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# Quest

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Getting kids involved in extracurricular activities

## School Days



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How research leads to treatments



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for more people  
with SMA**

See **What is Evrysdi** below  
and visit **Evrysdi.com** for  
more information



### 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.**

**Please see accompanying brief summary for additional Important Safety Information.**

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

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

**The most common side effects of Evrysdi include:**

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

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

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

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

**Genentech**  
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**Evrysdi**<sup>®</sup>  
risdiplam 60 mg  
powder for oral solution

**LIFE IN ACTION**



**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)

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94080-4990

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For more information, go to [www.EVRYSDI.com](http://www.EVRYSDI.com) or call 1-833-387-9734.

## On the Road Again

Local volunteers and communities have always been at the heart of MDA's mission. As the world has begun to reopen and our talented staff has been out on the road, I embarked on my own "CEO roadshow."

To begin the year, I attended and spoke at the International Association of Fire Fighters (IAFF) Affiliate Leadership Training Summit Human Relations Conference. The IAFF's 68-year partnership with MDA has helped bring treatments to hundreds of thousands of people with neuromuscular diseases, and it was an honor to be there.

Earlier this summer was a Tribute Tour stop in Houston. On this tour, the MDA team is traveling the country to visit with local partners, volunteers, MDA Care Centers, and families. In Houston, we honored world-renowned researcher Stanley H. Appel, MD, and had a listen-and-learn session with the care team and patients at the MDA ALS Clinic at Houston Methodist. We also had fun visiting with campers, staff, and volunteers at the local MDA Summer Camp.

In June, I supported our partners at CITGO and Mansfield Energy at the 37th Annual Golf Classic hosted by CITGO and the 36th annual Mansfield Cares Golf Classic.

Dutch Bros then welcomed us to tour the company's headquarters in Grants Pass, Oregon, and meet co-founder Travis Boersma and the fantastic Dutch Bros team. Since 2007, Dutch Bros has raised millions for MDA and ALS research and care.

More recently, I visited several of MDA's biopharma industry partners in the Boston area, including Amylyx Pharmaceuticals, argenx, Entrada Therapeutics, Fulcrum Therapeutics, and Wave Life Sciences. Be assured, they are working tirelessly to quickly get new treatments to patients with neuromuscular diseases.

Up next is our Labor Day event in St. Louis, where we will host a community celebration for longstanding local news anchor Mike Bush, who celebrated 35 years of volunteering for MDA in 2021. We'll also visit our partners at Burn Boot Camp and celebrate their incredible "Be Their Muscle" campaign.

My recent travels have proven that our community and our partnerships are stronger than ever. We are here for you, and we look forward to seeing you soon!



Donald S. Wood, PhD



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

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.

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### Harley-Davidson Ride for Life



Over 500 bikers from 21 dealerships gathered in Lebanon, PA for Harley-Davidson's 35th Annual MDA Ride for Life, raising over \$900k.

### Furniture Mart



To support MDA Summer Camp, Furniture Mart and Unclaimed Freight in the Dakotas, Iowa, and Minnesota, raised over \$27k this March.

### NALC Branch Challenges



72 NALC Branches and State Associations raised over \$200k in Branch Challenges in 2021. Branch Challenges are underway, with over \$55k raised in 2022.

### Go Yard with Rhys and Jayme Hoskins



First baseman for the Philadelphia Phillies and former MDA Summer Camp counselor, Rhys Hoskins, raised over \$115k hosting Go Yard for MDA.



### Magical Mission Moments

MDA Tribute Awards kicked-off the Tribute Tour in Houston, TX. Maanav Gupta, Marcus Mann, & Dr. Stanley Appel were honored for their tireless support of MDA.



In honor of Lou Gehrig Day, MDA has been hosting MDA Tribute Tailgates around the country. MDA families joined the Arizona Diamondbacks in the fight to End ALS.



MDA partnered with Magic Wheelchair to surprise 9-year-old, Carter, with a custom Ghostbusters-themed wheelchair!



## Style Momentum

Gaining independence through accessible design and self-expression

The Year of Independence shines a light on ways the MDA community can effect change in 2022 and beyond — from careers to personal relationships to the clothes we wear.

“Adaptive clothing is important because it accomplishes the goal of giving more independence in how we dress and express ourselves,” says Grace Jun, the founder and CEO of Open Style Lab, a nonprofit organization dedicated to using design and technology for educational programs and research on accessible style.

Over the last few years, adaptive fashion has gained more traction, with major retailers offering collections and models with disabilities appearing in clothing ads and on fashion show runways. Momentum is building for adaptive style.

### A seat at the table

Advocacy is leading to collaboration, and more companies recognize that people with disabilities must contribute to the design of adaptive fashion. Created in 2014 as a public service project at the Massachusetts Institute of Technology

(MIT), Open Style Lab brings together a multidisciplinary team that includes people with disabilities.

“Our work also requires understanding the perspectives of occupational therapists people might work with, or the people they live with, whether it’s their parents or their spouses who may

help with dressing,” Grace says. “The team we collaborate with keeps getting bigger.”

In 2020, Open Style Lab collaborated with MDA for its Summer Fellowship Program. “Together, we created design hacks on how to make dressing easier, functional, and stylish — anything from designing the pulley for your zipper to looking at different pocket designs,” Grace says.



A Summer Fellowship Program participant shows off a creation.



Collaboration is an important component of Open Style Lab's Summer Fellowship Programs.

### Local and affordable

Major retailers like JCPenney and Kohl's now offer adaptive fashion choices for all ages. Last year, JCPenney recognized a need for adaptive children's clothing and launched the Thereabouts brand, a line of kids' apparel that includes functional, adaptive features. “Today, we're pleased to offer adaptive options for the whole family across our private brands,” says Michelle Wlazlo, JCPenney's chief merchandising officer.

The Kohl's team got to work on an adaptive assortment of apparel in 2019. “By listening to personal experiences from both associates and customers, Kohl's was able to understand the challenges that families face and the opportunity we had to support their needs through accessible apparel,” says Katherine Finder, Kohl's senior vice president of design.



Major retailers like Kohl's and JCPenney now offer adaptive clothing.

### Looking to the future

Cosmetics designers are also recognizing the need for easier-to-use products, with more products coming on the market to meet the demand, according to Stephan Kanlian, professor and chairperson of the department of cosmetics and fragrance marketing and management at the Fashion Institute of Technology in New York City.

“There are several niche players in the market in the past five years who have created tools that offer a more fastened grip, twist 180 degrees, bend backward and forward, and stand up,” Stephan says.

Stephan and Grace both feel adaptive design will only get more exciting, and Grace encourages people living with disabilities and designers to continue working together.

“Disability isn't a monolith,” Grace says. “It's really about everyone's personal experiences and coming together to find the commonality in the ways we experience life.” [Q](#)

### +MORE ONLINE

Read an expanded version of this article at [mdaquest.org/YearofIndependence3](https://mdaquest.org/YearofIndependence3).



## New School Year, New Opportunities



Those of us who live with neuromuscular illness have enough reasons to worry. In the pages of *Quest*, we want to give you ideas that will help you make positive impacts on your life.

For example, as kids and adults gear up for back-to-school season, look for opportunities to boost self-esteem with products that make style more accessible (page 20) and to improve social wellness by helping kids branch out into new

and exciting extracurricular activities (page 25).

While exercise is healthy, it can be tricky for people with neuromuscular diseases. We've gathered advice from experts and talked with people living with neuromuscular conditions about how they incorporate physical activity in their lives (page 30).

There is so much more to our overall health and wellness than our neuromuscular disease. Some aspects of wellness are very much within our control, and in this issue of *Quest*, you'll find ideas, tools, and resources to add to your arsenal to help you stay healthy.

To all of you who are gearing up to go back to school, whether you're starting kindergarten, seventh grade, high school, trade school, college — or beyond — I want to take this opportunity to wish you success in the year ahead. Going the distance and getting an education is not always an easy path, but it is always a worthwhile one. Cherish the opportunity to learn, and make the most of your year!

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

**ALEXION**  
 AstraZeneca Rare Disease

### ADVERTISEMENT

ALXN1210-DM-310\_Quarterly Magazine Feature\_V2\_08Mar2022



## A Clinical Research Study on Dermatomyositis (DM)

### About the ALXN1210-DM-310 Study

Alexion's mission is to deepen our understanding and transform the lives of people affected by rare diseases and who are living with devastating conditions. This knowledge allows us to innovate and evolve into new areas, where there is great unmet need and opportunity to help patients and families fully live their best lives. As part of our focus on new areas, Alexion is now researching dermatomyositis (DM) in the ALXN1210-DM-310 Study, which is enrolling approximately 180 adult participants across North America, Europe, and Asia Pacific.

The ALXN1210-DM-310 Study is a double-blind, randomized, placebo-controlled, parallel-group, multicenter study to evaluate the effectiveness and safety of ravulizumab in adult participants with DM. The purpose of this study is to evaluate the investigational medication compared to standard of care on reducing symptoms in patients with DM. This is a phase 2 study that leads to a phase 3 study.

Before enrolling in the study, interested individuals will enter the screening period to determine if they are eligible to join. Eligible participants must be at least 18 years of age with a diagnosis of DM, and they must have had an inadequate response or be intolerant to two or more DM treatments. Participants who complete the last visit in the study treatment period may have the opportunity to receive the investigational medication in the open-label extension period.

To learn more, please visit [DM310.com](https://DM310.com).





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Cover image: iStock.com/ArdeaA

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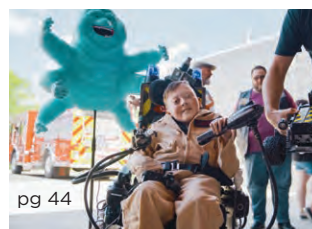
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## MORE ONLINE

**PHOTO CONTEST**  
 Share a photo of a meaningful moment for you or a loved one with a neuromuscular disease, and it could be selected to appear in a future issue of Quest. Submit your photo at [SurveyMonkey.com/r/QuestPhoto](https://SurveyMonkey.com/r/QuestPhoto) or scan this QR code.



**BACK TO SCHOOL GOT YOU STRESSED?**  
 Read about strategies to help you manage stress and thrive in a Quest online exclusive article at [mdaquest.org/ManagingStress](https://mdaquest.org/ManagingStress).



# Ask the Resource Center

**My son has an individualized education program (IEP) and is in a school that does not meet his needs. What equipment and services should the school provide?**

—Enis, Connecticut

The Individuals with Disabilities Education Act (IDEA) requires public schools to provide special education and related services to any child with a disability. Examples of related services include transportation, speech-language pathology, physical therapy, and therapeutic recreation.

Start by talking with your son's school to make sure his IEP includes all the aids and services he needs. A school must provide the services specified in the IEP. If you feel your child's needs still are not met, contact the Special Education office at your state's Department of Education and your state's protection and advocacy system, which provides legal support to ensure people with disabilities can exercise their rights.

**Helpful resource:** Find state protection and advocacy systems at [acl.gov/programs/aging-and-disability-networks/state-protection-advocacy-systems](http://acl.gov/programs/aging-and-disability-networks/state-protection-advocacy-systems).

**I am a Duchenne muscular dystrophy (DMD) gene carrier and have pain and weakness in my legs. What are the health risks for female DMD carriers, and what kind of doctor should I see?** —Terri, North Carolina

Females who have a normal dystrophin gene on one X chromosome and an abnormal dystrophin gene on the other X chromosome are DMD carriers. Most carriers are not affected, but a small number experience symptoms of DMD, which can include muscle weakness and cramps, fatigue, and heart problems. Symptoms range from mild to severe and may start in childhood or adulthood.

