoring of magnesium levels.

The US Food and Drug Administration (FDA) has removed the clinical hold on SRP-5051 (vesleteplirsen), Sarepta's investigational therapy to treat individuals with DMD who are amenable to exon 51 skipping.

Part B of the MOMENTUM study was placed on hold after a serious adverse event of hypomagnesemia (reduced magnesium levels). Following discussions with

the FDA, Sarepta will adjust the global trial protocol to include expanded monitoring of urine biomarkers, which include magnesium levels.

SRP-5051 uses Sarepta's advanced chemistry and exon-skipping technology to enter cells and skip exon 51 of the DMD gene. Exon skipping is intended to allow cells to produce a shortened, functional dystrophin protein. Around 13% of DMD patients have mutations that make them amenable to skipping exon 51.

MOMENTUM is a phase 2, multiarm, ascending dose trial of SRP-5051, infused monthly. It will assess dystrophin protein levels in skeletal muscle tissue following treatment. Results from Part A of MOMENTUM showed that after 12 weeks, 30 mg/kg of SRP-5051 dosed monthly resulted in 18 times the exon skipping and eight times the dystrophin production as eteplirsen (Exondys 51), an approved DMD therapy that is dosed weekly for 24 weeks.

If successful, SRP-5051 offers the potential for improved efficacy and less frequent dosing for patients.

For more information, visit ClinicalTrials.gov and enter **NCT04004065** in the "Other terms" search box.

ACTING LIKE A 6-YEAR-OLD BECAUSE HE CAN



EMFLAZA® has been shown to preserve muscle strength and function

Improved muscle strength

A clinical trial of 196 boys aged 5 to 15 with Duchenne muscular dystrophy (DMD) assessed the effectiveness and safety of EMFLAZA vs placebo (sugar pills) and prednisone. EMFLAZA improved muscle strength at 12 weeks vs placebo.

In a long-term study of steroids, deflazacort delayed disease progression by years

Not actual patients.



Loss of ability to stand from a lying-on-the-back position
—delayed by 2.1 years vs prednisone



Loss of ambulation (ability to walk)
—delayed by 2.7 years vs prednisone



Loss of hand-to-mouth function with retained hand function
—delayed by 2.7 years vs prednisone

STUDY INFORMATION

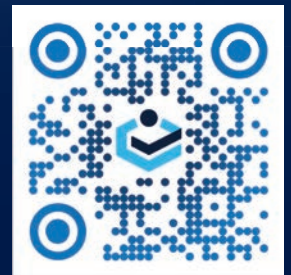
Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study.

Objective: This study examined the long-term effects of glucocorticoids on milestone-related disease progression across the lifespan and survival in patients with DMD

Methods: For this prospective cohort study, male patients aged 2 - 28 years with DMD were enrolled at 20 centers in nine countries. Patients were followed up for 10 years. The study measured the progression of nine mobility and upper limb milestones to compare no glucocorticoid treatment or cumulative treatment duration of less than 1 month versus treatment of 1 year or longer.

Results: 440 patients were enrolled during two recruitment periods (2006 - 09 and 2012 - 16). Time to all disease progression milestone events was significantly longer in patients treated with glucocorticoids for 1 year or longer than in patients treated for less than 1 month or never treated (log-rank). Glucocorticoid treatment for 1 year or longer was associated with increased median age at loss of mobility milestones by 2.1 - 4.4 years and upper limb milestones by 2.8 - 8.0 years compared with treatment for less than 1 month. Deflazacort was associated with increased median age at loss of three milestones by 2.1 - 2.7 years in comparison with prednisone or prednisolone (log-rank).

PLEASE NOTE This study is not in the approved prescribing label for EMFLAZA, but is consistent with the information that is included. Please talk to your son's healthcare provider if you have any questions.



Emflaza®
(deflazacort)

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

INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

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?

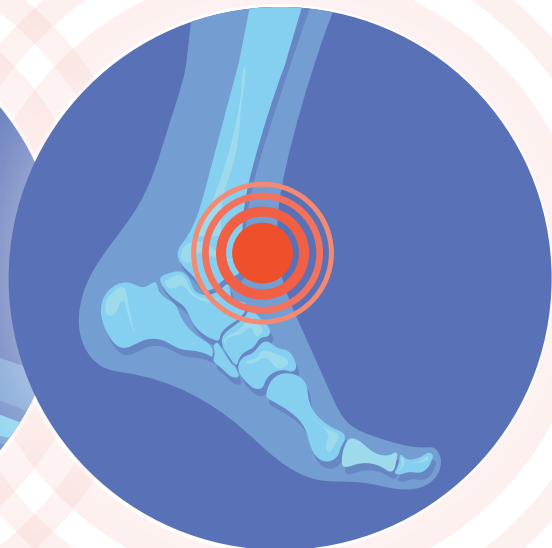
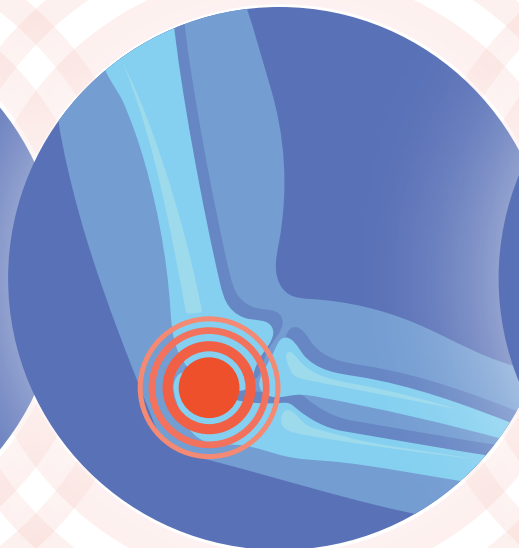
The most common side effects of EMFLAZA include facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds. These are not all of the possible side effects of EMFLAZA. 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.

Emflaza® is a registered trademark of PTC Therapeutics, Inc. © 2022 PTC Therapeutics, Inc. All Rights Reserved. US-EMF-0292v2 03/22

Understanding Bethlem Myopathy

A Q&A with Carsten Bönnemann, MD



Bethlem myopathy, also called Bethlem muscular dystrophy, is a rare disease affecting skeletal muscles and connective tissue. Considered a type of congenital muscular dystrophy, it was initially recognized in the 1970s by two Dutch physicians, Jaap Bethlem and George K. van Wijngaarden, and is characterized by slowly progressing muscle weakness and joint stiffness of the fingers, wrists, elbows, and ankles. The progressive weakness can eventually affect ambulation (the ability to walk and move around) and respiratory function. The severity of the weakness and stiffness can vary greatly, however. In addition, some may experience joint pain, which is thought to involve the joint connective tissue and cartilage, but this has not been studied extensively.

Symptoms of Bethlem myopathy can begin in any age group and can even be noticed before birth through decreased fetal movement. It is estimated that fewer than 5,000 people in the United States live with the condition.

To learn more about Bethlem myopathy, we spoke with Carsten Bönnemann, MD, senior investigator and chief of the Neuromuscular and Neurogenetic Disorders of Childhood Section at the National Institutes of Health (NIH).

What is Bethlem myopathy?

It is a degenerative muscle disease caused by mutations in one of the three genes, *COL6A1*, *COL6A2*, and *COL6A3*, that specify the genetic code for a protein called collagen type VI. Collagen VI is an important component of the extracellular muscle matrix (ECM) — the substance that surrounds the cells of a tissue, such as muscle, and provides physical and biochemical support. The ECM also is found in other tissues such as skin, tendons, and joints.

Although symptoms are frequently evident in early childhood, the disease course is prolonged, allowing for ambulation into adulthood.

