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MOVING AHEAD with

Evrysdi® (risdiplam)



1,800+

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

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

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

What is Evrysdi?

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

Important Safety Information

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

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

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

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



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

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

Important Safety Information (continued)

Tell your healthcare provider about all the medicines you take.

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

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

The most common side effects of Evrysdi include:

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

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

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

Please see accompanying brief summary for additional Important Safety Information.

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

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



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

What is EVRYSDI?

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

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

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

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

How should I take EVRYSDI?

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

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

Taking EVRYSDI

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

Reusable Oral Syringes

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

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

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

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

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

How should I store EVRYSDI?

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

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

General information about the safe and effective use of EVRYSDI.

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

What are the ingredients in EVRYSDI?

Active ingredient: risdiplam

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

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EVRYSDI® (risdiplam)

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Genentech, Inc.
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 1 DNA Way
 South San Francisco, CA
 94080-4990

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



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The Journey Ahead

As we at MDA approach the significant milestone of our 75th anniversary in 2025, I am filled with pride and anticipation for the journey that lies ahead. As the CEO of MDA, I am honored to lead an organization with such a rich and important history of impact for the neuromuscular disease community.

At the core of our mission are the children, adults, and families living with neuromuscular diseases. It is with unwavering dedication that we continue to innovate and enhance our offerings to better serve you. In 2024, we have some exciting projects in development, such as a new virtual platform for community education and dialogue, as well as the continuation of our MOVR Data Hub to maximize the development and impact of new therapies. With these and other signature initiatives, MDA is uniquely poised to revolutionize the landscape of patient connectivity and clinical support. As with all of our work in the past three quarters of a century, these initiatives reflect our dedication to advancing neuromuscular disease research and care; each underscores MDA's commitment to empowering and uniting our community.

Through initiatives like the MDA Clinical and Scientific Conference, MDA Summer Camp, and MDA Care Centers — the only national network of multidisciplinary care centers — we continue to expand our reach and impact, providing support and educational resources to our families. Additionally, initiatives like the MDA College Scholarship program (mda.org/scholarship) underscore our commitment to improving opportunities and outcomes for people in the neuromuscular disease community and other disability spaces.

As we think about the future, we recognize the importance of forging new partnerships and enhancing community engagement. I look forward to having you journey with us on the exciting path to our 75th year.



Donald S. Wood, PhD

+TELL US WHAT YOU THINK ABOUT QUEST

What topics would you like us to cover? Is there anything we can do better? Take the 2024 Quest Media Audience Survey to give us your feedback and help shape Quest content in the magazine, blog, and podcast.

Take the survey:
Go to [SurveyMonkey.com/r/QuestSurvey2024](https://www.surveymonkey.com/r/QuestSurvey2024) or scan this QR code.



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

For more than 70 years, MDA has led the way in accelerating research, advancing care, and advocating for the support of people living with muscular dystrophy, ALS, and related neuromuscular diseases and their families. MDA's mission is to empower the people we serve to live longer, more independent lives.

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SUBSCRIPTIONS

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Quest Volume 31, Issue 2

Published by
Muscular Dystrophy Association
1016 W Jackson Blvd #1073
Chicago, IL 60607
800-572-1717
email: quest@mdaUSA.org
Available on the internet at MDAQuest.org
ISSN 1087-1578
Postage paid at Bolingbrook, IL
Nonprofit postal permit number 1446.

Postmaster: Treat as Standard A Mail Only

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A Voice for Change

When I was younger, I used to believe I had to take what the world gave me and be gracious about it. Then, I began sharing bits and pieces of what was “inconvenient” in my life as a woman living with a disability and how I handled them. I quickly realized that most people who don’t live with or love someone with a disability have no idea of the inequities that still exist in the world.

What began as sharing has evolved into advocacy work that I am passionate about. Recently, I took on an expanded role with MDA as the Vice President of Disability Outreach & Empowerment. My overarching mission is to empower you with tools, information, resources, and stories.

Within that overarching mission, I will continue to lead the Quest Media team and will also join forces with other advocates working to create positive change in our world in areas such as accessibility, employment, and positive representation of people with disabilities.

As you read and listen to Quest Media content, I hope you see the potential for our world and feel empowered to speak up for what is right.

What I know for sure is this: Individually, our voices can be powerful agents of change, but when we combine them, they become far too loud to ignore.



Mindy Henderson
Editor-in-Chief, Quest Media
Vice President of Disability Outreach & Empowerment, Muscular Dystrophy Association

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DID YOU KNOW?

FIND YOUR GROUP. You don’t have to navigate your neuromuscular disease journey alone. Join an MDA Community Group to connect with others in similar circumstances, gather resources, and exchange valuable information. MDA offers groups in these categories:

- + **Newly diagnosed** — for those diagnosed within the past year
- + **ALS** — for individuals with amyotrophic lateral sclerosis and their families
- + **Gene therapy** — for those eligible for approved gene therapies

Learn more about MDA Community Groups at mda.org/care/community-groups.

HAVING A NEUROMUSCULAR DISEASE MIGHT AFFECT YOUR BONE HEALTH. Medications for neuromuscular disease symptoms and reduced physical activity can lead to low bone density. Learn what that means for your health and what you can do about it in a Quest Magazine online exclusive at MDAQuest.org/bone-health.