MDA Care Center neurologists and cardiologists have expertise in treating individuals with neuromuscular diseases, including DMD carriers.

**Helpful resource:** Free genetic testing for DMD carriers is available at [parentprojectmd.org/about-duchenne/decode-duchenne/carrier-testing](http://parentprojectmd.org/about-duchenne/decode-duchenne/carrier-testing).

## +GOT QUESTIONS?

Contact the Resource Center at **833-ASK-MDA1** or [ResourceCenter@mdaUSA.org](mailto:ResourceCenter@mdaUSA.org).



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APR279402 rev 1 17-SEP-2021 ENG – US

# 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](mailto:usmedinfo@ptcbio.com). You may also report side effects to FDA at 1-800-FDA-1088 or at [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

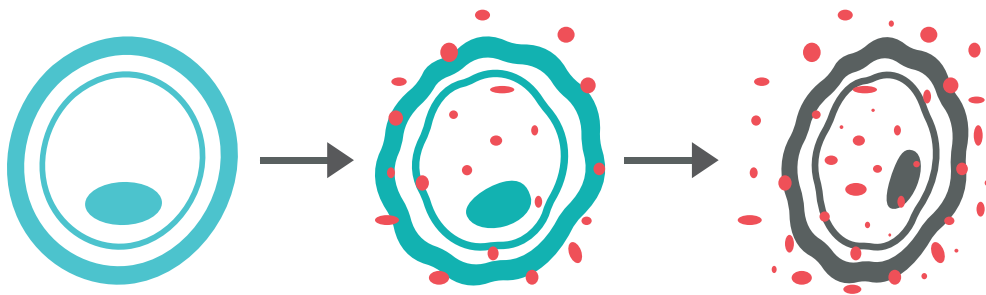
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# PROGRESS NOW

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

## New approvals

### FDA Approves Oral Radicava for ALS



Oxidative stress has been associated with several diseases that affect neurons, including ALS.

The US Food and Drug Administration (FDA) has granted approval to an oral suspension of the antioxidant drug edaravone (Radicava ORS) for the treatment of amyotrophic lateral sclerosis (ALS).

The intravenous (IV) version of Radicava was approved to treat ALS in 2017, while the newly approved Radicava ORS is a different formulation designed to be taken orally or via feeding tube.

Radicava ORS is thought to protect muscle-controlling nerve cells called motor neurons from damage caused by oxidative stress (toxicity caused by harmful free radicals) in people with ALS.

A phase 3 clinical trial in 137 people with ALS showed Radicava treatment slowed loss of physical function by 33% compared to placebo, as measured by the ALS Functional Rating Scale-Revised (ALSFRRS-R). A global phase 3 clinical trial in 185 people with ALS also demonstrated the safety and tolerability of Radicava ORS after 24 weeks of treatment.

Radicava and Radicava ORS were generally well-tolerated in trial participants. The most common side effects were bruising, problems walking, and headache. Fatigue was observed in some people taking Radicava ORS.

For more information, visit [radicava.com/update](https://www.radicava.com/update).



#### CLINICAL TRIAL TERMS TO KNOW

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

**Multicenter:** The trial will be completed at more than one site.

**Randomized:** Participants are randomly placed in groups taking the drug or placebo.



# Ultomiris Approved to Treat gMG



To date, Ultomiris is the third disease-modifying drug approved to treat gMG.

The FDA has approved ravulizumab (Ultomiris) for the treatment of generalized myasthenia gravis (gMG) in adults who test positive for the anti-acetylcholine receptor (AChR) antibody.

Ultomiris is designed to inhibit the C5 complement protein, a component of the body's immune system. In gMG, uncontrolled activation of C5 and related complement proteins may cause the immune system to attack healthy cells in the body. Following a high first dose, called a loading dose, a lower dose of Ultomiris is administered intravenously (in the vein) every eight weeks. Ultomiris will not cure gMG, but it could lead to better function in daily life.

The FDA based its decision to approve Ultomiris on the positive results of the CHAMPION-MG phase 3 trial, which evaluated its safety and efficacy in 175 adults with gMG. This trial showed that Ultomiris treatment in people with AChR-positive gMG resulted in decreased severity of symptoms, as measured by the Myasthenia Gravis Activities of Daily Living (MG-ADL) score.

Ultomiris was generally well-tolerated. The most common adverse reactions were upper respiratory tract infection and diarrhea.

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To learn more, visit [ultomiris.com](http://ultomiris.com).

## Amyotrophic lateral sclerosis (ALS)

# Phase 3 Drug Trial Enrolling

Researchers at Cytokinetics, Inc. are seeking adults living with ALS to participate in the phase 3 COURAGE-ALS clinical trial. The trial aims to evaluate the safety of investigational drug reldesemtiv and its effect on functional outcomes in ALS.

Reldesemtiv is designed to slow the rate of calcium release from skeletal muscles, leading to an increase in skeletal muscle force in response to signals from motor neurons.

COURAGE-ALS is a phase 3, double-blind study. Participants will be randomly assigned to receive reldesemtiv or an inactive placebo administered as an oral tablet for the first 24 weeks of the study. All participants will receive the drug in the second 24 weeks. The trial could last up to 55 weeks and will require 17 visits (nine remote).

Key tests and outcome measures will include: ALS Functional Rating Scale-Revised (ALSFRS-R), Forced Vital Capacity (FVC), ALS Assessment Questionnaire-40 (ALSAQ-40), grip strength, and EuroQol-5D-5L (EQ-5D-5L)/EQ-Visual Analog Scale (EQ-VAS) questionnaires.

To be eligible, individuals must be between ages 18 and 80, have a diagnosis of ALS, have experienced the first symptoms of ALS less than 24 months prior to screening, have an ALSFRS-R score of 44 or less at screening, be able to swallow whole tablets, and meet additional criteria.

Travel support may be available.

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To learn more about the study or site locations, visit [ClinicalTrials.gov](http://ClinicalTrials.gov) and enter **NCT04944784** into the "Other terms" search box.



Participants will be evaluated using a combination of health- and function-related questionnaires, physical assessments, and laboratory testing.

## Amyotrophic lateral sclerosis (ALS)

# Study on Tracking ALS at Home

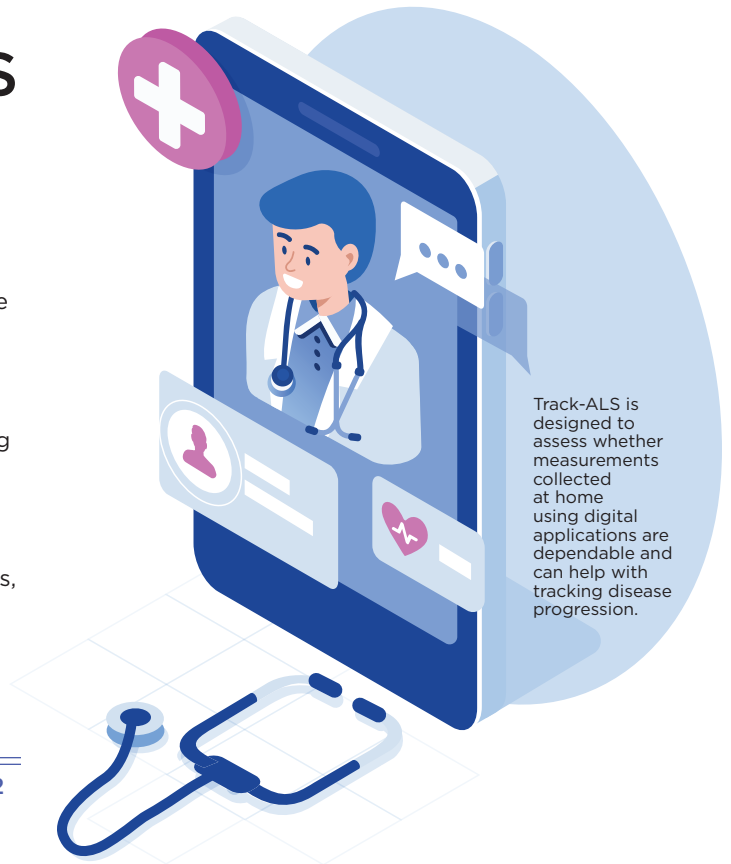
Researchers at the Barrow Neurological Institute (BNI) and Emory University are seeking adults living with ALS to participate in the Track-ALS research study to assess whether measurements collected at home using digital applications are dependable and can help with tracking disease progression.

Study participants will be asked to perform various at-home tests, including a breathing test, speech recordings, movement tracking, and questionnaires, over 12 months using apps installed on a smartphone. Study coordinators will provide the equipment needed to complete these assessments.

To be eligible, individuals must be between the ages of 18 and 90, have received a diagnosis of ALS in the last five years, own a smartphone with Bluetooth capabilities, have continuous internet access at home, and meet additional criteria.

To be considered, complete the pre-screening survey at [barrowneuro.org/TrackALS](http://barrowneuro.org/TrackALS).

For questions, contact the BNI study team at 602-406-6262 or [TrackALSStudy@DignityHealth.org](mailto:TrackALSStudy@DignityHealth.org), or the Emory study team at 404-727-5193 or [Katherine.Cummings@Emory.edu](mailto:Katherine.Cummings@Emory.edu).



## Becker muscular dystrophy (BMD)

# Natural History Study Enrolling

Edgewise Therapeutics is conducting an observational study in individuals with BMD to understand the progression of the disease, as assessed by imaging and functional measures.



The goal of this natural history study is to provide a more comprehensive understanding of BMD to advance potential future therapies.

This study is being led by the General Resolution and Assessments Solving Phenotypes (GRASP) Consortium and Virginia Commonwealth University (VCU), in collaboration with ImagingDMD University of Florida (UF). They plan to enroll approximately 150 individuals with BMD, ages 8 and older, at multiple locations across the United States and Europe. As a natural history study, it will not test any investigational drugs, but rather will monitor participants over a two-year period to examine their disease course.

There are currently no therapies approved in the United States to treat people with BMD, and wide variability in disease course among individuals makes devising investigational therapy trials a challenge. Studies like this can provide information about the typical course of the disease and assist in the design of future therapeutic trials.

For more information, visit [ClinicalTrials.gov](http://ClinicalTrials.gov) and enter NCT05257473 into the “Other terms” search box.



## Dermatomyositis

# Phase 2 Study Seeks Participants



Treatment with Ultomiris could improve the muscle weakness and skin manifestations associated with dermatomyositis.

In this phase 2/3 double-blind, randomized, multicenter study, participants will be randomly assigned to receive Ultomiris or an inactive placebo, administered via intravenous (IV) injection. The phase 2 portion of the trial (part A) is actively enrolling. The trial will include a four-week screening period followed by a 26-week treatment period, and participants will be required to attend six clinic visits.

Participants will be evaluated using a combination of health- and function-related questionnaires, physical assessments, and laboratory testing.

To be eligible to participate, individuals must be at least 18 years old, have a diagnosis of dermatomyositis, have had an inadequate response and/or been intolerant to two or more dermatomyositis treatments, and meet additional study criteria.

Travel support may be available for participants and families.

To learn more, visit [DMFlexStudy.AlexionClinicalTrials.com](https://DMFlexStudy.AlexionClinicalTrials.com).

Researchers at Alexion Pharmaceuticals are seeking adults living with dermatomyositis to participate in a phase 2 clinical trial to evaluate the safety, effectiveness, and pharmacological properties of the investigational drug ravulizumab (Ultomiris).