When it runs in families, Bethlem myopathy is typically inherited in an autosomal dominant manner, which means a person has to inherit a flawed gene from only one parent to have disease symptoms.



Carsten Bönnemann, MD

How does it fit into the congenital muscular dystrophy category?

Mutations in the same three collagen VI genes that lead to Bethlem myopathy can also cause a more severe condition referred to as Ullrich congenital muscular dystrophy (UCMD). Collagen VI-related muscular dystrophies occur within a spectrum of severity, with UCMD on the severe end and Bethlem representing the milder end, with cases of intermediate severity connecting the two ends of the spectrum.

As noted by Bethlem and Wijngaarden, Bethlem myopathy can also be clinically evident at birth or in early childhood, just with considerably milder symptoms compared to UCMD. In other individuals, it may only become evident later in life. But there are no fundamental differences in the disease's causation; it is just the degree of severity with which it plays out.

What is the current standard of care?

The standard of care includes careful and proactive monitoring for pulmonary (lung) and orthopedic (musculoskeletal) complications, as well as physical therapy that includes moderate exercise and stretching. Sometimes surgical interventions are performed, such as lengthening the Achilles tendons, which often show the most prominent contractures.

Rare diseases can be difficult to diagnose. Are there any recent or upcoming advances that could streamline the diagnostic journey?

The characteristic combination of weakness and multiple joint stiffness results in a suggestive clinical picture that should help clinicians suspect a diagnosis of Bethlem myopathy upon physical examination. In addition, certain patterns of muscle involvement on imaging tests (such as with muscle MRI) have also been highly suggestive of the diagnosis.

But even without a specific clinical suspicion that the disease might be present, the more common use of gene panel and whole exome testing now leads to the discovery of the disease-causing mutations and, hence, a diagnosis. On the other hand, the same type of testing also frequently results in the detection of "variants of uncertain significance" in the collagen type VI genes, which must then be further evaluated by a specialist in the clinical context of the patient to avoid a misdiagnosis.

Are there any promising therapies on the horizon?

There are exciting new developments in gene-directed therapies based on the concept that inactivating a dominantly inherited mutation will result in significant improvement of disease symptoms.

Joint contractures (stiffness) can cause additional problems with mobility and hand function. Lung function may become progressively impaired later in the disease.

In Bethlem myopathy, effective inactivation of the flawed copy of the disease-causing gene would allow the other healthy copy of the gene to produce collagen VI in an undisturbed manner, which should be enough to maintain good collagen VI matrices. These approaches are still in the preclinical phase and are advancing toward testing in animal models. An important hurdle to overcome is effective targeting of the cells that make the ECM in muscle, because it is in these cells that collagen type VI is made. So it will still be a few years until one of these therapies is ready to be tested in people, but sometimes breakthroughs can happen quickly. [Q](#)



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

ADVERTISEMENT

Take control of your health!

- Eliminate sleep deprivation
- Prevent painful and life-threatening pressure injuries (bed sores)
- Prevent pneumonia & other respiratory problems

**ALEXA VOICE CONTROL
OPTION AVAILABLE!**

The Freedom Bed is the most advanced patient positioning system on the market, providing:

- Automated Body Rotation
- Programmable Times & Angles
- Smooth And Silent Operation
- Uninterrupted Sleep



HEAD & LEG ELEVATION



LATERAL ROTATION

CALL TOLL FREE: 800.816.8243
EMAIL: INFO@PRO-BED.COM
WEB: WWW.PRO-BED.COM



The Fight to Stay Seated

When will air travel for passengers with wheelchairs get better?

BY SHAILA WUNDERLICH



Image: iStock.com/miakleiv

A July 2022 report from the US Department of Transportation cited a 108% increase in complaints from flyers with disabilities compared to May 2019.

In September 2022, American Airlines stranded passenger Xavi Santiago for more than five hours at the Orlando airport after misplacing their power wheelchair. One month earlier, Lufthansa stopped Rhode Island congressman Jim Langevin from boarding his flight to Italy, citing unwarranted safety concerns over his power wheelchair battery.

News reports of the challenges of air travel with a disability are nothing new to travelers in wheelchairs. Since the Air Carrier Access Act of 1986 was passed, airlines have been prohibited from discriminating on the basis of disability, but they do require passengers to check their personal wheelchairs, board in manual “aisle chairs,” and transfer to regular on-board seating. The result? Innumerable injuries, accidents, damages, and missed and delayed flights.

“The things we hear about most often are wheelchairs being damaged, flight crew dropping or injuring people during seat transfers, and restroom inaccessibility,” says Michael Lewis, MDA’s director of disability policy. “It’s common practice for people to dehydrate themselves in order to avoid using the restroom during flight.”

These personal anecdotes don’t take into account the number of people in wheelchairs who avoid flying altogether, which a 2021 survey conducted by Paralyzed Veterans of America estimates at around 67%.

Fortunately, the world is starting to become more aware of the problem, fueled by increased media coverage, hyper-focused advocacy groups, and a small group of determined



HOLIDAY TRAVEL TIPS

The number of travelers flying for the winter holidays in 2022 is expected to exceed pre-pandemic levels. With major airlines cutting schedules, that's sure to lead to flight disruptions. Here are tips to help you arrive at your destination with your holiday cheer intact.

- 1 **Call ahead.** Ask for any accommodations or services you need when you book, and call the airline 24 to 48 hours before your flight to confirm.
- 2 **Prepare for delays.** Travel with extra medications and any equipment you might need if you are at the airport or your destination longer than planned.
- 3 **Attach instructions.** Until the rules change, you'll have to check your wheelchair. Write out key instructions for handling your wheelchair, like parts not to remove or fold, and laminate it and attach it to your chair.
- 4 **Bring a wheelchair repair kit.** Assemble a small kit of spare parts and tools for emergency repairs.
- 5 **Know your rights.** Review the Airline Passengers with Disabilities Bill of Rights at [transportation.gov](https://www.transportation.gov).

legislators. “Since I’ve been here, this is the most we’ve talked about and worked on air travel,” says Mark Fisher, director of advocacy engagement at MDA. “In the news and on social media, it’s the most it’s been picked up.”

Signs of progress

A July 2022 report from the US Department of Transportation (DOT) cited a 108% increase in complaints from flyers with disabilities compared to May 2019. “There is more momentum around this issue now than we’ve seen in quite a while,” says Heather Ansley, associate executive director of government relations for Paralyzed Veterans of America (pva.org), a key advocate in the movement for accessible air travel. “I’m getting more stories sent to me about the experiences of wheelchair users, and it does seem the media is becoming more aware.”

Together, these forces have affected several major developments toward the cause of improving wheelchair travel accessibility. One such development, an October 2021 report published by the Transportation Research Board, studied the feasibility of a “wheelchair securement concept” that would allow passengers in wheelchairs to remain in their own devices during flight — similar to what city buses, Amtrak trains, and subway systems already do. The study ultimately found no significant, deal-breaking barriers to the concept.

“The biggest takeaway is that a wheelchair spot on planes is technically feasible,” says Michele Erwin, founder and president of All Wheels Up (AllWheelsUp.org), a nonprofit organization dedicated to the right and ability of people in wheelchairs to independently maneuver themselves on

and off airplanes in their own devices. “All the red tape has been cut, and we now have a clearer path to true accessible air travel.”

Another recent development includes the DOT’s summer 2022 release of an Airline Pas-

+ADVOCATING FOR CHANGE

Learn more about what MDA is doing to help improve air travel for individuals with disabilities and how you can join the effort at mda.org/advocacy.

sengers with Disabilities Bill of Rights. “This doesn’t establish new rights, but it does translate existing law in a way for the average traveler to clearly understand what they’re supposed to be getting from the airlines,” says Heather, who recommends passengers with disabilities review the document before making flight arrangements. (Find it at transportation.gov.)