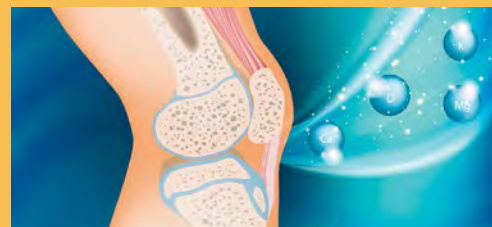


Image: Sakurra/Getty Images



Managing MG Gracefully

Spreading a love for dance inspires Carol Alvarez to keep going

BY REBECCA HUME

Carol Alvarez is a professional ballet dancer, dance instructor, and founder of a New York-based program teaching dance to school children. For Carol, who uses the pronoun they, building a dance career involved chasing their dreams with optimism, hard work, and a few unique challenges. Diagnosed with myasthenia gravis (MG) at 14, the now 25-year-old has found balance and grace while navigating their disease on their quest for success.

“I love how expressive you can be with ballet,” Carol says. “When you are performing and you are able to express yourself, you can forget about everything else in the world — for me, that includes my diagnosis and the challenges of living with MG.”

Carol’s MG causes a degree of muscle weakness, fatigue, and limited stamina every day.

“When you are performing and you are able to express yourself, you can forget about everything else in the world — for me, that includes my diagnosis and the challenges of living with MG.”

— *Carol Alvarez*

Flare-ups can affect their whole body, making it difficult to walk and speak, resulting in hospital stays. Carol receives Vivgart infusions to manage daily symptoms and intravenous immunoglobulin (IVIG) treatments during flare-ups. Physical therapy helps Carol regain strength after flare-ups.

Discovering dance

Carol’s passion for dance began when they took ballroom dance classes in fifth grade, leading to a love of



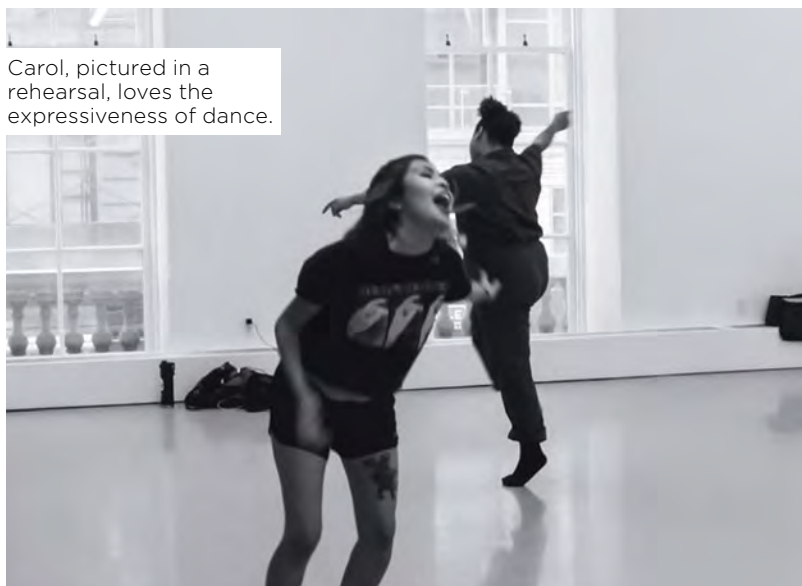
Carol Alvarez began taking ballet as a teen.

ballet in their early teens. They attended the Frank Sinatra School of Performing Arts in Queens, New York. After two weeks of college, Carol realized that dance was their professional calling and left to attend the Joffrey Ballet School in Manhattan.

Ballet school was one of the most challenging — and rewarding — chapters in their life.

“I told myself that if I could get through these four years and do it by myself financially while attending

Carol, pictured in a rehearsal, loves the expressiveness of dance.



Joffrey with an eight-hour dance day, going to work afterward, and managing MG flare-ups — and do the same thing the next day and the next day — if I was able to do that, then I wouldn't let anything get in my way," Carol says. "That was a difficult time in my life, but I decided that I was going to make it, and I did."

Symptoms and sashays

Understanding their body's needs and limitations is an important component of Carol's health. "I get fatigued a lot quicker and more easily than others," Carol says. "I have to be really smart about meds and taking care of myself before and after teaching and rehearsals, and sometimes I sit on the floor and teach from there on hard days."

Their mobility and stamina are most significantly impacted during medication changes and MG flare-ups. During those times, they often need to take a break.

At Joffrey, if Carol had a flare-up or needed rest and recovery, their neurologist provided a note to request that they be excused while in the hospital or allowed to observe dance classes for a few days. When

[Carol's] mobility and stamina are most significantly impacted during medication changes and MG flare-ups. During those times, they often need to take a break.

Carol interviewed to be a dance instructor at the Mark Morris Dance Center after graduation, they were up-front about their diagnosis and needs.

"Before working at Mark Morris, I didn't openly share my diagnosis. I didn't want anyone to think that I was different or weird. But when I interviewed, I was very blunt and clear about my diagnosis and that flare-ups might require me to be hospitalized for a week or two. They are very accommodating; I love working there," Carol says.

Their employer's and fellow teachers' support has helped Carol rest, reset, and come back stronger after flare-ups and setbacks. They continue to perform and teach. The connections they've made through Mark Morris have also inspired them to spread their love of dance.