Ultomiris is designed to target a component of the immune system called complement, which underlies many autoimmune disorders, including dermatomyositis.

In this phase 2/3 double-blind, randomized, multicenter



## 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](https://mda.org/clinical-trial-updates).

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## Duchenne muscular dystrophy (DMD)

# Encouraging Results for Corticosteroid Study



Results from FOR DMD are expected to greatly impact clinical management of people with DMD.

Results from the phase 3 FOR DMD study of corticosteroid dosing regimens for treatment of people with

DMD showed that daily treatment with the corticosteroids prednisone or deflazacort resulted in significantly better outcomes compared with intermittent prednisone treatment.

Corticosteroids have been shown to increase muscle strength in boys with DMD. However, long-term use can lead to undesirable side effects,

including weight gain and low bone density. The optimal corticosteroid dosing for people with DMD has been an open question for decades.

FOR DMD investigators followed 196 boys with DMD between the ages of 4 and 7 over a three-year study period. Participants were randomly assigned to one of three oral corticosteroid regimens to determine which method increased muscle strength the most with the fewest side effects.

Assessments included functional ability, respiratory capacity, and satisfaction with treatment.

The study investigators reported that the findings support the use of a daily corticosteroid regimen as an initial treatment for boys with DMD.

**For more information about the FOR DMD trial, visit [ClinicalTrials.gov](https://ClinicalTrials.gov) and enter NCT01603407 into the “Other terms” search box.**

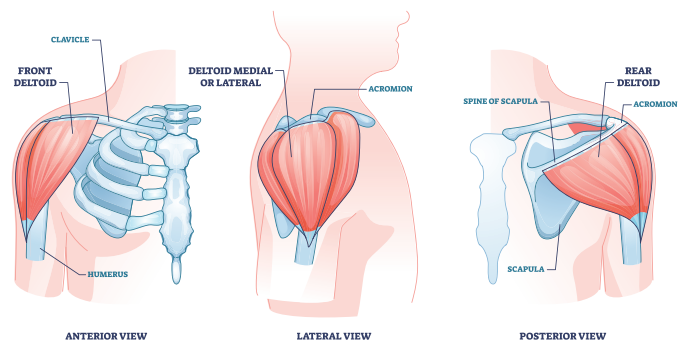
## Boys Needed for Phase 1b/2a Study

Researchers at Wave Life Sciences are seeking boys living with DMD amenable to exon 53 skipping to participate in a phase 1b/2a clinical trial. This trial will evaluate the safety, effectiveness, and correct dosage of the investigational drug WVE-N531.

WVE-N531 is designed to promote skipping over a section of genetic code, exon 53. Treatment with the drug could increase levels of dystrophin protein in the body, potentially decreasing symptoms of the disease.

The study will enroll approximately 15 participants, all of whom will receive the drug intravenously (injection in the vein). An initial group will receive increasing doses of WVE-N531 (up to four doses administered more than four weeks apart) in order to determine the correct dose of the drug, and then up to three additional doses every other week. All participants will receive a maximum of seven doses, followed by a minimum eight-week safety monitoring period.

Participants will be evaluated for side effects, the concentration of WVE-N531 in muscle tissue, and dystrophin



Trial participants must have adequate deltoid muscle at screening to allow for open muscle biopsies.

protein levels in muscle tissue following several doses of treatment.

To be eligible, boys must be 5 to 18 years old with a documented mutation of the DMD gene that is amenable to exon 53 skipping intervention, have stable pulmonary and cardiac function, currently be on a stable corticosteroid therapy regimen, and meet additional study criteria.

**To learn more about the study, contact Wave Life Sciences Clinical Operations at [ClinicalTrials@WaveLifeSci.com](mailto:ClinicalTrials@WaveLifeSci.com) or 855-215-4687.**



## Myasthenia gravis (MG)

# Phase 2 ALXN2050 Study Seeks Participants

Researchers at Alexion Pharmaceuticals are seeking adults living with generalized myasthenia gravis (gMG) for a phase 2 clinical trial to evaluate the safety, effectiveness, and pharmacological properties of the investigational drug ALXN2050.

ALXN2050 is designed to target a component of the immune system known as complement, which underlies many autoimmune disorders, including gMG. Treatment with ALXN2050 has the potential to decrease disease symptoms and increase strength in people with gMG.

Participants in this double-blind, multicenter study will be randomly assigned to receive the drug or an inactive placebo administered orally. Participation will require 14 clinic visits over the course of the study, which will include an initial screening period, an eight-week primary evaluation period, and a 26-week extended treatment period.

Participants will be evaluated using a combination of questionnaires, physical assessments, and laboratory testing.



Alexion's phase 2 study will evaluate the efficacy and safety of ALXN2050 in participants with generalized myasthenia gravis (gMG).

To be eligible, individuals must have a diagnosis of MG at least three months prior to screening, have a Myasthenia Gravis Foundation of America Clinical Classification Class II to IV at screening, and meet additional criteria.

Travel support may be available for participants and families.

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To learn more, contact study coordinator Christine Rowe at [Christine.Rowe@alexion.com](mailto:Christine.Rowe@alexion.com).

# Gait Sensor Study Seeks Participants With or Without Neuromuscular Diseases



Some volunteers may be asked to wear a small sensor on their ankle.

Researchers at the University of California, Davis's Neuromuscular Research Lab are seeking participants with or without neuromuscular diseases, ages 2 years or older, for a research study. It will test the feasibility of using a wearable gait sensor to collect data as part of a new outcome measure for use in future clinical trials.

This study does not involve an intervention. Enrolled participants will be asked to perform approximately 30 minutes of light to moderate functional mobility testing (walking, jogging) while wearing a belt with a mobile phone or smartwatch to collect health status data. Participants will be required to meet with a study team member at the site in Davis or Sacramento or a local community space, or participate over a video call. They can take part in just one or multiple visits.

To be eligible, individuals must meet the following criteria:

- Ambulatory children, teens, and adults, ages 2 and older
- Individuals with Duchenne or Becker muscular dystrophy, facioscapulohumeral muscular dystrophy, Charcot-Marie-Tooth disease, or spinal muscular atrophy
- Or volunteers without a neuromuscular condition who are healthy enough to exercise

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To inquire about participation, contact study coordinator Erik Henricson at [EHenricson@UCDavis.edu](mailto:EHenricson@UCDavis.edu) or 301-466-2531.



**VYVGART**<sup>®</sup>  
(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<sup>®</sup> (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.



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<p><b>Important Information about VYVGART® (efgartigimod alfa-fcab); Rx only.</b></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 <a href="http://www.vyvgart.com/pi">www.vyvgart.com/pi</a> 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><b>What are the common side effects of VYVGART?</b></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><b>What is VYVGART?</b></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>• <b>Undesirable immune reactions (hypersensitivity reactions).</b> 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><b>What are the effects of VYVGART on other drugs?</b></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><b>What is the most important information I should know about VYVGART?</b></p> <p>VYVGART may cause serious side effects, including:</p> <ul style="list-style-type: none"> <li>• <b>Infection.</b> 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</li> </ul>	<p><b>Immunization</b></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><b>What information should I know about VYVGART and pregnancy and breastfeeding?</b></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><b>Can VYVGART be used in children?</b></p> <p>The safety and efficacy in children (pediatric patients) have not been established.</p>



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# Facioscapulohumeral Muscular Dystrophy Update

A Q&A with Jeffrey Statland, MD

**F**acioscapulohumeral muscular dystrophy (FSHD) is a genetic disorder that causes the weakening of muscles in the face (facio), shoulders (scapulo), arms (humeral), legs, and trunk. It is estimated to affect about 1 in 15,000 people. The average age of symptom onset is in the teens or 20s, but people can be diagnosed throughout their lifespan, from infancy to old age.

While there can be considerable variability in age of onset and rates of progression, on average it is a chronic

progressive muscular dystrophy; approximately 20% of people over 50 will require a wheelchair.

We talked with Jeffrey Statland, MD, professor of neurology at the University of Kansas Medical Center in Kansas City, Kansas, about FSHD and research developments.



Jeffrey Statland, MD

## What is FSHD?

It's one of the most common muscular dystrophies and the third most prevalent after Duchenne muscular dystrophy (DMD) and myotonic dystrophy (DM).

The most common form, FSHD type 1, is inherited in an autosomal dominant fashion, meaning that only one parent needs to carry the gene for a child to have the condition, so it tends to appear in multiple generations.

## What are current treatments?

Right now, our best treatment is multidisciplinary care, like that offered in the MDA clinics: physical therapy, occupational therapy, braces, or walking aids.

## Have there been any recent advances in treatment?

It's a very hopeful time for people with FSHD, and there's been real progress in the last five to 10 years. In 2010, researchers published the "unified model" for FSHD, which helps explain what happens in FSHD type 1 and type 2. Of the two types of FSHD, 95% of people have type 1, but both types are caused by a genetic flaw that leads to inappropriate expression of a gene called double homeobox protein 4 (*DUX4*).



FSHD is a genetic disorder that causes the weakening of muscles in the face (facio), shoulders (scapulo), arms (humeral), legs, and trunk.

Knowing this has created a target for therapy. We're just starting to see our first disease-targeted therapies in clinical trials. There's a phase 3 trial right now from a company called Fulcrum using a repurposed drug called losmapimod that, in preclinical models, affects the expression of the *DUX4* gene. Other companies are looking into using an antibody to deliver an RNA-based therapy directly to the muscle. They're all probably one to two years away from their first human trial.

## What do you tell individuals waiting for new treatments?

Sometimes people get discouraged because the pace of drug development is slow, but I think we've turned a corner. There are many companies investigating approaches for treating FSHD. We'll see more clinical trials in addition to the one underway.

I want to encourage people who are affected and their family members to get involved. Let your clinics know you're interested because there'll be opportunities in the next few years. We still have lots of questions about FSHD that we don't understand, so people being involved is very important. It takes everyone working together for us to develop these therapies. [Q](#)

# Back to School in Style

8 adaptive products to help people of all ages express themselves through style

When you look good, you feel good. That maxim is true at every age, from the preschooler who insists on wearing their superhero cape everywhere to the college student sporting their school colors. Quest is proud to present our picks for stylish adaptive products to help students with disabilities (and parents, too!) confidently take on the back-to-school season.



### Get your kicks

Billy Footwear's innovative flip-top design makes their shoes a cinch to put on, even with braces or orthotics. Their sneakers, boots, loafers, and boots for men, women, and children are available in a variety of widths. [BillyFootwear.com](http://BillyFootwear.com)

### Love what's underneath

Slick Chicks underwear are the perfect base for anything else you wear. Low-profile side fasteners make them easy to put on or take off whether you are sitting, standing, or lying down. The soft antimicrobial fabric and attractive cuts are comfortable and cute. Also check out their front-fastening adaptive bras. [SlickChicksOnline.com](http://SlickChicksOnline.com)



### Sit in style

A great pair of jeans is a style must, and the popular Mutual Weave Adaptive Seated Denim Men's Straight Fit Jean, sold at JCPenney, comes in stretchy light, medium, or dark-wash denim. These jeans have a high back, Velcro side seams, and pull-on loops, making for easy dressing and undressing for wheelchair users. [JCPenney.com](http://JCPenney.com)



### Make a face

Inspired by universal design, GUIDE Beauty wants to make applying makeup a breeze. Their luxurious applicators are shaped to guide your hand. Their makeup formulas are also cruelty-free and vegan. [GuideBeauty.com](http://GuideBeauty.com)





## Claim your all-weather superpowers

The KoolKape, by Koolway Sports, has a long list of awesome features: a water-resistant outer shell, moisture-wicking lining, adjustable sleeves, zip-off hood, multiple pockets, and a variety of vibrant colors. Available in child and adult sizes, the KoolKape can be tucked around the body for coziness or spread over power wheelchair controls to protect them from the elements.