Make your voice heard

Heather and her colleagues encourage the public to speak now to their federal lawmakers on the matter, as the government is crafting reforms in preparation for the Federal

Aviation Administration’s (FAA) November 2023 reauthorization. “Reauthorization has to happen every five years, or planes don’t fly,” Mark says. “In a bill like that, it’s not uncommon that reforms are added.”

Because the proposed changes to flying with wheelchairs would entail redesigning commercial aircraft, deliverables on this issue promise to be slow. Ideally, the pending FAA reauthorization bill will act as the official green light to start the process.

What can you do to speed the process? “Stay in contact with your members of congress, tell your airline travel story, and push for accessibility priorities to be included in this FAA reauthorization bill,” Michael says.

“Results are never quick enough, especially in cases like this,” Mark says. “But I do believe we’re poised to see some reform very soon.” 

Shaila Wunderlich is a St. Louis-based writer with more than 20 years’ experience in the publishing industry.

Advocate



for Your Healthcare Needs

Speaking up for yourself or a loved one in the hospital or doctor's office helps you get the best care

BY DONNA ALBRECHT

Advocating for yourself when you are in a health-care setting means communicating what you need or want to the doctors, nurses, and other providers who can help you get it. That sounds simple, but in practice it can be intimidating or confusing. Fortunately, it is a skill you can learn.

Barriers to self-advocacy

What keeps people from advocating for themselves or a loved one when they're in a hospital or doctor's office? Vovanti Jones, MD, director of physical medicine and rehabilitation at the MDA Care Center at the University of Missouri has found the main reasons people are reluctant to speak up include:

1. Fear of going against an authority figure
2. Lack of knowledge about a medical condition or treatment
3. Lack of financial resources
4. Lack of a support network



Vovanti Jones, MD

Don't hold back

Dr. Jones brings more than her knowledge and experience as a doctor to this topic — she lives with limb-girdle muscular dystrophy (LGMD) and uses a mobility scooter to get around the hospital.

Dr. Jones understands the fear of going against authority figures because she has experienced it in her own professional life.

Inserting a central venous catheter is a medical procedure a doctor generally performs while standing, but she felt she could do it better sitting on her scooter. “I was afraid of how my request for this change would be taken and how that would impact me in my job, so it took years for me to speak

“

I was afraid of how my request for [an accommodation] would be taken and how that would impact me in my job, so it took years for me to speak up for myself.

— Vovanti Jones, MD

up for myself,” Dr. Jones says. Finally, with the support of senior resident physicians, she began performing the procedures from her scooter. “I only regret that I didn’t speak up earlier,” she says.

Like Dr. Jones, many people worry that requesting an accommodation or asking too many questions can have negative consequences. But Andrew Kufta, 35, director of marketing and communications for the Governor’s Prevention Partnership in Connecticut, who lives with facioscapulohumeral muscular dystrophy (FSHD), says asserting yourself generally has the opposite effect.

“Don’t look at your efforts at self-advocacy as making trouble,” he says. “When people understand why you are asking for what you need, they’re much more likely to respond positively.”

Recently, Andrew was at an MDA Care Center and had trouble standing up from



Andrew Kufta

HOW TO PREPARE FOR A DOCTOR'S APPOINTMENT

Having a plan and supporting materials can help you make the most of your appointment, whether you are seeing a new doctor or an established member of your care team. Here are tips to prepare for your visit:

- Make a list of everything you want to discuss. This may include new or ongoing symptoms, medication side effects, and questions about your treatment plan. Don't put off any pressing concerns until the end of your appointment — bring them up right away.
- Gather any test results or medical records that the doctor hasn't seen before. Be prepared to share your health history and the names and phone numbers of any other doctors you've seen.
- Bring a list of all your medications. Include any over-the-counter medicines, vitamins, and herbal supplements you take, so your providers can flag potential interactions between substances.
- Bring paper or a device to take notes. Medical appointments can be overwhelming, especially when dealing with a new or scary diagnosis. Sometimes it's best to have a support person with you to take notes or help you retain information.
- Before the day of the appointment, make sure you know how to get to the doctor's office, where to park, and how much it will cost. This will help you arrive on time and relaxed.

the chair in the waiting room. He mentioned to the clinic staff that the low chairs were difficult for him and others with muscle weakness. Far from being annoyed, they were grateful that he pointed out the problem, and they addressed it by adding seating aids.

Open communication

Asking questions and having discussions with your medical team reinforces to them that you have done your homework and want to be a partner in your care. Doctors are humans, too, and should be open to having their ideas challenged in the interest of helping you find the best solutions.

Denise and John Szymczak of Racine, Wisconsin, have two teens living with LGMD: Holly, 14, and Joshua, 17. Joshua, a high school senior, has received oral steroids for many years for a condition not related to LGMD. Over time, John says, “We noticed that Joshua has maintained and even gained strength in some areas. We asked Holly’s MDA Care Center team if steroids would help her.” Corticosteroid treatment is common for Duchenne muscular dystrophy (DMD) and is being studied for use with LGMD. After consulting the research, Holly’s care team agreed that it was worth trying. She has received steroid treatment for about three years, and while Holly hasn’t seen the same improvements Josh has, the



family appreciates that her care team listened to them and considered their wishes — as well as the latest research and Holly’s safety — in her treatment plan.



The Szymczak family

Doctor-patient relationships

Dr. Jones notes that mutual respect impacts the quality of communication between patients and providers. You should trust that your doctor is knowledgeable and that your health is their top priority. Your doctor should listen when you describe symptoms and express your wishes and concerns. If a doctor is not meeting your needs, you have the right to find a different provider.

“Even in a good doctor-patient relationship, you may want to get a second opinion to gather more information about your diagnosis and treatment options,” Dr. Jones says. Second opinions are common in medical care, and you shouldn’t be afraid of offending your doctor by asking for one. In fact, your doctor may be able to help by recommending other physicians, filing a second opinion request with your health insurance, or providing your medical records and test results for the second doctor.

Denise and John Szymczak appreciate that the care team listened to them and considered their wishes — as well as the latest research and Holly’s safety — in her treatment plan.

It's likely that many of the healthcare providers you see outside of an MDA Care Center don't have extensive experience with neuromuscular diseases. You may need to take some time to educate your providers about your disease and how it affects your daily life. Bringing disease fact sheets and other educational materials to your appointments shows your providers that you are engaged and looking for them to bring their best to your care. (Find disease fact sheets and other educational materials at mda.org/education.)



“

A lot of what we do is education about what has worked for other patients in similar situations.

— Jaclyn Omura, MD

“

I have never had a parent come to me with too many questions or requests when they came with love for their child.

— Craig Zaidman, MD



Gaining knowledge

It's difficult to advocate for yourself when you don't know what you can advocate for. Jaclyn Omura, MD, Rehabilitation Medicine Physician at the MDA Care Center at Seattle Children's Hospital, finds this is a barrier not only for families dealing with a new diagnosis, but also when a disease progresses, requiring new services or support. "A lot of what we do is education about what has worked for other patients in similar situations," she says.

To effectively advocate for yourself, you need to understand your diagnosis and treatment options. (Read "Well-Informed" on page 31 to learn how to find reliable information on neuromuscular conditions, treatments, and the latest research.)

Craig Zaidman, MD, is director of the pediatric MDA Care Center at Washington University. Good advocacy, to him, includes having parents come with specific goals in mind after they have done some reading about what achieving those goals will entail. "The parent knows their child best, and I appreciate it when they let me know when they disagree with me or need something more from me," he says. "I have never had a parent come to me with too many

To effectively advocate for yourself, you need to understand your diagnosis and treatment options.



questions or requests when they came with love for their child.”