Sharing their passion

In 2020, Carol started a program to teach dance to children in daycares and public schools throughout New York City. Carol's program gives children an opportunity to express themselves through dance and reminds children to pursue their own dreams.

Carol also became an MDA Ambassador to share their story and mentality with others living with neuromuscular diseases. They hope to show that believing you can achieve whatever goals you set for yourself can open new pathways in life.

"My advice to children and adults is never be afraid of your goals or what you want to do — just keep going," Carol says. "You'll get to your end goal eventually, and it might take longer than you planned, but you will be able to do whatever you set your mind to." [Q](#)

Rebecca Hume is a Senior Specialist and Writer for Quest Media.



PERSONAL STORIES

Find more real-life stories from members of the neuromuscular disease community at [MDAQuest.org/personal-stories](https://mdaquest.org/personal-stories).

SKYCLARYS is used for the treatment of Friedreich ataxia (FA) in adults and children 16 years of age and older.

Not an actual patient.

SKYCLARYS[®]
(omaveloxolone) 50 mg capsules

I CANNOT TREAT MY FA PROGRESSION

Transform the way you talk about treatment with SKYCLARYS, the first and only prescription medicine for Friedreich ataxia.



It's time to fundamentally change the conversation.

Talk to your doctor or visit BiogenREACH.com to learn how you can get access to SKYCLARYS.

WHAT IS SKYCLARYS?

- SKYCLARYS is used for the treatment of Friedreich ataxia in adults and children 16 years of age and older. It is not known if SKYCLARYS is safe and effective for use in children younger than 16 years of age

IMPORTANT SAFETY INFORMATION

What are the possible side effects of SKYCLARYS?

SKYCLARYS may cause serious side effects, including:

- Increase in blood liver enzymes:** Some people taking SKYCLARYS have had an increase in the level of liver enzymes in their blood. Your healthcare provider will do liver function tests
 - before you start taking SKYCLARYS
 - every month for the first 3 months after starting your treatment with SKYCLARYS
 - during certain times as needed while taking SKYCLARYS

If your liver enzymes increase, your healthcare provider may change your dose during treatment, stop treatment for some time, or completely stop treatment with SKYCLARYS.

- Increase in a blood protein called B-Type Natriuretic Peptide (BNP).** BNP tells how well your heart is working. Your healthcare provider will check your BNP levels before your treatment with SKYCLARYS. Tell your healthcare provider if you have signs and symptoms of your heart not working well such as too much fluid in your body (fluid overload). Signs and symptoms may include:
 - sudden weight gain (3 pounds or more of weight gain in 1 day, or 5 pounds or more of weight gain in 1 week)
 - swelling in your arms, hands, legs, or feet (peripheral edema)
 - fast heartbeat (palpitations)
 - shortness of breath

If you have symptoms of fluid overload that is considered a side effect of SKYCLARYS, your healthcare provider may stop treatment with SKYCLARYS.

- Changes in cholesterol levels.** Increases in low density lipoprotein cholesterol (LDL-C) or bad cholesterol and decreases in high density lipoprotein cholesterol (HDL-C) or good cholesterol have happened during treatment with SKYCLARYS. Your healthcare provider will check your cholesterol levels before and during your treatment with SKYCLARYS.

The most common side effects of SKYCLARYS include: increased liver enzymes (ALT/AST), headache, nausea, stomach pain, tiredness, diarrhea, and muscle pain.

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

- have liver problems
- have a history of heart problems, including heart failure
- have a high level of fat in your blood (high blood cholesterol)
- are pregnant or plan to become pregnant. It is not known if SKYCLARYS will harm your unborn baby. Women who use hormonal birth control should use another form of birth control such as a non-hormonal intrauterine system or an extra non-hormonal birth control such as condoms while using SKYCLARYS and for 28 days after stopping SKYCLARYS
- are breastfeeding or plan to breastfeed. It is not known if SKYCLARYS passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby if you take SKYCLARYS

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements such as St. John's Wort.

- Taking SKYCLARYS with other medicines can cause serious side effects
- SKYCLARYS may affect the way other medicines work, and other medicines may affect how SKYCLARYS works
- Know the medicines you take. Keep a list of them to show to your healthcare provider and pharmacist when you get a new medicine

What should I avoid while taking SKYCLARYS?

- Do not drink grapefruit juice or eat grapefruit. These may change the amount of SKYCLARYS in your blood

These are not all the possible side effects of SKYCLARYS. 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.

Please see next page for brief summary and full Prescribing Information at SKYCLARYS.com.

BRIEF SUMMARY OF PATIENT INFORMATION

SKYCLARYS® (skye klar' is) (omaveloxolone) capsules, for oral use

What is SKYCLARYS?

SKYCLARYS is used for the treatment of Friedreich's ataxia in adults and children aged 16 years and older. It is not known if SKYCLARYS is safe and effective for use in children younger than 16 years of age.