[KoolwaySports.com](http://KoolwaySports.com)



### +KOOLKAPE SPECIAL OFFER

Koolway Sports is offering MDA families special discounted pricing on the KoolKape. Use the QR code at left to claim the special offer through Koolway Sports. In addition, the nonprofit Lori's Voice is willing to fund a large portion of a KoolKape purchase on an individual basis and Koolway Sports will waive the nonfunded amount, making it available to MDA families at no cost. To apply for this funding, contact Koolway Sports at [info@KoolwaySports.com](mailto:info@KoolwaySports.com) for an initial quote for your product, then visit [LorisVoice.org/app-for-assistance.html](http://LorisVoice.org/app-for-assistance.html) and include the quote in your application.

## Button up

Looking for more independence when dressing? The RMS Button Hook with Zipper Pull is an essential daily living tool for anyone with limited dexterity. The easy-to-grip rubber handle has a wire loop on one end for navigating button-holes and a hook on the other end for pulling zippers. Available at [amazon.com](http://amazon.com)



### +MORE ONLINE

Read about Madison Lawson, a style influencer in the MDA community, at [mdaquest.org/BeautyHacks](http://mdaquest.org/BeautyHacks).



## Treat your hair right

An Allure “Best of Beauty” winner, the soft silicon Manta Healthy Hairbrush has a unique grip design that is great for people with limited dexterity, and it frees knots with minimal hair breakage. [MantaHair.com](http://MantaHair.com)

## Splurge on fashion

Never have to fish your phone out of your bag again with the Bandolier Crossbody phone case. It pairs the style of a designer handbag with the ease of a phone case. The open face offers easy screen access while the flip side has a compartment for cash, cards, etc. Choose from tons of colors, materials, and accessories to create your unique look.

[BandolierStyle.com](http://BandolierStyle.com)



## ADVERTISEMENT

RADICAVA ORS® (edaravone) helps slow the loss of physical function in patients with ALS.

# FOR YOUR MOMENTS BIG AND SMALL



Actor portrayals.

## RADICAVA ORS® NOW AVAILABLE FOR ALS

RADICAVA ORS® offers an **oral option** that's formulated to fit your life's routines.

ALS=amyotrophic lateral sclerosis.

### INDICATION

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

### IMPORTANT SAFETY INFORMATION

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

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

- have asthma.
- are allergic to other medicines.



Not actual product size.

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

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



# RADICAVA ORS<sup>®</sup>: Oral Formulation of a Proven Treatment for ALS



RADICAVA ORS<sup>®</sup> helps **slow the loss of physical function** in patients with ALS. Results may vary.



The 5 mL dose may take **only a few minutes to administer** on treatment days.<sup>a</sup>

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

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

- 5.9% of patients discontinued RADICAVA ORS<sup>®</sup> because of side effects in a 24-week safety study in 185 patients with ALS

<sup>a</sup>The initial treatment cycle starts with daily dosing of RADICAVA ORS<sup>®</sup> for 14 days followed by a 14-day drug-free period. Subsequent treatment cycles include daily dosing 10 out of 14 days followed by a 14-day drug-free period.



**Talk to your doctor today about RADICAVA ORS<sup>®</sup> and view informative videos at [RadicavaORSVideos.com](http://RadicavaORSVideos.com).**

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

## IMPORTANT SAFETY INFORMATION

(continued)

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

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

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

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

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

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

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

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



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**Radicava ORS<sup>®</sup>**  
(edaravone) Oral Suspension  
105mg/5mL

<p style="text-align: center;"><b>PATIENT INFORMATION</b>  <b>RADICAVA</b> (ra di kā vah)  (edaravone) injection  for intravenous use  <b>RADICAVA ORS</b> (ra di kā vah o r s)  (edaravone) oral suspension</p>	<p><b>What are the possible side effects of RADICAVA and RADICAVA ORS?</b></p> <p>RADICAVA and RADICAVA ORS may cause serious side effects including:</p> <p>1. Hypersensitivity (allergic) reactions. Hypersensitivity reactions have happened in people receiving RADICAVA or taking RADICAVA ORS and can happen after your medicine has been given. Tell your healthcare provider right away or go to the nearest emergency room if you have any of the following symptoms:</p> <ul style="list-style-type: none"> <li>• hives</li> <li>• breathing problems</li> <li>• itching</li> <li>• swelling of the lips, tongue, face</li> <li>• dizziness</li> <li>• wheezing</li> <li>• fainting</li> </ul>
<p><b>What are RADICAVA and RADICAVA ORS?</b></p> <p>RADICAVA and RADICAVA ORS are prescription medicines used to treat people with amyotrophic lateral sclerosis (ALS). It is not known if RADICAVA or RADICAVA ORS are safe and effective in children.</p>	<p>2. Sulfite allergic reactions. RADICAVA and RADICAVA ORS contain sodium bisulfite, a sulfite that may cause a type of allergic reaction that can be serious and life-threatening. Sodium bisulfite can also cause less severe allergic reactions, for example, asthma episodes, in certain people. Sulfite sensitivity can happen more often in people who have asthma than in people who do not have asthma. Tell your healthcare provider right away or go to the nearest emergency room if you have any of the following symptoms:</p> <ul style="list-style-type: none"> <li>• hives</li> <li>• trouble breathing or swallowing</li> <li>• itching</li> <li>• swelling of the lips, tongue, face</li> <li>• dizziness</li> <li>• asthma attack (in people with asthma)</li> <li>• wheezing</li> <li>• fainting</li> </ul>
<p><b>Do not receive RADICAVA or RADICAVA ORS if you</b> are allergic to edaravone or any of the ingredients in RADICAVA and RADICAVA ORS. See the end of this leaflet for a complete list of ingredients in RADICAVA and RADICAVA ORS.</p>	<p>Your healthcare provider will monitor you during treatment to watch for signs and symptoms of all the serious side effects and allergic reactions.</p>
<p><b>Before you take RADICAVA or RADICAVA ORS, tell your healthcare provider about all of your medical conditions, including if you:</b></p> <ul style="list-style-type: none"> <li>• have asthma.</li> <li>• are allergic to other medicines.</li> <li>• are pregnant or plan to become pregnant. It is not known if RADICAVA or RADICAVA ORS will harm your unborn baby.</li> <li>• are breastfeeding or plan to breastfeed. It is not known if RADICAVA or RADICAVA ORS passes into your breastmilk. You and your healthcare provider should decide if you will receive RADICAVA or RADICAVA ORS or breastfeed.</li> </ul> <p>Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.</p>	<p>The most common side effects of RADICAVA and RADICAVA ORS include bruising (contusion), problems walking (gait disturbance), and headache.</p> <p>These are not all the possible side effects of RADICAVA and RADICAVA ORS. For more information, ask your healthcare provider or pharmacist.</p> <p><b>Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088. You may also report side effects to <a href="http://www.fda.gov/medwatch">www.fda.gov/medwatch</a> or Mitsubishi Tanabe Pharma America, Inc. at 1-888-292-0058.</b></p>
<p><b>How will I receive RADICAVA?</b></p> <ul style="list-style-type: none"> <li>• You will be prescribed RADICAVA by a healthcare provider and told how often you will receive RADICAVA.</li> <li>• RADICAVA will be given by intravenous (IV) infusion into your vein.</li> <li>• It takes about 1 hour to receive the full dose of RADICAVA.</li> <li>• Your healthcare provider will monitor you closely during your treatment with RADICAVA.</li> </ul> <p><b>How will I take RADICAVA ORS?</b></p> <ul style="list-style-type: none"> <li>• See the detailed <b>Instructions for Use</b> on how to take RADICAVA ORS at the end of this Patient Information leaflet.</li> <li>• You will be prescribed RADICAVA ORS by a healthcare provider and told how often you will take RADICAVA ORS.</li> <li>• RADICAVA ORS is to be taken by mouth or by using a feeding tube.</li> <li>• RADICAVA ORS should be taken in the morning on an empty stomach. You should stop eating at bedtime. <ul style="list-style-type: none"> <li>– <b>Do not</b> eat or drink anything 8 hours before each dose of RADICAVA ORS if you eat a high-fat meal.</li> <li>– <b>Do not</b> eat or drink anything 4 hours before each dose of RADICAVA ORS if you eat a low-fat meal.</li> <li>– <b>Do not</b> eat or drink anything 2 hours before each dose of RADICAVA ORS if you take a calorie supplement.</li> </ul> </li> <li>• You should wait at least 1 hour after taking your medicine before eating or drinking anything except water.</li> </ul>	<p><b>What are the ingredients in RADICAVA and RADICAVA ORS?</b></p> <p><b>Active ingredient:</b> edaravone</p> <p><b>RADICAVA Inactive ingredients:</b> L-cysteine hydrochloride hydrate, sodium bisulfite, sodium chloride, phosphoric acid, and sodium hydroxide.</p> <p><b>RADICAVA ORS Inactive ingredients:</b> L-cysteine hydrochloride hydrate, polyvinyl alcohol, simethicone emulsion, sodium bisulfite, sorbitol, and xanthan gum. Phosphoric acid and sodium hydroxide are added to adjust to pH 4.</p> <p>Marketed and distributed by: Mitsubishi Tanabe Pharma America, Inc., a US subsidiary of Mitsubishi Tanabe Pharma Corporation, 525 Washington Blvd., Suite 400, Jersey City, NJ 07310</p> <p>For more information, go to <a href="http://www.Radicava.com">www.Radicava.com</a> or call 1-888-292-0058.</p>



Anastasia Corp joined her high school cheer squad.