When in doubt, ask. There are no silly questions when it comes to your health.

Financial resources and insurance

Today’s healthcare system is complex, and your healthcare team can be an amazing resource. If you’re worried about how you’ll pay for your healthcare, including medications and treatments, share those concerns with your doctors and nurses. “Often we can provide resources in clinic to help patients navigate the insurance application process and know which treatment options being considered are available for funding,” Dr. Omura says.

If you go to an MDA Care Center, a social worker can help you navigate the healthcare landscape. They typically have extensive knowledge of available insurance programs and private funding. Many hospitals have a patient advocate who can help determine if you are eligible for government programs and reimbursement for covered services.

To learn more about health insurance, take the MDA Access Workshop Access to Coverage: Health Insurance at mda.org/AccessWorkshops.

Support networks

A support network generally is an informal collection of friends, family, neighbors, and co-workers — the people you turn to when you need a sounding board or helping hand. These connections can help give you the emotional strength you need to be a strong self-advocate.

They also can be allies in stressful healthcare situations. Consider bringing a family member or friend to appointments. Be clear with your support person beforehand about what role you’d like them to play: Are they there to help with physical transfers or moral support if you feel nervous? Or do you want them to speak up to support your requests or ask questions?

Finding other individuals or families with similar experiences is a great way to build your support network. MDA’s National Connections Program offers a way for people in the neuromuscular disease community to connect with others living with neuromuscular diseases, caregivers, parents, spouses,



EMERGENCY! BE READY FOR THE UNEXPECTED

Emergency department staff are trained to be cool under pressure, but they may not know much about neuromuscular diseases. In some cases, this can lead them to overlook signs of distress or give incorrect treatment. The best way to be your own advocate in an emergency health situation is to prepare before it happens.

Visit MDA’s Education Materials webpage and scroll down to Emergency Care Resources to find alert cards listing specific precautions and medication safety information that are designed to be printed and kept with you at all times in a wallet, purse, or backpack. Download them at mda.org/education.

For more tips and stories from the community about emergency care, read the Quest article “Be Prepared” at MDAQuest.org/be-prepared.

or siblings. To join the program, contact MDA’s Resource Center at **833-ASK-MDA1** or ResourceCenter@mdaUSA.org. You can request to be matched based on criteria like diagnosis, age, or interests.

You can also expand your network by joining support groups, participating in disease-specific online forums, and volunteering.

When you have healthcare providers you can count on to listen to you and help you understand your condition and treatment options, consider them part of your support network, too. You have everything to gain by advocating for yourself or a loved one in healthcare settings. [Q](#)

Donna Albrecht is a health writer in the San Francisco Bay area. She and her husband had two wonderful daughters who lived with spinal muscular atrophy (SMA).




Did You Know?

MDA is committed to providing program options for youth with neuromuscular diseases to build independence, confidence, and lifelong relationships. With support from General Motors, MDA integrates science, technology, engineering, and math (STEM) activities into Summer Camp and the month-long STEM Connections mentoring program for teens and young adults. This year’s program includes a special session with the Smithsonian Science Education Center. Learn more at mda.org/young-adults/stem-connections and mda.org/summer-camp.

Acts





Climate-related disasters are on the rise. It's vital to prepare yourself and your community.

BY MYRNA TRAYLOR

of Nature

When Hurricane Ian ripped through Florida in September, an estimated 1.2 million people with disabilities were impacted by the devastating winds and flooding, and many of them needed disability-specific aid or accessible services.

Climate-related disasters like Hurricane Ian are becoming more frequent and widespread. And while natural disasters affect everyone in their path, people with disabilities are 2-to-4 times more likely to die or be injured in a disaster, emergency, or crisis than nondisabled people, according to the Partnership for Inclusive Disaster Strategies.

In this environment, it is more important than ever for those with disabilities and their families, caregivers, and communities to make disaster-preparedness plans and establish connections with local and state responders.

Disability rights activist Judith Heumann has experience in this area, both as a private citizen and a government official. She has served in a number of influential positions, including as the US State Department's special advisor on international disability rights from 2010 to 2017.

"This should not be seen as a disability issue alone," Judith says. Taking active steps to prepare for disasters not only increases your chances of getting through one but also alerts responders and officials in your community to the needs of people with disabilities. Increasing awareness of those needs before a disaster occurs can lead to better outcomes for the whole community.

Preparing communities

Judith advises everyone — disabled and not — to learn how emergency management will occur in their community. “Do you know what jurisdiction you’re in? Do you know what the preparedness plans are? Have you made yourself known to various emergency entities?” she asks.

Fortunately, some organizations are tackling the issue of how disabled people can make sure their needs will be met. One such group, the Partnership for Inclusive Disaster Strategies (DisasterStrategies.org), is a disability-led organization focused entirely on disaster preparedness and response. Its co-executive directors, Germán Parodi and Shaylin Sluzalis, offer an expert understanding of the history and current state of preparedness as it affects people with different access and functional needs.

“Historically, emergency management has been inherently ableist,” Germán says. “It excludes people with disabilities. It was built in a system of survival of the fittest; you had to get yourself to a distribution point or evacuation center. Maybe 70 years ago that was the understanding, but today, in 2022, we need to respond better to the increase in disasters’ intensity and frequency — not only with tornadoes, hurricanes, and wildfires, but extreme heat that’s been ongoing across the country.”

“It’s not just government, but the different relief organizations who likewise are not knowledgeable about preparedness inclusive of disabled people,” Judith says. This makes it especially important to speak up and make the disability community’s needs known before a disaster occurs.

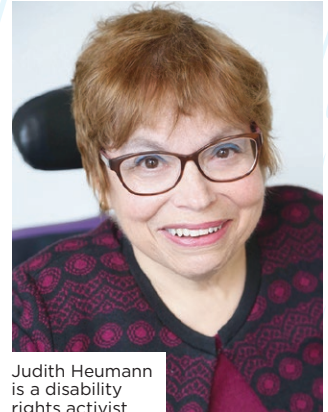


QUEST PODCAST

Listen to a conversation with Judith Heumann about how she became a disability rights activist and the issues she’s currently passionate about at MDAQuest.org/podcast/JudithHeumann.

Here are four actions you can take now:

1. Educate yourself about potential hazards in your area using the Federal Emergency Management Agency’s (FEMA’s) National Risk Index at hazards.fema.gov/nri.
2. Learn what services your local emergency management agency provides and alert them about services you or others in your community need. Find your emergency management agency at usa.gov/state-emergency-management.
3. Ask your workplaces and schools about their emergency plans and make sure those plans include people with disabilities.
4. Find out if your state or city offers an emergency registry to self-identify as someone who needs special assistance in the event of an emergency. Ask your emergency management office or local fire station how to register.



Judith Heumann is a disability rights activist.

EMERGENCY CHECKLIST FOR PEOPLE WITH NEUROMUSCULAR DISEASES

- Create a disaster supply kit and escape plan.
- Prepare a list of your medications.
- Keep a list of the style and serial numbers of your medical devices.
- Send copies of important documents to an out-of-town contact person in case the originals are lost or destroyed in a disaster. Or save digital copies in the cloud so you can access them from anywhere.
- Ask your local emergency management office which shelters are prepared to accept people with disabilities.
- Have a pet care plan. Shelters don’t always accept pets, but they must accept service animals. Contact your local animal shelter.
- Have an extra battery for a motorized wheelchair or scooter. Know how to recharge the battery.
- Have a patch kit, can of seal-in-air product, and inner tubes for a mobility device with inflatable tires.
- Have a lightweight manual wheelchair for backup.
- Have a converter for your communication device.
- Pack a low-tech communication board and preprinted key phrases in your disaster kit.
- Have an adapter kit for your ventilator so it can be plugged into your car or a marine battery.
- Contact your electricity provider to see if they offer a “priority reconnection service” that will help ensure your power is restored as soon as possible.