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

- have liver problems.
- have a history of heart problems, including heart failure.
- have a high level of fat in your blood (high blood cholesterol).
- are pregnant or plan to become pregnant.
 - It is not known if SKYCLARYS will harm your unborn baby.
 - Women who use hormonal birth control should use another form of birth control such as a non-hormonal intrauterine system or an extra non-hormonal birth control such as condoms while using SKYCLARYS and for 28 days after stopping SKYCLARYS.
- are breastfeeding or plan to breastfeed. It is not known if SKYCLARYS passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby if you take SKYCLARYS.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements such as St. John's Wort.

Taking SKYCLARYS with other medicines can cause serious side effects.

SKYCLARYS may affect the way other medicines work, and other medicines may affect how SKYCLARYS works.

Know the medicines you take. Keep a list of them to show to your healthcare provider and pharmacist when you get a new medicine.

How should I take SKYCLARYS?

- Take SKYCLARYS exactly as your healthcare provider tells you to take it.
- Take SKYCLARYS capsules on an empty stomach at least 1 hour before eating.
- Swallow SKYCLARYS capsules whole. Do not crush or chew.
- If SKYCLARYS capsules cannot be swallowed whole, the capsules may be opened and the entire contents of both halves sprinkled onto 2 tablespoonfuls (30 mL) of applesauce.
 - Stir the mixture.
 - Swallow all the mixture of medicine and applesauce right away. Do not store the mixture of medicine and applesauce to use at a later time.
 - The contents of the SKYCLARYS capsules should not be mixed with milk or orange juice.
 - Do not administer SKYCLARYS by an enteral feeding tube.
- If you miss a dose, then you should skip the missed dose and take the next dose at the regular time the next day. Do not double your next dose or take more than the prescribed dose.

What should I avoid while taking SKYCLARYS?

Do not drink grapefruit juice or eat grapefruit. These may change the amount of SKYCLARYS in your blood.

What are the possible side effects of SKYCLARYS?

SKYCLARYS may cause serious side effects, including:

- **increase in blood liver enzymes.** Some people taking SKYCLARYS have had an increase in the level of liver enzymes in their blood. Your healthcare provider will do liver function tests
 - before you start taking SKYCLARYS
 - every month for the first 3 months after starting your treatment with SKYCLARYS
 - during certain times as needed while taking SKYCLARYS

If your liver enzymes increase, your healthcare provider may change your dose during treatment, stop treatment for some time, or completely stop treatment with SKYCLARYS.

- **increase in a blood protein called B-Type Natriuretic Peptide (BNP).** BNP tells how well your heart is working. Your healthcare provider will check your BNP levels before your treatment with SKYCLARYS.

Tell your healthcare provider if you have signs and symptoms of your heart not working well such as too much fluid in your body (fluid overload). Signs and symptoms may include:

- sudden weight gain (3 pounds or more of weight gain in 1 day, or 5 pounds or more of weight gain in 1 week)
- swelling in your arms, hands, legs, or feet (peripheral edema)
- fast heartbeat (palpitations)
- shortness of breath

If you have symptoms of fluid overload that is considered a side effect of SKYCLARYS, your healthcare provider may stop treatment with SKYCLARYS.

- **changes in cholesterol levels.** Increases in low density lipoprotein cholesterol (LDL-C) or bad cholesterol and decreases in high density lipoprotein cholesterol (HDL-C) or good cholesterol have happened during treatment with SKYCLARYS.

Your healthcare provider will check your cholesterol levels before and during your treatment with SKYCLARYS.

The most common side effects of SKYCLARYS include: increased liver enzymes (ALT/AST), headache, nausea, stomach pain, tiredness, diarrhea, and muscle pain.

These are not all the possible side effects of SKYCLARYS. 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 SKYCLARYS?

- Store SKYCLARYS at room temperature between 68°F to 77°F (20°C to 25°C)

Keep SKYCLARYS and all medicines out of the reach of children.

General information about the safe and effective use of SKYCLARYS

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

For additional information about SKYCLARYS, the full Prescribing Information, and the Patient Product Information, go to SKYCLARYS.com or call Reata Pharmaceuticals, Inc. at 1-800-314-3934.

Manufactured for Reata Pharmaceuticals, Inc., a wholly owned subsidiary of Biogen, Cambridge, MA 02142 USA

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225 Binney Street, Cambridge, MA 02142

SKYCLARYS[®]
(omaveloxolone) 50 mg
capsules

 **Biogen.**



One-on-One

MDA offers many opportunities to connect with our team

Knowing the importance of human-to-human support and guidance, MDA offers multiple ways for people in the neuromuscular disease community to connect one-on-one with MDA specialists. From answering questions about gene therapy to helping you find durable medical equipment, our specialists are ready to assist you and your family on your neuromuscular disease journey. Through MDA’s Resource Center, Connect program, Gene Therapy Support Network, and Care Centers, anyone can find the right person to guide them.

“MDA wants to be accessible and available in multiple ways based on community preference,” says Nora Capocci, Vice President of Healthcare Services at MDA. “So we offer options to email, call, or video chat. We’re here to support the community no matter where they are in their journey.”

Follow the chart below to see some of the ways you can connect with MDA, starting with the Resource Center.

Start here: The MDA Resource Center

Handling more than 10,000 inquiries a year, the Resource Center is known as the “front door to MDA.” It helps individuals, family members, and caregivers find answers to their questions and can help direct them to local resources and other MDA programs. “You can call with any question,” Nora says. “If you don’t know where to start — start with the Resource Center.” And language isn’t a barrier. The Resource Center has translators available for more than 100 languages.