# After-school Activities

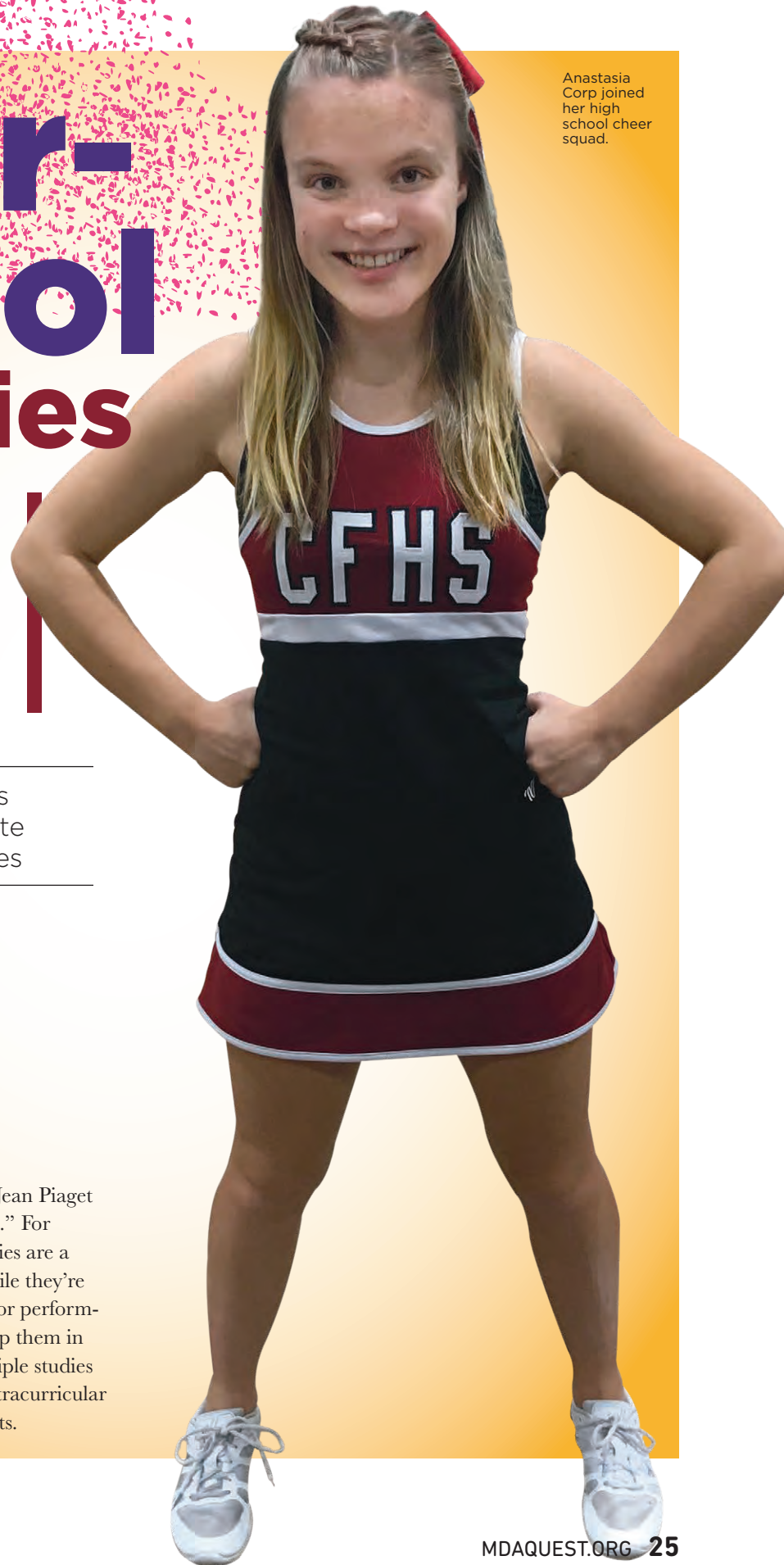
for **All**

Children with disabilities can and should participate in extracurricular activities

BY DONNA ALBRECHT



**R**enowned developmental psychologist Jean Piaget wrote, “Play is the work of childhood.” For school-age kids, extracurricular activities are a way of incorporating play into their day. While they’re having fun playing a sport, making artwork, or performing, they’re also developing skills that will help them in school and throughout life. In addition, multiple studies have found positive relationships between extracurricular participation and mental health in adolescents.



## +QUEST PODCAST

Listen to a conversation with Leah Zelaya, a teen in the MDA community who dances, cycles, models, and acted in the movie "Marry Me" with Jennifer Lopez at [mdaquest.org/podcast/LeahZelaya](https://mdaquest.org/podcast/LeahZelaya).



Miguel Morris joined his high school marching band.



Every child, including, of course, those living with neuromuscular diseases, deserves the opportunity to participate in extracurricular activities in school.

The start of a new school year is a good time to evaluate the extracurricular opportunities at your child's school, identify what appeals to them, and determine how they can get involved. Finding ways to participate may take some planning for students with physical disabilities, but the results are worth it.

### Pursuing interests

Each student will find themselves drawn to different activities. Some enjoy activities like the school newspaper and yearbook that blend language skills with interacting with the school community. Participation in the arts can include playing an instrument, singing, photography, painting, and drama. Other students might prefer foreign language or service clubs, chess or online gaming competitions, student government, or the debate team.

Miguel Morris, 27, who lives with spinal muscular atrophy (SMA), has always loved drums. When he was a young child, his mother bought him a drum set for Christmas so he could pursue his interest. When he went to high school in the Baltimore area, he wanted to join the marching band as a snare drummer.

Miguel, who uses a power wheelchair, doesn't fit the

typical idea of a marching band drummer, but he did it with a little help from aides who carried his drum onto the field while he rolled into position, keeping time with the rest of the band.

"Miguel has never thought about why he couldn't do something. He knows what he wants and he finds a way," says his mother, Michelle Jones.

A heaping portion of credit for that attitude goes to Michelle, who supported Miguel every step of the way and advocated for his needs. For example, his high school gym was accessible by either going upstairs or exiting the building and going to an accessible exterior door. For Miguel and other disabled students, this was a burden in bad weather. Michelle brought this to the school's attention and convinced them to install an elevator.

"Be ready to speak up and stand out," Michelle advises other parents and caregivers. "My dream is that if Miguel wanted to be president of the United States, they'd be making the White House accessible."

### It's the law

Giving your child the opportunity to pursue their interests starts with understanding their rights.

The Individuals with Disabilities Education Act (IDEA) and Section 504 of the Rehabilitation Act, federal laws governing special education, both provide for participation in nonacademic and extracurricular activities. A child's Individualized Education Program (IEP) or 504 plan should include any after-school activities they'd like to participate in and the support services and aids necessary to support those activities.

Schools are required to make accommodations for children with disabilities to participate in extracurricular activities, including athletics, unless doing so would change the nature of the activity or be an undue burden on the school.

For example, having a provision for a particular extracurricular activity in an IEP does not guarantee participation if the activity requires tryouts or auditions. However, reasonable accommodations should be made to allow a student the opportunity to try out.

"Undue burden" is defined as an action requiring significant difficulty or expense. If you feel a school is unfairly claiming an undue burden or using it as an excuse to enable discrimination against your child, you can file a complaint with the US Department of Education Office for Civil Rights (OCR). (See Education Resources on page 27.)

If your child's educational needs change or they want to do an activity that will require the school district to supply equipment, transportation, or services, you can call an IEP meeting to work through the details. Come to the meeting



Marcus Onsum  
coaches  
adaptive sports.



with documentation to educate any school staff who are not aware that extracurricular activities or adapted athletics can be made a part of a student’s educational plan, and be prepared to advocate for your child’s rights.

Depending on your child’s maturity and passion for the activity, it may be a good idea to bring them to the meeting to show their commitment to the activity and to teach them negotiating skills they can use in the future.

### Athletic pursuits

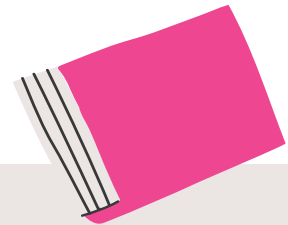
In 2013, the OCR released guidance addressing participation in extracurricular athletic programs. The guidance stated that students with disabilities should not be excluded from existing athletic programs based on stereotypes or assumptions about their disabilities, and schools must provide reasonable modifications or necessary aids that would allow participation. When students with disabilities cannot be included in existing sports with reasonable modifications, the guidance urges — but does not require — school districts to create opportunities for students with disabilities to participate in inclusive or adaptive athletic activities.

Robbinsdale Area Schools in suburban Minneapolis is



Growing up, I was always finding ways to modify neighborhood games and activities with my friends so my brother and sister who have spinal muscular atrophy could participate.

—Marcus Onsum



## EDUCATION RESOURCES

Use these resources to help children with disabilities pursue their interests in and out of the classroom.

### Access Workshops

Access to Education: K-12 is an on-demand virtual workshop covering common considerations for K-12 educational planning, including 504 plans and Individualized Education Plans (IEPs). This is one in a series of workshops addressing access barriers at [mda.org/AccessWorkshops](http://mda.org/AccessWorkshops).

### American Association of Adapted Sports Programs

This organization shares strategies and resources for developing school adapted athletics programs at [AdaptedSports.org/services](http://AdaptedSports.org/services).

### Center for Parent Information & Resources

Browse the resource library of this information hub for parents of children with disabilities, or search for a specific topic, such as “Specifying Related Services in the IEP,” at [ParentCenterHub.org/cpir-resource-library](http://ParentCenterHub.org/cpir-resource-library).

### K-12 Education Resources

The in-depth Teacher’s Guide, IEP Process overview, and materials to teach educators about specific diseases are available on MDA’s Community Education webpage. Visit [mda.org/education](http://mda.org/education) and scroll down to K-12 Education Resources.

### MDA National Resource Center

The Resource Center provides support via phone or email for individuals and families looking for information about neuromuscular diseases, services, activities, and more. Resource Center staff are available Monday through Friday, 9 a.m. to 5 p.m. CT, and are typically able to answer questions within one to two business days. Call **833-ASK-MDA1** or email [resourcecenter@mdausa.org](mailto:resourcecenter@mdausa.org).

### Office for Civil Rights (OCR)

Find instructions for filing a complaint against a school if you believe they are discriminating against a student with a disability and learn about laws protecting students at [ed.gov/ocr](http://ed.gov/ocr), or call the OCR at **800-421-3481 (TDD 800-877-8339)**.

“

Our kids compete in the same state tournaments and earn the same championship trophies as their non-disabled peers.

—Marcus Onsum

one school district putting this guidance into practice.

“Growing up, I was always finding ways to modify neighborhood games and activities with my friends so my brother and sister who have spinal muscular atrophy could participate,” says Marcus Onsum, a teacher and head coach of Developmental Adapted Physical Education in Robbinsdale.

His program provides varsity-level athletic opportunities for students with disabilities to play adapted soccer, floor hockey, and softball in two divisions: one for students who have physical impairments and another for students who have cognitive impairments.

“Our kids compete in the same state tournaments and earn the same championship trophies as their non-disabled peers and are regularly featured on local news stations and in newspapers just like other high school athletes,” Marcus says.

According to the American Association of Adapted Sports, when a school system adds even one adapted sports program, the overall physical and emotional health of their students with physical disabilities is positively affected, along with their academic performance.

### Positive outcomes

Extracurricular activities are about a lot more than having fun. They help kids make friends, develop deeper connections with their schools

and communities, and explore interests that can not only be enriching, but also lead to careers.

Inspired by watching her older sister, Anastasia Corp, 19, started cheerleading in the fourth grade. At the time, the symptoms of her ocular myasthenia gravis (MG) were mild. As she pursued the sport through the 10th grade, she and her supportive stunt squad practiced extra hard to

work around her eye muscle fatigue, which causes drooping eye lids and poor depth perception. “It was a challenge that I had to overcome, and it took close to two seasons — football and basketball — to get the precision moves down,” she says.

At her high school in Conway, South Carolina, she joined the show choir, which allowed her to perform for a crowd without the high-flying stunts. This experience influenced her direction in life.

Now Anastasia, a sophomore at Horry-Georgetown Technical College in Myrtle Beach, South Carolina, plans to transfer to Appalachian State University in North Carolina to complete her bachelor’s degree in music therapy.

Miguel, who enjoyed being on the field with his high school marching band, developed a deep love of sports, especially football and basketball, during his college years. By studying the elements of gameplay, he learned strategy and took up volunteer coaching for youth basketball and other leagues. Miguel felt almost universally welcomed by the players and parents.

In the summer, he graduated from Concordia University in Chicago with a doctorate in sports administration and leadership. He will enter law school at Drexel University in Philadelphia in the fall, and he hopes to become a sports agent.

Miguel and Anastasia’s stories show the value of ensuring kids with disabilities have equal opportunities to pursue their passions through extracurricular activities. Every child’s future is full of potential and wonder. [📍](#)



Anastasia is now a college student studying music therapy.