Image: iStock.com/Macrovector



Image: iStock.com/Macrovector

Importantly, Shaylin and Germán note that emergency registries are most effective in the event of a personal emergency, such as a house fire, but are limited in the case of a large-scale disaster when resources are stretched. They caution that you should not count on a registry to mean that responders are aware of your needs. Be proactive about seeking the help you need in an emergency.

Pushing for improvement

In many states, emergency management departments are now listening when community members explain their differing needs regarding disaster preparedness and response. “There is even a term in use for it: ‘access and functional needs,’” Germán says. “In the emergency response world, that term covers not only people with disabilities, but older adults, children, people with low English proficiency, pregnant people — what some might call vulnerable communities.”

After years of advocacy on these issues by disability-rights advocates, things are getting better.

“We see improvement in areas where there is a position within emergency management agencies that is specific to the needs of people with disabilities,” Shaylin says. “Access and functional needs coordinators within emergency management agencies make sure that the agency is being inclusive in planning, mitigation, and response and recovery. They act as liaisons with the disability community and push their needs up the chain and throughout the agency.”

Germán offers an example of how, when a state is properly engaged on this issue, significant improvements are achievable. “We can point to the 2021 Marshall Fire in Colorado where there is an excellent access and functional needs coordinator who quickly engaged the disability community-led organizations, Spanish-speaking residents, and people on tribal lands to make sure their needs were being identified and sent up to emergency management

EMERGENCY PREPAREDNESS RESOURCES

MDA Emergency Resources: Check out MDA’s list of emergency resources tailored to the neuromuscular disease community at mda.org/care/resource-list/emergency-resources.

Disability and Disaster Hotline: The Partnership for Inclusive Disaster Strategies runs a hotline to provide information, referrals, and guidance to individuals with disabilities and people and organizations assisting individuals with disabilities who are impacted by disasters. Visit DisasterStrategies.org/hotline or call **800-626-4959**.

Emergency Alerts: Sign up to be notified of approaching weather-related and other emergencies at ready.gov/alerts.

Ready.gov: The US Department of Homeland Security’s emergency preparedness website includes information on preparing for disasters and emergencies with disabilities. Visit ready.gov/disability.

operations as quickly as possible,” he says. “Then the disability community organizations themselves were engaged and the immediacy in response was quicker than we’ve ever seen before.”

The Partnership for Inclusive Disaster Strategies engages with emergency managers across the nation and generally finds they are open to discussing the needs of people with disabilities.

“Emergency response starts at the local level,” Germán says. “Immersing ourselves as people with disabilities in emergency management conversations and contacting local emergency management agencies is vital.” [Q](#)

Myrna Traylor is writer and editor for Quest.

Shaylin Sluzalis (left) and Germán Parodi direct the Partnership for Inclusive Disaster Strategies.



Did You Know?

MDA Summer Camp partners with fire fighters and fire departments across the country to ensure a safe and quality experience for campers. Fire departments support camp directors in planning for situations that might occur, monitor weather and air quality, and are prepared to respond if an emergency arises. Visit mda.org/summer-camp.



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:



*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.



VYVGART is a registered trademark of argenx.

For U.S. audiences only.

© 2022 argenx
 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> | | <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>• 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> | <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>Can VYVGART be used in children?</p> <p>The safety and efficacy in children (pediatric patients) have not been established.</p> |



Manufactured by: argenx BV, Industriepark 7, 9052 Zwijnaarde, Belgium
Distributed by: argenx US, Inc., 33 Arch Street, Boston, MA 02110 License Number 2217
VYVGART is a registered trademark of argenx BV. For U.S. audiences only.
© 2022 argenx BV US-VYV-22-00075 V2 06/2022

Well-Informed

How to find reliable information about neuromuscular diseases and research

BY AMY BERNSTEIN

For individuals living with neuromuscular diseases and their families and caregivers, the internet is an incredible resource for finding information about diseases, treatments, and biomedical research, putting a seemingly unlimited amount of information at your fingertips. Social media brings the internet to you, allowing you to learn from likeminded individuals and organizations and engage with campaigns and causes.

And yet, despite this wealth of websites and digital tools, finding the exact information you want — such as the latest research on a treatment or a how to enroll in a clinical trial — can feel like an impossible task.

Part of the problem is that not all health information on the internet and social media is good information — plenty of people post health advice that is misleading or flat out wrong. In fact, in one study, nearly half the pages returned in a search about a specific life-threatening condition included treatment information without citing any scientific backing. “Social media in particular is linked to misinformation spread by nonprofessionals and/or predatory companies who try to sell unproven therapies,” says Angela Lek, PhD, MDA’s vice president of research.

When it comes to your health, it’s important to have information that empowers you to make good decisions.



3 steps to good information

Dr. Lek offers three guidelines for staying well-informed on neuromuscular diseases and research:

1. Evaluate health information you find through the internet or social media.
2. Establish a trusted network of institutes and organizations that you read information from.
3. When in doubt, ask an expert in the field, such as a doctor, scientist, or patient advocacy organization.

How to evaluate health information

Examine any health-related information you find on the internet or social media for trustworthiness by asking three simple questions:

Who

Don't take what you read at face value; look for the original source of the information. Many reliable online resources cite their scientific sources at the bottom of the page or note if an expert has reviewed and approved the information. Any site that does not make it easy to learn who is responsible for the site and its information should be viewed with skepticism.

Why

Think about the intent of the organization or individual providing the information. Are they presenting opinions as facts? Is their site or social media account trying to sell something? A commercial site generally is not as trustworthy as a public health institution or nonprofit organization dedicated to improving health.

When

Find out when the health information or its sources were written, reviewed, or updated. Medical and scientific knowledge changes with each new discovery, so make sure you're reading the most up-to-date information.



Image: iStock.com/Aleksei Naumov

Ask your healthcare team

Doctors, therapists, and other providers keep up with the latest research and case studies as part of their job. They can share up-to-date information with you and help you evaluate what you've read.

"It is important to discuss what you read with your primary care provider, neurologist, and other trusted specialists," Dr. Lek says. "They are at the forefront of the field and can tell you if what you have read is reliable and if you have correctly interpreted the information."

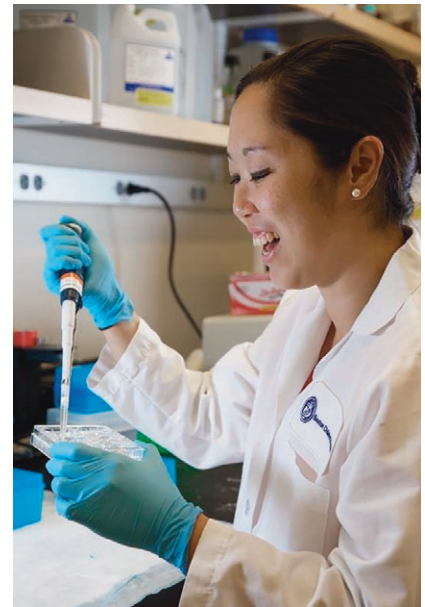
It's especially important to consult with an expert when you come across research studies or other health information written for the scientific community. These use scientific terms and principles without explaining them, which can lead to misinterpretation. Dr. Lek provides an example: "A reputable scientific journal can publish positive results on stem cell research, which may cause patients to misinterpret that the technology is close to being used in the clinic; whereas in reality, the article was targeted at other researchers in the field with the intention of sharing a small piece of the puzzle," she says.