Resource Center specialists are dedicated and committed MDA employees from diverse backgrounds, from social workers to caring individuals with friends or family members living with neuromuscular diseases. These specialists are knowledgeable and may answer all of your questions or refer you to another MDA program. Follow the arrows to learn about more ways to connect with MDA team members.

+ The MDA Resource Center is available Monday through Friday, 9 a.m.-5 p.m. CT. Call [833-ASK-MDA1 \(833-275-6321\)](tel:833-ASK-MDA1) or email ResourceCenter@mdaUSA.org. Emails are answered within a week.



Do you want to have a conversation about navigating life with a neuromuscular disease?

MDA Connect

MDA Connect gives community members the opportunity to have a 30-minute video call with an MDA Support Specialist. These specialists can help with education, careers, accessibility, caregiving, transportation, community engagement, general disease education, other MDA programs, and more. When booking online, you can choose an appointment time, select an available specialist, and provide any notes so the specialist comes prepared with relevant guidance.

Anyone can schedule an appointment — members of the neuromuscular disease community, their family members, caregivers, and clinicians.

+ Call the Resource Center, or schedule your video call at mda.org/connect.



> For an in-depth look into MDA Care Centers, read “A Look Inside: MDA Care Centers” at [MDAQuest.org/network-of-care](https://mdaquest.org/network-of-care).

Have more in-depth clinical questions or want a peer-to-peer connection?

Do you have questions about gene therapy?

> Looking for peer-to-peer support? Visit mda.org/CommunityGroups or join the MDA Peer Connections program by calling the Resource Center.

MDA Care Center Support

Similar to patient navigators, MDA Support Specialists connect with individuals in need of more in-depth knowledge or support. These specialists offer resources and guidance but do not provide direct medical care or advice. Anyone in the neuromuscular disease community can receive this support — those living with neuromuscular diseases, their caregivers, or family members. Support Specialists can assist with a variety of requests, such as how to navigate an MDA Care Center visit, join the MDA Peer Connections Program to connect with others with

a specific diagnosis, or participate in an MDA Community Group.

“Our specialists also help patients connect with anything they need after a Care Center visit,” Nora says. MDA Support Specialists are available for in-person meetings at MDA Care Centers or scheduled video calls.

MDA Support Specialists are invested in supporting the neuromuscular disease community and have a background in healthcare and/or lived experience with neuromuscular disease.

+ Call the Resource Center, or schedule your video call at mda.org/connect.

Gene Therapy Support Network

The award-winning Gene Therapy Support Network offers community education and support to anyone eligible to receive gene therapy or who is looking to learn more, including family members or caregivers. The network offers one-on-one video calls, phone calls, or emails to talk with a Gene Therapy Support specialist about anything gene therapy-related.

“They can help you facilitate access, navigate insurance, answer general questions, and share resources or educational materials,” Nora says. “They have answered questions from those who are currently eligible and those who are curious if their disease has a gene therapy in the pipeline.”

+ Call the Resource Center, or visit mda.org/GeneTherapySupport to schedule your video call.



PATIENT ADVOCACY AWARD

The Gene Therapy Support Network was recently honored with Phacilitate’s Advanced Therapies Patient Advocacy Award for Nonprofits, an award for nonprofit organizations that place the patient at the heart of their development and patient advocacy organizations that are dedicated to spreading awareness of the potential of cell and gene therapy (CGT). Phacilitate’s Advanced Therapies Awards recognize and celebrate progress in the advanced therapies field.

GET IN TOUCH!

For a quick connection to any of these specialists, contact the Resource Center. This team is available Monday through Friday, 9 a.m.–5 p.m. CT. Call **833-ASK-MDA1 (833-275-6321)** or email ResourceCenter@mdaUSA.org.

GENERALIZED MYASTHENIA GRAVIS

*doesn't get to
steal this walk*



IMPORTANT SAFETY INFORMATION

Do not use VYVGART if you have a serious allergy to efgartigimod alfa or any of the other ingredients in VYVGART. Do not use VYVGART HYTRULO if you have a serious allergy to efgartigimod alfa, hyaluronidase, or any of the other ingredients in VYVGART HYTRULO. VYVGART and VYVGART HYTRULO can cause serious allergic reactions and a decrease in blood pressure leading to fainting.

VYVGART and VYVGART HYTRULO may cause serious side effects,

including:

- **Infection.** VYVGART and VYVGART HYTRULO may increase the risk of infection. The most common infections for efgartigimod alfa-fcab-treated patients were urinary tract and respiratory tract infections. Signs or symptoms of an infection may include 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.
- **Allergic Reactions (hypersensitivity reactions).** VYVGART and VYVGART HYTRULO can cause allergic reactions such as rashes, swelling under the skin, and shortness of breath. Hives were also observed in patients treated with VYVGART HYTRULO. Serious allergic reactions, such as trouble breathing and decrease in blood pressure leading to fainting have been reported with efgartigimod alfa-fcab.
- **Infusion-Related Reactions.** VYVGART and VYVGART HYTRULO can cause infusion-

related reactions. The most frequent symptoms and signs reported with efgartigimod alfa-fcab were high blood pressure, chills, shivering, and chest, abdominal, and back pain.