# Do You Have Becker Muscular Dystrophy?

Are you interested in participating  
in a clinical trial?

Edgewise Therapeutics is seeking adolescents and adults with **Becker Muscular Dystrophy** for a Phase 2 Trial of EDG-5506 to evaluate safety, tolerability, biomarkers of muscle damage and change in functional measures of an **investigational treatment for BMD**.

Actively recruiting at multiple sites in the US, UK and the Netherlands.

To be eligible, participants must be:

- Male, aged 12 to 50 years with genetic confirmation of, and phenotype consistent with, BMD
- Able to complete functional measure testing such as the NSAA and 100-meter timed test
- Willing and able to travel to one of multiple study sites
- Willing and able to have an MRI

Travel expenses will be paid for by the study for eligible participants.



Taking a New Approach to Protect Muscles

To learn more, please go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov), enter **NCT05291091**  
or contact [studies@edgewisetx.com](mailto:studies@edgewisetx.com)

# Looking for Answers on

# EXercisi

Our knowledge of how physical activity benefits people with neuromuscular disease is growing

BY LARRY LUXNER

**E**lisabeth Kilroy, PhD, is director of MDA's neuroMuscular ObserVational Research (MOVR) Data Hub, which gathers medical and genetic data on several neuromuscular diseases to help researchers gain better understanding of the diseases and design effective clinical trials. She got into the field because her father, brother, and aunt are affected by an unknown type of muscular dystrophy.

"My brother asked me what exercise he could do to maintain his strength so he could play golf," she says.

This is a common question among people living with neuromuscular diseases, who want to get the benefits of physical activity — better cardiovascular health, weight management, mood-boosting endorphins, and the camaraderie of sports — but are all too aware of the realities of living with a progressive muscle disease.

Dr. Kilroy attempted to answer her brother's question in her doctoral dissertation. She studied zebrafish lacking dystrophin protein, which serve as an animal model for Duchenne muscular dystrophy (DMD), and applied neuromuscular electrical stimulation, which sends electrical impulses to nerves, causing muscles to contract.

"We found that high frequency and low voltage was very beneficial for muscle health," she says. This implies that resistance training could improve muscle function for people with muscular dystrophies. The results were published in the journal *eLife* in 2022.

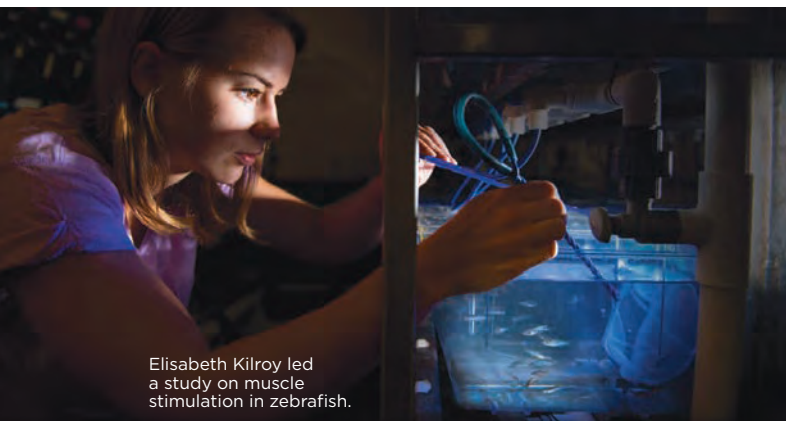
## Research on exercise

"Exercise with a neuromuscular disease is still a very gray area; not enough research has been done on it," Dr. Kilroy says. Yet, the body of knowledge is growing.

In addition to Dr. Kilroy's study on zebrafish, a study published in *Muscle & Nerve* in 2020 found that mild to moderate isometric leg exercises — exercises that involve holding a position for a period of time, such as a wall sit — are not only safe but improve muscle strength and functional ability in boys with DMD.

Similarly, published results from an MDA-supported study conducted at the University of Sydney, Australia, show that moderate-intensity resistance exercise not only is safe but can help significantly reduce the muscle weakness experienced by people with Charcot-Marie-Tooth disease (CMT).

In the study, which involved 60 children ages 6-17 with different types of CMT, participants completed an exercise



Elisabeth Kilroy led a study on muscle stimulation in zebrafish.





Michele MacArthur makes stretching a regular part of her exercise routine.

regimen three times per week for six months using a weighted cuff for the foot (similar to an ankle weight). Researchers found that after six months the progression of muscle weakness was up to 30% slower in the study participants compared to people with CMT who did not exercise. The regimen also strengthened the muscles over a two-year period for those who continued the exercises on their own.

People with neuromuscular disease should talk to their doctors before beginning an exercise program, because an individual's exercise needs and tolerance will vary based on their neuromuscular condition and other factors.

"Always start slow and see how your body responds," Dr. Kilroy cautions.

### Real-world experiences

Growing up in North Texas, Michele MacArthur was an outgoing teenager who enjoyed horseback riding and sports. But in her 20s, she was diagnosed with dermatomyositis — an inflammatory myopathy in which the body's immune system attacks the blood vessels that supply muscles and skin.

"As my disease progressed, I couldn't raise my hands. I couldn't brush my hair. I didn't know if I was going to live," she says. In addition, the long-term corticosteroids she took to treat the disease caused negative side effects, including high blood pressure and poor blood sugar control.

Yet, rather than give up on physical activity, Michele made it a part of her life. Now 55, she's an international

master-certified health and wellness coach with the Dr. Sears Wellness Institute. She focuses on a holistic approach to healthy living and regularly swims, runs, walks her dog, and stretches.

Michele credits exercise, attitude, and diet with helping her thrive with her condition, and she coaches others on maintaining wellness and staying active with health challenges.

"You really have to listen to your body," she says. "Everybody is different. For one person, walking to the mailbox is enough, while another goes to the gym. I paced myself to make sure I was exercising consistently, but not pushing myself too much."

Roberto C. Baez Hernández, who lives in the mountain town of Adjuntas, Puerto Rico, with an unspecified form of muscular dystrophy, calls himself a gym addict. "The best thing I can do is exercise and eat well, and that's what I've been doing," the 41-year-old says.

"My condition affects my balance and muscles, but doing exercise helps," Roberto says. He swears by weightlifting, which he does with help due to his lack of balance, and using a rowing machine.



Roberto C. Baez Hernández calls himself a gym addict.

## PHYSICAL THERAPY RESOURCES

Physical therapy is beneficial for many neuromuscular diseases and a great way to start moving. Learn more with these resources.

### At-Home Physical Therapy Series

These guides include illustrations and detailed instructions on performing physical therapy exercises for certain diseases. The series started with a guide for Duchenne muscular dystrophy (DMD) and is expanding to other diseases. Visit [mda.org/education](https://mda.org/education) and scroll down to “Disease Specific Resources.”

### MDA Care Centers

Multidisciplinary care teams at MDA Care Centers often include physical and occupational therapists. Find a Care Center at [mda.org/CareCenters](https://mda.org/CareCenters).

### MDA Engage Webinar: Physical Therapy, Exercise, and Pompe Disease

This recorded webinar includes exercises to do at home. [youtube.com/watch?v=Fd24fiYowWk](https://youtube.com/watch?v=Fd24fiYowWk)

Check [mda.org](https://mda.org) often for resources related to physical therapy, health, and wellness with a neuromuscular disease.

### Early start

Amanda Shirk, a Pennsylvania mom whose 4-year-old son, William, has spinal muscular atrophy (SMA), says exercise became an important part of William’s life even before he was diagnosed.



William Shirk loves his aquatic therapy sessions.

“Until he was 9 months old, William met all his milestones right on time. But as he got closer to his first birthday, he still had no interest in crawling and would not bear any weight on his legs,” she says. “He began early-intervention physical therapy once a week, and soon we were incorporat-

ing PT into almost every part of his daily routine — playtime, bath time, mealtime; he was constantly working those muscles.” Yet he didn’t seem to be improving. In fact, he could not reach his arms over his head and frequently fell over while sitting.

A pediatric neurologist at the MDA Care Center at Children’s Hospital of Philadelphia diagnosed William with SMA type 2. Four weeks later, William received his first injection of nusinersen (Spinraza), the first drug approved by the US Food and Drug Administration (FDA) to treat SMA.

In another stroke of luck, two months after his diagnosis, William received the newly approved single-dose gene therapy Zolgensma. Within a month, the toddler was bearing weight on his legs with support, and he was able to raise his hands above his head.

“Since then, he’s only gotten stronger every day,” Amanda says. “He works hard, with two physical therapy and two occupational therapy appointments every week, as well as sessions of aquatic therapy.”

### Beginning to exercise

William’s experience involves two of the best ways to start moving for people who have been inactive or feel that they’re losing strength: physical therapy and water exercise.

Jennifer Wallace Valdes, owner of the Los Angeles-based Duchenne Therapy Network, is also director of the physical therapy program at CureDuchenne and has given presentations for MDA. She calls physical therapy “one of the few treatments that can be lifelong, and it can help manage a lot of the impairments or symptoms related to a neuromuscular disease.”

Jennifer emphasizes that exercise does not have to stop once an individual is no longer ambulatory. “Once you’ve stopped walking, the need for physical therapy and occupational therapy actually increases,” she says.

Claudia Senesac, clinical associate professor of physical therapy at the University of Florida’s College of Public Health, suggests swimming or any type of water exercise. In a pool, the water carries about 90% of a person’s body weight, which feels freeing. Plus, Senesac points out, swimming can be social and fun. “It’s usually a family activity,” she says.

While there is no definitive piece of advice on exercising with neuromuscular disease, a growing body of evidence shows that it can be beneficial if approached carefully. [Q](#)

Larry Luxner is a journalist based in Israel.



### Did You Know?

**Supporting mental health** is an important part of MDA’s mission to transform the lives of people living with neuromuscular diseases. Find resources to support your mental health needs in MDA’s Mental Health Hub at [mda.org/mental-health](https://mda.org/mental-health).





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**B**illy Ellsworth is beating the odds against Duchenne muscular dystrophy (DMD). Diagnosed at age 4, the Pittsburgh native, now 21, is still able to walk. Most boys with DMD lose that ability around age 11.

Billy's DMD may have been slowed thanks to the drug eteplirsen, which he began taking at age 10, when he enrolled in a clinical trial.

An MDA seed grant awarded in 1998 to Steve Wilton, PhD, BSc (Hons), professor and Foundation Chair in Molecular Therapy at Murdoch University in Australia, helped fuel the development of eteplirsen, which was approved by the US Food and Drug Administration (FDA) in 2016 under the name Exondys 51.

# Path to



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The story behind a breakthrough DMD drug illustrates how MDA-funded research leads to new treatments for neuromuscular diseases

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BY KAREN DOSS BOWMAN



# Progress

Image: iStock.com/DRAfter123

“This was research we first funded over 20 years ago, which was seen as a very outside-the-box idea at the time, but one we felt was worth exploring,” says Sharon Hesterlee, PhD, MDA’s chief research officer.

Throughout our history, MDA has been supporting research across the spectrum of drug development, from discovery to clinical trials. As the nation’s largest nonprofit supporter of neuromuscular disease research, encompassing more than 40 diseases, MDA has invested more than \$1 billion since our founding in 1950, contributing to many advances in muscle biology and therapy development.

### Treatment breakthrough

A lead researcher in the development of Exondys 51, Dr. Wilton used the MDA seed grant to investigate exon skipping, an innovative technique to modify certain genetic mutations causing DMD. (See Understanding Exon Skipping, below.)