Your healthcare providers should welcome you bringing them questions. This shows them that you want to be a partner in your care. (Read "Advocate for Your Healthcare Needs" on page 18 for tips on communicating with providers in healthcare settings.)

Build a trusted network

By evaluating the information you find and communicating with your healthcare team, you can build a network of trusted organizations and websites that provide the information you seek. Check them regularly to stay up to date on the latest neuromuscular disease research and news.

Get started by tearing out and keeping the chart on the next page, which lists resources for reliable disease information, research news, and education. [Q](#)



Angela Lek, PhD

Amy Bernstein is a writer and editor for Quest.

I WANT TO



LEARN MORE ABOUT NEUROMUSCULAR DISEASE

MDA Disease Fact Sheets and Resources

Overviews of some of the most common neuromuscular diseases and other educational resources
mda.org/education

MDA Disease Webpages

Summaries of many of the diseases MDA covers
mda.org/disease/list

MDA Engage Disease Symposia

Recorded and upcoming educational events that each focus on one disease
mda.org/care/mda-engage/disease-symposia

Quest Diseases A-Z

Quest content, including personal stories and research updates, related to specific diseases
MDAQuest.org/diseases-a-z

NIH Genetic and Rare Disease Information Center

Searchable database of rare and genetic diseases from the National Institutes of Health (NIH)
RareDiseases.info.nih.gov

NORD Rare Disease Database

Overviews of thousands of rare diseases from the National Organization for Rare Disorders (NORD)
RareDiseases.org/rare-disease-information

NORD Rare Disease Video Library

Educational videos about many rare diseases
RareDiseases.org/rare-disease-video-library

+MORE EDUCATION

MDA Engage is a community education program bringing accessible knowledge and resources to the neuromuscular disease community. Watch seminars and webinars covering a variety of topics — from research updates to nutrition and parenting tips — live or on-demand. Visit mda.org/engage.



UNDERSTAND GENETIC INHERITANCE

MDA Education Materials

See Disease Specific Resources and General Neuromuscular Disease Resources for fact sheets about genetics and inheritance patterns
mda.org/education

Quest Feature: Family Inheritance

How genetic neuromuscular disease is passed from one generation to another
MDAQuest.org/family-inheritance



LEARN ABOUT GENETIC TESTING

MDA Education Materials

See Disease Specific Resources and General Neuromuscular Disease Resources for fact sheets about genetics and genetic testing
mda.org/education

Quest Feature: The Importance of Genetic Testing

The advantages of getting a genetic diagnosis and how to get started
MDAQuest.org/importance-genetic-testing



UNDERSTAND GENE THERAPY

Quest Feature: Targeting Genes

How gene-targeted therapies for neuromuscular diseases work
MDAQuest.org/targeting-genes

Quest Feature: Express Delivery

How viral vectors are used in gene-targeted therapies for neuromuscular diseases
MDAQuest.org/express-delivery

ASGCT Gene Therapy 101

Explanations and videos from the American Society of Gene and Cell Therapy
PatientEducation.asgct.org/gene-therapy-101



CUT AND SAVE

I WANT TO

⚙️ READ ABOUT RESEARCH

Quest Blog

Covers breaking news on treatments, and research and clinical trial updates
[MDAQuest.org/blog](https://mdaquest.org/blog)

MDA Grants at a Glance

Research projects funded through MDA's grants program
mda.org/gaag

BioNews

Online health news service with five disease-specific publications covering neuromuscular diseases
[ALSNewsToday.com](https://alsnews.com)
[FriedreichsAtaxiaNews.com](https://friedreichsataxia.com)
[MuscularDystrophyNews.com](https://muscular-dystrophy.com)
[MyastheniaGravisNews.com](https://myasthenia.com)
[SMANewsToday.com](https://smadaily.com)

ANCAN: A Layperson's Guide to Reading Medical Research

Strategies to help you understand research articles from the Answer Cancer Foundation
[AnCan.org/special-presentation-a-laypersons-guide-to-reading-medical-research](https://an-cancer.org/special-presentation-a-laypersons-guide-to-reading-medical-research)

⚙️ FIND CLINICAL TRIALS

MDA Clinical Trials Finder

The Trial Finder tool helps sort through available neuromuscular disease clinical trials
mda.org/clinical-trials

MDA Clinical Trial Updates

List of open clinical trials for neuromuscular diseases
mda.org/clinical-trial-updates

ClinicalTrials.gov

A database of clinical studies around the world from the US National Library of Medicine
[ClinicalTrials.gov](https://clinicaltrials.gov)

Trials Today

A quick way to search the thousands of studies available on ClinicalTrials.gov
[TrialsToday.org](https://trials.today)

⚙️ FIND A DISEASE REGISTRY

MDA MOVR Data Hub

Collects data on multiple neuromuscular diseases to help researchers build clinical trials and understand treatment outcomes
mda.org/MOVR

National ALS Registry

Collects information from individuals with amyotrophic lateral sclerosis (ALS) to help researchers learn more about the disease and its risk factors
cdc.gov/als

NIH List of Registries

List of disease registries available nationally
nih.gov/health-information/nih-clinical-research-trials-you/list-registries



Image: iStock.com/Aleksei Naumov

+ MORE RESOURCES

The MDA Resource Center answers questions and can assist with finding services and programs for people in the neuromuscular disease community. Resource Center staff are available Monday through Friday, 9 am to 5 pm CT. Call **833-ASK-MDA1 (1-833-275-6321)** or email ResourceCenter@mdaUSA.org.

Fishing for MD Foundation (FFMD)



Over the last 6 years, the Fishing for MD Foundation (FFMD) has raised over \$3.1M through raffles, galas, and golf tournaments to support MDA’s mission.

Over \$1.3M raised this summer!



Community members and generous supporters from across the country teamed up for 8 golf events and 2 galas raising over \$1.3M for MDA. Meridian Clinical Research Golf Outing (pictured) raised over \$256,000.

2022 Top 5 Boot Drives (As of 9/15/22)



1. Fairfax County, L2068 (\$270k)
 2. Phoenix, L0493 (\$166k)
 3. Philadelphia, L0022 (\$133k)
 4. Corpus Christi, L0936 (\$127k)
 5. Chicago, L0002 (\$116k)
- Stay tuned! More to come!

Muscle Walk Top Team: Team Matthew



Team Matthew, the top fundraising team in the nation, raised more than \$40k at the 2022 Muscle Walk of Missouri – bringing their total to over \$150k just in the last 4 years!



Mike Bush: 35 Years of Community Strength

Leading the way as the #1 Voluntary Health Organization for over 70 years, volunteers are our legacy. At MDA, families are at the heart of our mission, and we couldn’t fulfill our mission without our volunteers who work tirelessly to inspire and move communities. Thank you to all volunteers, new and longstanding, because you are making a difference!

Mike Bush celebrated 35 years of volunteering and was presented with the MDA Tribute Award, which honors the legacy and life’s work of those who have been tireless in their efforts to support the patients and families served by MDA. What started as a local weatherman hosting the MDA Labor Day Telethon on KSDK-TV has grown into an awe-inspiring legacy of volunteerism. Thanks to the team at KSDK-TV, the Show of Strength remains one of the most valued MDA Labor Day legacies. The 2022 MDA Show of Strength raised over \$458,000!

We hope you will join us in communities around the country this year. You can learn more at mda.org/volunteer.

MAKING A DIFFERENCE IN DUCHENNE MUSCULAR DYSTROPHY

**FEATURING:
JOHN BRANDSEMA, MD**

John Brandsema, MD is the neuromuscular section head in the division of neurology at Children's Hospital of Philadelphia (CHOP). He has a special interest in treating children with neuromuscular disorders and is enthusiastic about the promise that medical and scientific advances hold for people with these conditions.