Tell your doctor if you have signs or symptoms of an infection, allergic reaction, or infusion-related reaction. These can happen while you are receiving your VYVGART or VYVGART HYTRULO treatment or afterward. Your doctor may need to pause or stop your treatment. Contact your doctor immediately if you have signs or symptoms of a serious allergic reaction.

Before taking VYVGART or

Discover VYVGART Hytrulo for subcutaneous injection

VYVGART® Hytrulo

(efgartigimod alfa and hyaluronidase-qvfc)

Subcutaneous Injection
180 mg/mL and 2000 U/mL vial

VYVGART Hytrulo is a 30- to 90-second injection given at an infusion center, doctor's office, or at home.*†

VYVGART®

(efgartigimod alfa-fcab)

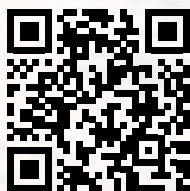
Injection for Intravenous Use
400 mg/20 mL vial

VYVGART is still available as an IV infusion and is given over 1 hour at an infusion center, neurologist's office, or at home.*†

VYVGART Hytrulo and VYVGART for IV infusion are given in treatment cycles with an individualized break between cycles (if additional cycles are needed). A treatment cycle consists of 1 treatment each week for 4 weeks (4 treatments per cycle).

*For at least 30 minutes after your subcutaneous injection or 1 hour after your IV infusion, a healthcare professional will monitor you for reactions.

†In some cases, VYVGART Hytrulo for subcutaneous injection or VYVGART for IV infusion may also be given at home by a trained nurse.



Scan the QR code or visit [VYVGART.com](https://www.vyvgart.com) to learn more

Questions? Call **1-833-VYVGART (1-833-898-4278)**

IV=intravenous. Visit [VYVGART.com/glossary](https://www.vyvgart.com/glossary) for a glossary of terms.

VYVGART HYTRULO, tell your doctor if you:

- take any medicines, including prescription and non-prescription medicines, supplements, or herbal medicines,
- have received or are scheduled to receive a vaccine (immunization), or
- have any allergies or medical conditions, including if you are pregnant or planning to become pregnant, or are breastfeeding.

What are the common side effects of VYVGART and VYVGART HYTRULO?

The most common side

effects in efgartigimod-alfa-fcab-treated patients were respiratory tract infection, headache, and urinary tract infection. Additional common side effects with VYVGART HYTRULO are injection site reactions, including rash, redness of the skin, itching sensation, bruising, pain, and hives.

These are not all the possible side effects of VYVGART and VYVGART HYTRULO. 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.

What is VYVGART® (efgartigimod alfa-fcab) for intravenous (IV) infusion and what is VYVGART® HYTRULO (efgartigimod alfa and hyaluronidase-qvfc) for subcutaneous injection?

VYVGART and VYVGART HYTRULO are both prescription medicines, each 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).

Please see the full Prescribing Information for VYVGART and VYVGART HYTRULO at [www.VYVGART.com](https://www.vyvgart.com) and talk to your doctor.

Please see the Consumer Brief Summary on the following page.

VYVGART is a registered trademark of argenx. VYVGART Hytrulo is a trademark of argenx. For U.S. audiences only. ©2024 argenx US-VYV-23-00432 V2 04/2024



Important Information about both VYVGART® (efgartigimod alfa-fcab) for intravenous (IV) infusion and VYVGART® HYTRULO (efgartigimod alfa and hyaluronidase-qvfc) for subcutaneous injection; Rx only.

The risk information provided here is not comprehensive. To learn more, talk about VYVGART and VYVGART HYTRULO with your healthcare provider. The US Food and Drug Administration (FDA)-approved product labeling can be found for VYVGART by visiting www.VYVGART.com/PI and for VYVGART HYTRULO by visiting www.VYVGARTHYTRULO.com/PI or calling 1-833-VYVGART (1-833-898-4278).

What is VYVGART and what is VYVGART HYTRULO?

VYVGART and VYVGART HYTRULO are both prescription medicines, each 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). It is not known if VYVGART or VYVGART HYTRULO are safe and effective in children under 18 years of age.

It is not known if VYVGART or VYVGART HYTRULO are safe and effective in children under 18 years of age.

Who should not use VYVGART and VYVGART HYTRULO?

Do not use VYVGART if you have a serious allergy to efgartigimod alfa or any of the other ingredients in VYVGART. Do not use VYVGART HYTRULO if you have a serious allergy to efgartigimod alfa, hyaluronidase, or any of the other ingredients in VYVGART HYTRULO. VYVGART and VYVGART HYTRULO can cause serious allergic reactions and a decrease in blood pressure leading to fainting.

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

VYVGART and VYVGART HYTRULO may cause serious side effects, including:

- **Infection.** VYVGART and VYVGART HYTRULO may increase the risk of infection. The most common infections for efgartigimod alfa-fcab-treated patients were urinary tract and respiratory tract infections. Signs or symptoms of an

infection may include 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.

- **Allergic Reactions (hypersensitivity reactions).**

VYVGART and VYVGART HYTRULO can cause allergic reactions such as rashes, swelling under the skin, and shortness of breath. Hives were also observed in patients treated with VYVGART HYTRULO. Serious allergic reactions, such as trouble breathing and decrease in blood pressure leading to fainting have been reported with efgartigimod alfa-fcab.