Dr. Wilton’s work on DMD began in 1991, but it was made possible by geneticist Louis Kunkel, PhD, of Boston Children’s Hospital, who received an MDA grant funding his

laboratory’s work that identified the gene that causes DMD in 1986. This was the first identified gene behind a neuromuscular disease.

DMD, which primarily affects males, is caused by a mutation in the dystrophin gene on the X chromosome. About 1 in every 4,600-6,300 males born worldwide has DMD, which causes muscle loss and progressive weakness due the body’s inability to produce functional dystrophin, a protein that gives strength and stability to muscle cells.

For more than a decade, Dr. Wilton’s laboratory worked to develop antisense oligonucleotides (ASOs) that give cells the ability to skip sections of the damaged dystrophin gene message. This allows the cells to create partially functional dystrophin protein.

“There was so much skepticism about this technology from the start,” Dr. Wilton says. “ASO work had promised a lot, but there were problems with making these drugs and understanding how it was working. The advantage we had was that instead of trying to knock out the gene, we were changing the expression. If it was working, we would see an altered gene message and the appearance of a functional dystrophin protein.”

Exondys 51 specifically targets a section of genetic code called exon 51 in the dystrophin gene. It’s estimated that 13% of boys with DMD could benefit from skipping exon 51.

The process of developing the first exon-skipping drug took 20 years. The drug went through three rounds of clinical trials, starting around 2008, and involved a small cohort of boys with DMD who had the same type of errors in their dystrophin genes. Its approval in 2016 marked a watershed moment — the first FDA-approved disease-modifying therapy for a disease in MDA’s program.

Since then, three more exon-skipping drugs have been developed to treat DMD: Vyondys 53, approved in 2019, and Viltepso, approved in 2020, are designed to skip exon 53. Amondys 45, approved in 2021, is designed to skip exon 45. Together, the currently available exon-skipping therapies could benefit more than 20% of boys with DMD.

Now, Dr. Wilton’s team is examining the application of exon-skipping for other conditions, including spinal muscular



Billy Ellsworth and Steve Wilton

## UNDERSTANDING EXON SKIPPING

Some neuromuscular diseases, like Duchenne muscular dystrophy (DMD), are caused by genetic mutations that result in little or no production of a protein that muscles need. Exon skipping is a strategy that coaxes cells to skip over a targeted exon (section of genetic code), allowing cells to create shortened but partially functional protein.

To understand this better, think of the genetic code for a protein as a sentence. Cells have to read the genetic “sentence” in units of three “letters” each. For example:

**The mad cat ate the fat rat and the big bat.**

Errors can occur when a deletion mutation disrupts the three-letter reading pattern, creating “words” that don’t make sense. When cells can’t read the genetic sentence, they can’t make functional protein.

**The mad cat ate the fat rat and the big bat.**

Deletion

**The mad cat ate the tra tan dth ebi gba t.**

Exon skipping causes cells to skip a targeted section, creating a readable sentence.

**The mad cat ate the tra tan dth ebi gba t.**

Skipped exon

**The mad cat ate the big bat.**



## A HISTORY OF BREAKTHROUGHS

MDA's research program awards grants to the world's best scientists investigating promising theories and therapies that may accelerate treatments and cures for families living with neuromuscular diseases. Here are FDA-approved drugs for neuromuscular diseases that MDA has directly supported.

Disease	Generic Drug Name	Brand Name	Company	FDA Approval Year	How Do You Take It?	Type of Therapy	Potential Benefit
<b>Amyotrophic lateral sclerosis (ALS)</b>	riluzole	Rilutek/ (Tiglutik)	Sanofi/(ITF Pharma )	1995/2018	Tablet or liquid	Small molecule that blocks glutamate	May slow progression; extends time before breathing tube in the throat needed
<b>Pompe disease</b>	alglucosidase alfa	(Myozyme)/ Lumizyme	Genzyme	(2006)/2010	Intravenous	Enzyme replacement therapy (ERT)	Aids normal breakdown of glycogen
<b>Periodic paralysis</b>	Dichlorphenamide	Keveyis	Strongbridge	2015	Tablet	Small molecule	Decreases attacks of muscle weakness
<b>Spinal muscular atrophy (SMA)</b>	nusinersen	Spinraza	Biogen	2016	Injection	Antisense oligonucleotide (ASO)	Increases the amount of a protein required by nerve cells for the muscles to work properly
<b>Duchenne muscular dystrophy (DMD)</b>	eteplirsen	Exondys 51	Sarepta	2016	Injection	ASO	Targets specific gene mutations to help the body produce functional dystrophin protein, which helps keep muscle cells intact
<b>DMD</b>	deflazacort	Emflaza	PTC Therapeutics	2017	Tablet or liquid	Small molecule	Corticosteroid that helps reduce inflammation; may improve muscle strength and slow disability progression
<b>DMD</b>	golodirsen	Vyondys 53	Sarepta	2019	Intravenous infusion	ASO	Targets specific gene mutations to help the body produce functional dystrophin protein, which helps keep muscle cells intact
<b>DMD</b>	casimersen	Amondys 45	Sarepta	2021	Intravenous infusion	ASO	Targets specific gene mutations to help the body produce functional dystrophin protein, which helps keep muscle cells intact

atrophy (SMA), congenital muscular dystrophy (CMD), and late-onset Pompe disease.

### Great beginnings

MDA's scientific program supports research at critical stages in development, with funding priority given to projects focused on developing therapies. MDA research grants help cultivate talent among emerging investigators and encourage collaboration among researchers.


These grants are especially critical for early-stage research projects. Most grants funded by federal agencies, such as the National Institutes of Health (NIH), require preliminary data. Support for that early data typically comes from private foundations and organizations, such as MDA.

"You can't move ahead without a starting point," says Jerry Mendell, MD, the Curran-Peters Chair of Pediatric Research at Nationwide Children's Hospital's Abigail Wexner Research Institute and professor of pediatrics and neurology at The Ohio State University. "Seed money that helps researchers generate that preliminary data allows investigators to move forward with promising new approaches to treatment."

Dr. Mendell led the clinical trials of eteplirsen and has also been involved in trials of gene therapy drugs for SMA and limb-girdle muscular dystrophy (LGMD).

### Hope around the corner

MDA's research funding plays an essential role in developing therapies for all neuromuscular diseases. Breakthrough therapies can offer individuals living with neuromuscular diseases increased survival and a better quality of life. And a breakthrough drug for one disease has the potential to lead to discoveries for other diseases.

Progress in one area shows that "we can change disease progression," Dr. Wilton says. "There's a cross-fertilization of ideas that come together to ultimately benefit a greater number of patients." 

Karen Doss Bowman is a freelance writer and editor living with progressive muscular atrophy, a subset of ALS, in Bridgewater, Virginia.

### +GRANTS AT A GLANCE

Learn more about research projects MDA is funding at [mda.org/gaag](https://mda.org/gaag).

# 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.

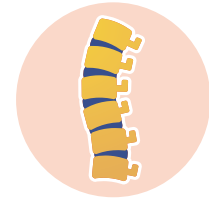
## Corticosteroid treatment now and over time

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](mailto:usmedinfo@ptcbio.com). You may also report side effects to FDA at 1-800-FDA-1088 or at [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

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## A DE&I Winner



Seraphina Shutt

MDA and DECA, which provides marketing and business training for high school and college students, joined forces earlier this year in pursuit of spreading DEI awareness to students.

As part of the Diversity, Equity, and Inclusion (DE&I) Challenge, DECA student members across the country were asked to devise a DE&I strategy and marketing plan that MDA can use to reach other students.

Seraphina Shutt, 15, from West Lincoln High School in North Carolina, was named the first-place winner and received her award in April at the International Career Development Conference in Atlanta.

Along with gaining presentation and communication skills, Shutt also became an MDA supporter and says she wants to plan an MDA fundraiser with her DECA chapter.

“I learned that when we fix a problem like discrimination before it starts — like starting in schools for example — we won’t need to worry about issues like ableism and job discrimination going forward,” Shutt says.

The other students in the top three were Darshan Kommanapalli and Elan Schonfeld of Glenbrook North High School in Illinois.

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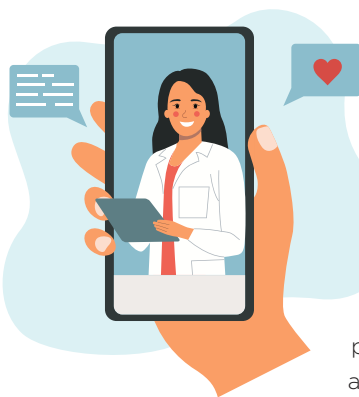
Learn more about the DECA-MDA partnership at [mda.DonorDrive.com/DECA](https://mda.DonorDrive.com/DECA).

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**Micah Thomas**, who lives with merosin-deficient congenital muscular dystrophy, was all smiles for his second birthday MDA fundraiser. The Thomas family, of Carrollton, Texas, has raised more than \$22,000 between his two birthdays. “The prayers and support of our village of people who love Micah will truly be able to impact so many others,” says Jacob, Micah’s father. To create your own fundraiser, visit [mda.org/YourWay](https://mda.org/YourWay).

## Advocacy at Work



MDA continues to advocate for national policies and programs that support families with neuromuscular diseases. The advocacy team is working toward making the temporary emergency measures for telehealth access during the pandemic

permanent, ensuring expanded access to these virtual health services continues. To further this

effort, MDA’s Vice President of Policy and Advocacy Paul Melmeyer recently spoke at the American Telemedicine Association’s annual conference

about the importance of telehealth for the neuromuscular disease community.

The advocacy team has also been hard at work:

- Urging the US Department of Transportation to quickly make lavatories more accessible on the most common airplanes.
- Ensuring the MDA community receives better therapies faster through much-needed reforms at the US Food and Drug Administration.
- Continuing to fight for increased access to lifesaving newborn screening to detect certain neuromuscular conditions early, resulting in better health outcomes.
- Working to pass numerous bills to ensure economic independence for individuals with neuromuscular diseases.

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Learn more and join the effort at [mda.org/advocacy](https://mda.org/advocacy).

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## Community Education Opportunities

### Engage Events

**Genetic Testing in Becker Muscular Dystrophy Webinar:** Sept. 15, 3-4:30 pm ET

**Dental Health in Neuromuscular Disease Webinar:** Sept. 15, 3-4 pm ET

**Technology for Independence Seminar:** Oct. 12 & 13, 3-6 pm ET

**Myasthenia Gravis Symposium:** Oct. 18 & 19, 3-6 pm ET

### Access Workshops

**Access to Medical Care:** Available on demand in August

**Access to Employment:** Available on demand in October

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Sign up at [mda.org/engage](http://mda.org/engage) and [mda.org/AccessWorkshops](http://mda.org/AccessWorkshops).

## Neverending Support

For decades, Branch #38 of the National Association of Letter Carriers (NALC) — the second largest branch in the country — has devised creative ways to raise money for MDA, including mail campaigns and golf tournaments.



NALC #38 officers

This year, the Springfield, New Jersey-based branch came out in full force again. Through office challenges and with the support of the local community, Branch #38 raised more than \$13,000 for Shamrocks. “This exceeded anything I expected,” says Dan Szucs, the vice president of NALC #38. “Our carriers really stepped up to the plate and did not hesitate to donate.”

Since 2018, the branch’s 174 stations have raised more than \$45,000 through Shamrocks. Branch officers also give their time, volunteering at MDA Summer Camp with face painting, popcorn, and letter-writing activities.