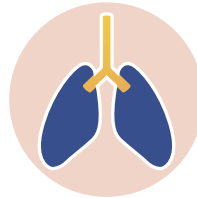
Corticosteroid treatment now and over time

Clinical studies have shown that starting boys with Duchenne muscular dystrophy (DMD) on corticosteroids soon after diagnosis can help delay, or slow, disease progression. Corticosteroids have helped boys who are still ambulatory extend ambulation and preserve muscle function.

Corticosteroid treatment can also be helpful to boys who have lost ambulation. According to care consideration guidelines, boys who are non-ambulatory should continue with corticosteroids.

Guidelines also suggest balancing the benefit of corticosteroids with proactive management of possible side effects, such as facial puffiness, high blood pressure, cataracts, abnormal behavior changes, and effects on growth and bone health.

**MULTIPLE STUDIES HAVE SHOWN
CORTICOSTEROIDS MAY HELP
PRESERVE LUNG FUNCTION AND
DELAY SCOLIOSIS**



Corticosteroid research in boys who are non-ambulatory is ongoing.

To learn more about the benefits and risks of corticosteroids, speak to your healthcare professional.

Please see the Brief Summary of Information for EMFLAZA® (deflazacort) on the following page.

In my decades of caring for boys and men with DMD, I've found that corticosteroid treatments, like deflazacort or prednisone, are the standard of care for the majority of patients.

INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

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.

Emflaza® is a registered trademark of PTC Therapeutics, Inc. © 2022 PTC Therapeutics, Inc. All Rights Reserved. US-EMF-0362 04/22



MDA On the Road



Paul Melmeyer, fifth from left, at the Chan Zuckerberg Initiative

Our talented staff at MDA is on the road 24/7, working and advocating for the neuromuscular disease community. From the White House and Capitol Hill to high-impact conferences around the world, MDA is here for you today and every step of the way. We invite you to follow us on this impactful journey.

We are proud to launch our MDA On the Road series with recent participation by MDA staff members in the Chan Zuckerberg Initiative’s Rare as One project at Facebook and the Smithsonian Science Education Center’s Zero Barriers in STEM Summit.

Follow us on our social channels to check out where MDA On the Road is next.

Highlighting LGMD at Facebook

Paul Melmeyer, MDA’s vice president of public policy and advocacy, joined the Chan Zuckerberg Initiative’s Rare As One project at Facebook this summer to present MDA’s efforts to improve diagnostics for different types of limb-girdle muscular dystrophy (LGMD) by obtaining special classification codes. Founded by Facebook’s Mark Zuckerberg and his wife, Priscilla Chan, the Chan Zuckerberg Initiative’s purpose is to “help solve some of society’s toughest challenges.”

Healthcare providers in the United States use a coding system to record all diagnoses, symptoms, and procedures. Obtaining disease-specific classification codes is critical for financial coverage and increases healthcare providers’ awareness of the types of LGMD.



Scan this code to donate to MDA!

“The LGMDs are under-researched diseases with limited efforts to capture their true prevalence and incidence,” Paul says. “Adopting codes for the most prevalent LGMDs should substantially accelerate the understanding of LGMD, thus advancing efforts to better treat these diseases.”

Discussing STEM accessibility at the Smithsonian

Marissa Lozano, MEd, MDA’s national director of community education, participated in a panel discussion on accessibility and inclusion in STEM education at the Smithsonian Science Education Center’s Zero Barriers in STEM Summit. The audience consisted of educators



Marissa Lozano, far left, at the Smithsonian

from across the country committed to making STEM learning more accessible for students with disabilities. Lozano shared learnings from MDA's STEM Connections program, launched in partnership with General Motors.

This program engages MDA Summer Campers in STEM-based activities, such as building working catapults and designing and constructing bridges. In addition, young adults (ages 16-21) with neuromuscular diseases interested in STEM can receive mentoring from professionals representing fields such as mechanical engineering, medicine, and computer science. Participants work with their mentors to complete a STEM project and learn about career pathways.

"It was great to share how the STEM programs are impacting the lives of young people living with neuromuscular diseases," Marissa says. "Individuals with disabilities are underrepresented in STEM fields, and we want to change that."

For information about the 2022 MDA STEM Connections mentoring program, visit mda.org/young-adults/stem-connections.



Image: iStock.com/slowgogo

MDA Advocates Are Back (Virtually) on Capitol Hill

After a three-year hiatus, MDA advocates returned to Capitol Hill — virtually this time. On Sept. 28 and 29, families across the country urged lawmakers to act on issues impacting the neuromuscular disease community. Advocates' policy priorities included improving air travel, increasing access to genetic counselors, and reforming clinical trials and drug approvals at the US Food & Drug Administration.

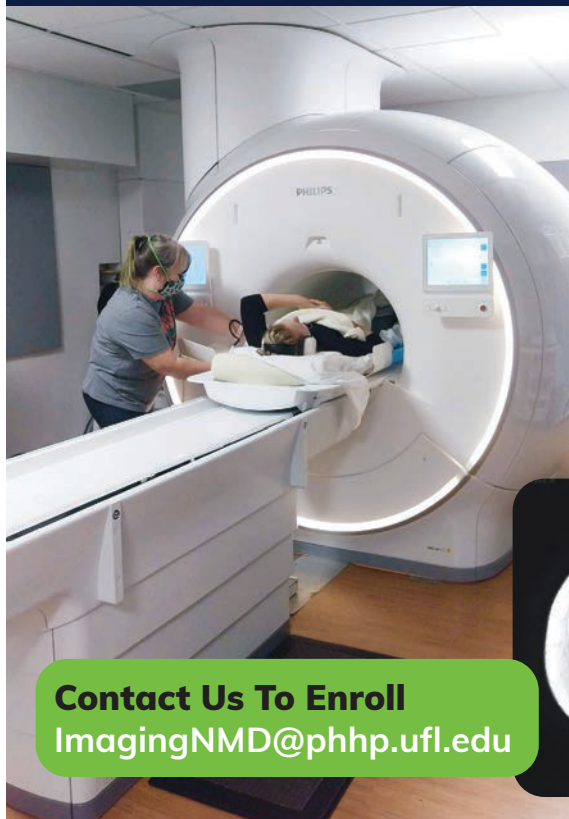
MDA advocates made a big difference, with:

- > 100 total Congressional meetings
- > 78 participating advocates and staff
- > 28 states represented

In 2023, MDA and its grassroots network of advocates will continue to meet with lawmakers to ensure policies are passed to empower the neuromuscular disease community. Join us at mda.org/advocacy.

ADVERTISEMENT

RESEARCH FOR BECKER MUSCULAR DYSTROPHY

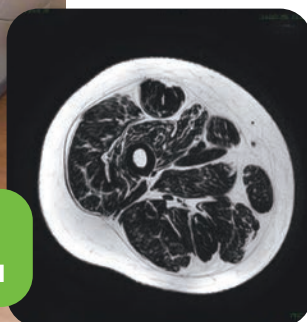


WHY PARTICIPATE?

Natural History research helps everyone in the community understand more about rare diseases like Becker Muscular Dystrophy. Armed with this invaluable information, companies and research organizations can develop and test the efficacy of new drugs and treatments more efficiently.

WHAT WOULD I HAVE TO DO?

The ImagingNMD natural history study involves traveling to one of our 2 research sites in either Florida or Oregon for a total of 3 yearly visits. Travel is paid for and you will be paid up to \$500 for participating in all 3 visits. Each visit is 1-2 days long and involves MRI scans of different parts of your body as well as functional tests and biosample collection.



Contact Us To Enroll
ImagingNMD@phhp.ufl.edu

WHO CAN JOIN?