- **Infusion-Related Reactions.**

VYVGART and VYVGART HYTRULO can cause infusion-related reactions. The most frequent symptoms and signs reported with efgartigimod alfa-fcab were high blood pressure, chills, shivering, and chest, abdominal, and back pain.

Tell your doctor if you have signs or symptoms of an infection, allergic reaction, or infusion-related reaction. These can happen while you are receiving your VYVGART or VYVGART HYTRULO treatment or afterward. Your doctor may need to pause or stop your treatment. Contact your doctor immediately if you have signs or symptoms of a serious allergic reaction.

Before taking VYVGART or VYVGART HYTRULO, tell your doctor if you:

- have any of the conditions or symptoms listed in the section **“What is the most important information I should know about VYVGART and VYVGART HYTRULO?”**, any allergies or any medical condition
- have received or are scheduled to receive an immunization (vaccine). It is not recommended to receive a “live vaccine” if you are being treated with VYVGART or VYVGART HYTRULO.
- are pregnant or plan to become pregnant. It is not known if VYVGART or VYVGART HYTRULO may harm your unborn baby.
 - **Pregnancy Registry:** There is a pregnancy registry for pregnant women who take VYVGART or VYVGART HYTRULO. The purpose of this registry is to collect information about the health of you and your baby if you take VYVGART or

VYVGART HYTRULO during pregnancy. To learn more, call 1-855-272-6524 or visit <https://www.VYVGARTpregnancy.com>. You may also talk to your healthcare provider about how you can take part in this registry.

- are breastfeeding or plan to breastfeed. It is not known if VYVGART or VYVGART HYTRULO passes into your breast milk.

Tell your doctor 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 and VYVGART HYTRULO?

The most common side effects of efgartigimod alfa-fcab-treated patients were respiratory tract infection, headache, and urinary tract infection. Additional common side effects of VYVGART HYTRULO are injection site reactions, including rash, redness of the skin, itching sensation, bruising, pain, and hives.

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

What are the ingredients in VYVGART?

Active ingredient: efgartigimod alfa-fcab

Each 20 mL single-dose vial contains 400 mg of efgartigimod alfa-fcab at a concentration of 20 mg/mL. In addition, each mL of solution contains L-arginine hydrochloride (31.6 mg), polysorbate 80 (0.2 mg), sodium chloride (5.8 mg), sodium phosphate dibasic anhydrous (2.4 mg), sodium phosphate monobasic monohydrate (1.1 mg) and water for injection, USP, at a pH of 6.7.

What are the ingredients in VYVGART HYTRULO?

Active ingredients: efgartigimod alfa and hyaluronidase (human recombinant)

Each 5.6 mL single-dose vial contains 1,008 mg of efgartigimod alfa and 11,200 units of hyaluronidase (human recombinant). Each mL of solution contains 180 mg of efgartigimod alfa, 2,000 units of hyaluronidase (human recombinant) and histidine (1.4 mg), L-histidine hydrochloride monohydrate (2.2 mg), methionine (1.5 mg), polysorbate 20 (0.4 mg), sodium chloride (5.8 mg), sucrose (20.5 mg), and water for injection, USP, at a pH of 6.0.



New approvals

Nonsteroidal Treatment Approved for DMD



In March, the US Food and Drug Administration (FDA) approved givinostat (Duvyzat), a new treatment for children and adolescents living with Duchenne muscular dystrophy (DMD). It is the first nonsteroidal drug approved to treat patients with all genetic variants of DMD.

In a phase 3 trial, the orally administered treatment was shown to slow disease progression. The multicenter, randomized, double-blind, placebo-controlled trial tested the drug in males with DMD who can walk, ages 6 and older. Compared to placebo, participants taking Duvyzat showed less decline in multiple outcomes measures after 18 months of treatment. All participants continued their standard-of-care steroid regimen during the study.

Duvyzat is a histone deacetylase (HDAC) inhibitor, which changes gene expression in cells by altering the three-dimensional folding of DNA. Because Duvyzat works differently than other DMD treatments, it could be combined with other therapies to further improve the lives of people living with DMD. ITF Therapeutics is expected to make it available in the United States in the fall of 2024.

For more information, visit duvyzat.com. To read more about the study, visit ClinicalTrials.gov and enter NCT02851797 in the “Other terms” search box.

CLINICAL TRIAL TERMS TO KNOW

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

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

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

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

Amyotrophic lateral sclerosis (ALS)

Study Seeks Remote Participants



MDA’s NeuroMuscular Observational Research (MOVR) program and Mitsubishi Tanabe Pharma America, Inc. (MTPA) are actively seeking participants for a remote ALS research study.

The study, ALS Go-Digital, aims to see how effective it is to collect health data from participants via digital devices

and smartphones to measure ALS progression over one year. The remote data will be paired with clinical data obtained from MOVR, which collects data from a patient’s medical records during routine clinic visits at MDA Care Centers. The findings could result in more fully remote ALS clinical trials, saving participants time and resources.

The study will recruit 75 individuals from the 2,000 ALS patients currently enrolled in MOVR who contribute data during their routine care visits.

Participants will be monitored using Fitbit smartwatches, smartphone apps, and self-reported assessments.