# Lessons Learned

Adaptive sports taught me to believe in myself and to strive for success

BY CHAD WILSON

I am a father, a husband, a lawyer, and an athlete. I did not always believe I would have the opportunity to call myself these things. As a child, I did not know how to deal with having Becker muscular dystrophy (BMD). I was angry and bitter, did not know how to control my emotions, and lacked self-confidence. But discovering adaptive sports helped me break away from that negative mindset. Finding success in athletics taught me lessons that have carried over from the court to my everyday life.

## Lesson 1: Hard work pays off.

My first experience with adaptive sports was playing wheelchair floor hockey at MDA Summer Camp. Each summer, the cabins competed in a hockey tournament, with campers using their power wheelchairs or being pushed in manual wheelchairs. That was my favorite thing about camp.

After I received my first power wheelchair at 15, I started playing power hockey, a form of floor hockey played 5-on-5 with a whiffle ball. I joined the Minnesota PowerHockey League and quickly found success, having a knack for scoring goals. This made me look at myself in a more positive light, and I started to realize that my mindset was making having a disability more difficult for me than it had to be.

Finding early success in a local league made me believe I had the sport figured out. But when I began competing on a traveling team called the Minnesota Saints, facing teams throughout North America, I figured out my talent alone would not be enough. I started practicing whenever I had free time, and over time I saw my hard work translate to my play on the court.

This taught me the value of hard work and dedication to achieve my goals.

## Lesson 2: It's OK to rely on others.

While my skills were improving, my team was not finding much success in competitive North American tournaments. This was frustrating, and I even contemplated playing for a different team.



Success in adaptive sports helped Chad Wilson build his overall confidence.

Thankfully, my team captain convinced me to stick with the Saints. He told me that for our team to find success I would need to start trusting my teammates; I couldn't do everything myself.

After we adjusted our team strategy and I embraced a more team-oriented play style, we won our first of four North American power hockey cups.

This experience taught me it is OK to ask for help. In fact, it is critical in difficult times.



### Lesson 3: Look for the positive in every situation.

About nine years ago, I began to play power soccer, a form of indoor soccer designed for power wheelchair users. Players use a guard on the front of their wheelchair to hit the ball instead of their feet.

My time playing power soccer began in a bittersweet fashion. I had been interested in the sport for years, but I could not afford the equipment. My opportunity came after a close friend who had a neuromuscular disease passed away, and her family generously gave me her soccer wheelchair and guard.

From the first practice, I was hooked. Moving from hockey to soccer was a refreshing change because the rules allow more well-balanced gameplay. Every player can make the same basic moves on the court, regardless of their strength. Having a progressive disease, I was ecstatic to participate in a sport I could keep playing even as my strength changed.

This experience taught me that positive outcomes can come from difficult situations.

### Lesson 4: Be open to building relationships.

Through adaptive sports, I have competed with and met amazing people, many who live with neuromuscular diseases and have had similar life experiences. The relationships I have formed because of adaptive sports go beyond friendships.

During one of my first major soccer tournaments, I competed in a game in which the coach for the opposing team would not be quiet the entire game. She kept yelling, even

with her team up two goals with seconds left in the game. After that game, I told my dad how annoying I thought the coach was.

Shortly after, I attended a power soccer camp. The loudmouth coach also attended, and we got to know each other. A few months later, we started dating.

That loudmouth coach is now my wife and the mother of my two children. We named our daughter after my fallen friend whose wheelchair allowed me to start playing power soccer.

Sports have done more than teach me life lessons — they have given me the opportunity to form lifelong friendships and personal relationships.

“

Sports have done more than teach me life lessons — they have given me the opportunity to form lifelong friendships and personal relationships.

### Putting lessons into practice

The lessons I learned in sports translate to my everyday life.

When I was younger, they helped me deal with having a disability in a healthier way and over time find better control over my emotions. I found a confidence in myself I did not previously possess, pushing myself to set goals I never would have even thought of previously.

This confidence helped push me to pursue and successfully complete law school, accomplishing my dream of becoming an attorney. The skills of discipline I cultivated through sports have been critical to my success in law school and in my career.

Knowing when to rely on others in my everyday life, much like teammates on the court, is an essential skill for life with a neuromuscular disease. Living with a progressive disease makes it difficult to keep a positive mindset all the time. Having friends and family to fall back on is critical to me when I am having a bad day.

Sometimes I remind myself that if I did not have a disability, I would have missed out on meeting all the wonderful people I have met through adaptive sports, including lifelong friends and my wife. Sports have done more than teach me life lessons; they have also given me the gift of family.

My experiences as an athlete impact my life in a positive way every single day. [@](#)



Chad enjoys his roles as father and husband.

Chad Wilson is a disability rights attorney with the Minnesota Disability Law Center. He still plays power hockey and power soccer.

# Who You Gonna Call?

9-year-old receives a Ghostbusters wheelchair from MDA and Magic Wheelchair

Carter Rhodes of North Fort Myers, Florida, had just about the best 9th birthday ever when he received a one-of-a-kind present — a Ghostbusters-themed costume fitted to a new wheelchair. In fall 2021, Carter’s name was drawn as the winner of the Halloween Holiday Joy Instagram contest launched by MDA in collaboration with Magic Wheelchair, a nonprofit that creates fun costumes for kids and adults in wheelchairs.

The wheelchair reveal was in June at the North Fort Myers Fire Department and organized with the International Association of Fire Fighters (IAFF) Local 1826, a branch active in Fill the Boot events and fundraising for MDA research, care, and advocacy.

Carter’s favorite Ghostbusters character is Phoebe from “Ghostbusters: Afterlife,” and his new wheelchair resembles the Ecto-1 vehicle with Phoebe’s gunner seat. It also features robotics that Carter can activate with an adaptive controller.

“He loves it,” says Tara, Carter’s mother. “He still has his wheelchair, and that’s one of the brilliant things about how they designed it. It fits through regular doorways so he can be a Ghostbuster every single day, not just on special occasions.”



The day was even more special because of Carter’s family ties to IAFF and MDA. His great-grandfather on his mother’s side was a fire captain and a liaison for fundraising with MDA before retiring. He helped organize events such as Fill the Boot.

“Our family has always been involved, even before I was born,” Tara says.

Between getting the costume, new wheelchair, Ghostbusters birthday cake, a special message from Ghostbusters actor Carrie



Carter Rhodes’ Ghostbusters-themed wheelchair features robotics he can activate with an adaptive controller.

Coon, and autographed sneakers from actress McKenna Grace, what was Carter’s favorite part?


“Pretty much all of it,” he says. [Q](#)

## + ENTER THE LASTING IMPRESSION PHOTO CONTEST


What amazing moments have you captured on camera? Share a photo of a meaningful moment for you or a loved one with a neuromuscular disease, and it could be selected to appear in a future issue of Quest. All photo entries must be submitted by Sept. 16, 2022. Submit photos at [SurveyMonkey.com/r/QuestPhoto](https://www.surveymonkey.com/r/QuestPhoto) or scan this QR code.





A stylized illustration of an astronaut in a blue suit with a white helmet, floating in space. The astronaut is holding a small red and white striped flag.

Do you know a boy  
with Duchenne  
muscular dystrophy?

A stylized illustration of an astronaut in a red suit with a white helmet, floating in space. The astronaut is holding a small red and white striped flag.

If so, consider CIFFREO—a  
clinical study for boys with  
Duchenne.

## Can we treat Duchenne differently?

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

That's why we're conducting CIFFREO—a clinical study that will assess if an investigational gene therapy (the study drug) is safe and the effect it has on muscles in boys with Duchenne.

## Who can join the CIFFREO study?

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

## What can you expect if participating?

This study will require approximately 49 study visits, will last for up to five or six years, (depending on whether your child is randomly assigned to initially receive the study drug or the placebo), and will require some overnight stays in the hospital. The study drug and any study-related assessments will be provided for you. If you do not live close to the study site, travel support (and accommodation, if needed) will be made available for you and some members of your family.

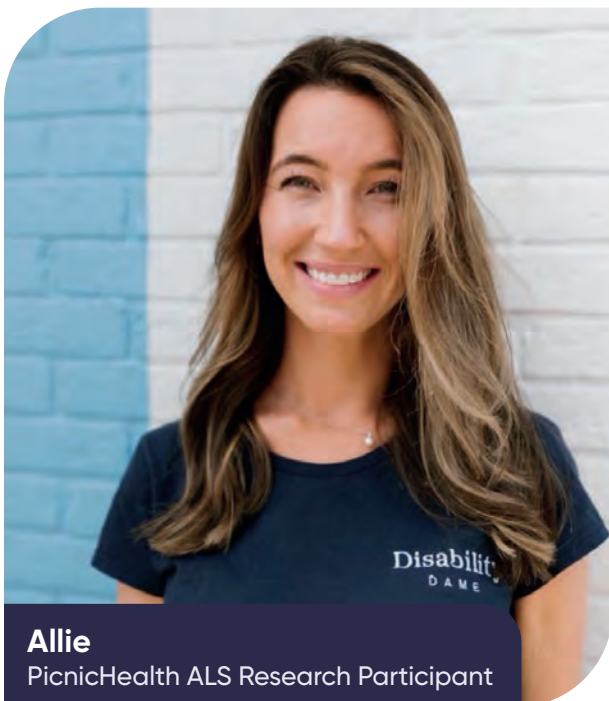
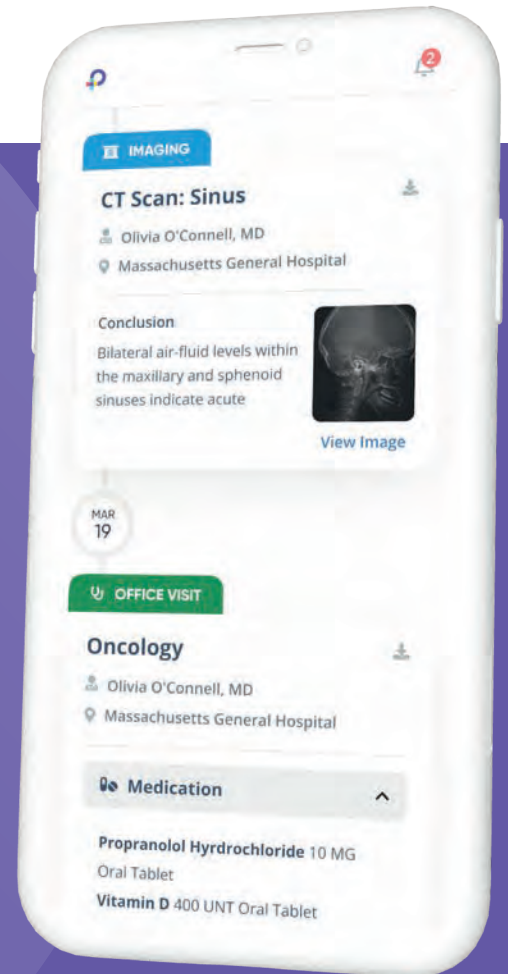
**If you think this study might be right for you and your child,  
visit [CIFFREODuchenneTrials.com](https://www.ciffreoduchennetrials.com) to learn more.**



# Get Your Medical Records

Join PicnicHealth to get all of you or your loved one's medical records in one place.

If qualified, you will get a **free** account—we do the work to collect and organize all of the medical records into a secure, online portal. Also, you can get paid to contribute to important research anonymously.



**Allie**  
PicnicHealth ALS Research Participant



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or **scan** this code with your phone camera



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Accelerate research from home — 5 minutes can make a huge impact.