- Participants must be
- Diagnosed with Becker muscular dystrophy
 - Between the ages of 18-62
 - Able to enter an MRI machine

HOW DO I SIGN UP?

Just contact one of our study coordinators to get started with an eligibility screening!

**IMAGING
NMD**

Quest Marketplace

NOTICE TO OUR READERS: MDA does not endorse products, services, or manufacturers. Such names appear here solely because they may provide valuable information. MDA assumes no liability for the use or contents of any product or service mentioned.

LiftSeat®
Powered toilet lifts™

Powered toilet lift & electric washing bidet



Get \$100 off your LiftSeat with code MDASAVINGS

CALL 1-877-665-4381
or go to www.liftseat.com to order

VOICE ACTIVATED DOOR

by
OPEN SESAME

- // Releases a locked door
- // Voice or remote control activated
- // Simple to install
- // Battery backup



OPEN SESAME

Call for more information
888-973-7263
OpenSesameDoor.com

LIFT YOUR LIFE TOWARD INDEPENDENCE



BATTERY-POWERED TOILET SEAT LIFT

- > Perfect for those with muscle weakness
- > Standard or mobile models available
- > Lifts straight up; no forward push

800-831-8580

for information and a FREE demo video

www.stand-aid.com | sales@stand-aid.com

STAND AID of America



TR EQUIPMENT
*Hygiene Equipment
Designed for People*



Bathing products include

- Bathtubs
- Shower Chairs
- Reclining
- Powered



Designed by TR Equipment AB and Rebotec GmbH.



Tel. (800) 752-6900
www.TREquipment.com

Learning to Adapt

Creativity helps me manage accessibility challenges, even as I advocate against them

BY TOMECA GOODWIN

I was once on the go all the time, but in recent years, I have found myself slowing down and trying to pace myself. I do not push the limits on my energy as I once did, such as meeting friends after work, attending events, visiting family, and traveling alone. I am not alone much, and although I love the company, it comes with the reality of my diminishing independence.

I was genetically diagnosed with limb-girdle muscular dystrophy (LGMD) type 2E in 2021, but I have lived with it for the last 27 years. The onset of my symptoms was a scary time. I did not see this coming, and I did not understand what was to come. After several years feeling like I was merely existing, I made up in my mind that I was going to live, keep moving as much as I could, and pray daily to be an exception to the diagnosis that I have been given.

I am constantly learning to adjust and find creative ways to do things while trying to remain ambulatory and as independent as my body will allow. Giving up is never an option.

Getting creative

I have worked for a large healthcare network for 30 years. I am still able to drive and walk short distances. At work, I have a drafting chair and sit/stand desk in my office and a Super Pole in the restroom that serves as a great grab bar.

In daily life, I clearly see and experience how accessibility is limited by the way normal, everyday things are designed and arranged. For example, accessible building entrances are sometimes down the street and around the corner, or there is not one at all. Public restrooms are my arch nemesis and the reason I either restrict my fluid intake or choose to stay home. There is normally one accessible stall that has a low toilet and one grab bar, and most times it is occupied by someone who just wants extra room. I do not know how many times I have waited for that one stall. I used to tote around a toilet riser, but now I have a foldable commode that goes over the seat that provides height and arm rests, and it folds nicely in my carry-on bag for travel.

Dining out is an experience that most of us enjoy, but many restaurants have heavy doors that are hard for someone like me



Tomeca Goodwin appreciates spaces designed for accessibility.

to open. Once inside, navigating to a seat between tightly packed tables can be even more challenging. If I cannot sit on a bar stool, I use Kaboost, a portable booster, or the Upeasy seat assist, so I can get in and out of my chair. I also have a folding counter height chair that stays in my car as another option.

So many things have become more noticeable now that I use a foldable powered wheelchair for long outings. I did not realize the number of places that have stairs without ramps, or the uneven sidewalks and high curbs. And why trashcans are often placed directly under the elevator buttons is a mystery to me.

“

I am reminded daily that people cannot really understand what they have not experienced, and I advocate for accessibility even more now that there are more things I struggle to do.

— Tomeca Goodwin

It brings a huge sigh of relief when I am in a place designed with every person in mind: for example, adequate parking spaces with ramps, barrier-free entrances, motion sensor doors, touchless faucets, touchless toilets with grab bars, taller seating with arms, and things placed at heights for those standing and sitting.

Keep fighting

My journey has had difficulties, and often I have felt as though I was on the losing end of life, but I continue to pray and push through because I know that I am here for a reason. I have a purpose to fulfill, even though I need assistance with

getting dressed, getting in and out of a chair or car, walking, combing my hair, and tying my shoes. It takes more than most people will ever know for me to just show up, but I continue to give it my best. I am reminded daily that people cannot really understand what they have not experienced, and I advocate for accessibility even more now that there are more things I struggle to do.

I have been blessed with a support system of family and friends who fight alongside me. Their support has been everything, and that helps keep me going. They pray, advocate for me, and encourage me in ways you could not imagine.

I encourage anyone reading this to advocate for yourself and others who need someone to speak up for them. Some people may count us out, but from where I sit, it takes strength beyond muscles to walk a day in our shoes. Keep pushing, keep going, keep praying, and know that you are not alone in this fight. [Q](#)

Tomeca Goodwin, 48, lives in Cincinnati, Ohio. She founded the Invisibly-Visible Foundation to support people with muscular dystrophy and multiple sclerosis with equipment and home modification grants, and she blogs at invisibly-visible.org. To connect with her, email her at InvisiblyVisible513@gmail.com.



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.

Best. Selfie. Ever.


Photo contest winner Sean Wallace goes above and beyond for a great shot

Congratulations to our 2022 Lasting Impression Photo Contest winner Sean Wallace, 31, of La Grange, Texas. The winning photo shows Sean, who lives with Becker muscular dystrophy (BMD), on a bridge that extends from the pier at Matagorda, Texas's Jetty Park. A professional photographer, Sean captured this photo using a tripod and the camera's timer. He set up the tripod on a pier facing the bridge and programmed the timer to snap a photo every five seconds until he manually turned it off. After two tries, he found his perfect placement on the bridge and was excited by the result.

"I was likely the first person in a power chair to be on that bridge," says Sean, who adds that he tested the

structure's sturdiness beforehand. "In this moment, I felt appreciative of the cool things I get to do while being in a wheelchair."

Sean loves the independence he feels during adventures like this one.

"This photo is special to me because I love the sound of the ocean waves and the wind blowing," Sean says. "Being outdoors in all of God's creation gives me peace and joy. Experiences like this make cool memories and stories." 

+MORE PHOTOS

Congratulations to the runners-up in our reader photo contest: Heidi MacCurtain of Plymouth, Massachusetts; Sarah Thatcher of St. Louis, Missouri; and Susan Manning of Worcester, Massachusetts. See their photos at [MDAQuest.org](https://www.mdaquest.org).



Living with Myasthenia Gravis?

A new research
study is enrolling.



FLEX

Learn about the FLEX research study of an injectable investigational drug that can be administered at home for adults with myasthenia gravis.

For more information, visit www.flexMGstudy.com



Duchenne.com

A place to learn, share knowledge, discover resources, and find hope.

Duchenne.com includes new resources and information that can assist all who are living with this rare disease

VISIT **DUCHENNE.COM** TO SEE WHAT'S NEW!

DILLON, living with Duchenne.

Highlights include:



Research: Read up-to-date management options and information about the latest science in Duchenne.



Community Voices: Watch videos showing real-life experiences and advice from patients and caregivers.



Knowledge: Access resources to help you better understand complicated subjects, including the importance of dystrophin and genetic testing, and also find questions to ask your doctor about Duchenne.



Stay in the Know about Duchenne. Sign up today.