For more information on the study, visit MDA-ALSGoDigital.com.



Amyotrophic lateral sclerosis (ALS)

Participants Needed for ALS Device Study

Researchers at PathMaker Neurosystems Inc. are seeking individuals with ALS to participate in a study to evaluate the safety and tolerability of MyoRegulator®, an investigational noninvasive neuromodulation device. This device delivers electrical stimulation to the nerves in the spinal cord and targeted limbs through electrodes placed on the skin.

The study will evaluate MyoRegulator’s ability to suppress motor neuron hyperexcitability (overactivity), potentially helping counter the motor dysfunction and decreased survival caused by the disease. Tolerability of device stimulation will be assessed by the occurrence of device-related adverse events and results from several validated questionnaires, such as the ALS Functional Rating Scale-Revised (ALSFRRS-R), Rasch Overall ALS Disability Scale

(ROADS), and the ALS Assessment Questionnaire (ALSAQ-40).

This trial is a single-center, single-arm, nonblinded clinical trial, meaning all participants at one study site will be treated with the medical device, with no placebo control. Participation will last 10 weeks and include 16 visits.

To be eligible, individuals must have a clinical diagnosis of ALS and meet other criteria. A full listing of inclusion and exclusion criteria is available from the study sponsor.

To learn more about the study and inclusion criteria or to inquire about participation, visit [PMNeuro.com](https://www.pmnuro.com) or contact the clinical site principal investigator J. Leon Morales-Quezada, MD, PhD, at jmorales-quezada@mgb.org or 617-952-6162.

Trial Shows Promising Results



NeuroSense Therapeutics Ltd. announced that its phase 2b trial (PARADIGM) with the ALS drug PrimeC has

met its primary and secondary endpoints.

PrimeC is a combination drug composed of two FDA-approved drugs: Ciprofloxacin and Celecoxib. It aims to inhibit the progression of ALS by decreasing the degeneration and inflammatory response of motor neurons in ALS.

The trial’s primary endpoints were to examine the safety and tolerability of PrimeC and evaluate ALS-related biomarkers TDP-43 and Prostaglandin2. The trial’s secondary endpoints were to evaluate the clinical efficacy outcome measures ALS Functional Rating Scale-Revised (ALSFRRS-R) and slow vital capacity (SVC), which measure function and breathing. Results show a statistically significant slowing of disease

progression with a 37.4% improvement in ALSFRRS-R scores and a 17.2% improvement in SVC scores, both favoring PrimeC versus placebo.

The multinational, randomized, double-blind, placebo-controlled clinical trial also reported positive top-line safety and efficacy data, with results comparable to placebo. Most of the trial participants who completed the six-month double-blind portion of the trial chose to receive treatment with PrimeC through a 12-month open-label extension. All participants who completed the 18-month trial treatment duration requested to continue taking PrimeC.

NeuroSense expects to report results from a primary analysis of ALS biomarkers in the first half of 2024 after analyzing participants’ plasma.

For more information on the study, visit [ClinicalTrials.gov](https://www.clinicaltrials.gov) and enter NCT05357950 in the “Other terms” search box.

Dermatomyositis



Phase 3 Study Recruiting

Researchers at Priovant Therapeutics are seeking adults with dermatomyositis to participate in a phase 3 clinical trial (VALOR) to evaluate the safety and effectiveness of the investigational therapy brepocitinib.

This phase 3, double-blinded, placebo-controlled study will test brepocitinib's ability to improve the visible skin changes and muscle weakness in dermatomyositis. Brepocitinib is designed to block the part of the immune system that causes inflammation in dermatomyositis.

For participants, the study will last up to 15 months for screening and treatment, up to 13 months for an optional open-label extension, and one additional month for follow-up.

Brepocitinib is designed to block the part of the immune system that causes inflammation in dermatomyositis.

Participants will be required to attend up to 12 study visits during screening and treatment, up to five visits if participating in the open-label extension, and one visit during the follow-up.

Participants will receive brepocitinib or a placebo control orally (by mouth). The

effects of the drug may be evaluated using various health tests, reviews, and questionnaires. To be eligible, individuals must have a diagnosis of dermatomyositis according to the 2017 EULAR/ACR Classification Criteria for Idiopathic Inflammatory Myopathies and be between ages 18 and 75, among other criteria. Travel support is available for eligible participants.

To learn more about the study or inquire about participation, visit [ValorStudy.com](https://www.valorstudy.com) or contact the patient evaluation manager Taryn Smith at PatientSupport@priosupport.com.

Becker muscular dystrophy (BMD) and Duchenne muscular dystrophy (DMD)

Seeking Data to Accelerate Research

Study coordinators at CureDuchenne Link[®] are seeking individuals living with DMD or BMD or carriers to participate in a noninterventional study. The objective is to facilitate sharing of participant-reported data and biosamples to accelerate

research for new treatments and a cure. The data collected will provide insight into the disease progression in individuals to give researchers a better understanding of the course of DMD/BMD and to identify biomarkers and therapy targets.

This study does not involve an intervention and does not exclude individuals currently participating in other studies or trials. Once every year, enrolled participants will be required to complete an online questionnaire and can choose to donate blood or urine. Participants can determine how long they participate and can pause at any time.

To be eligible, individuals must be at least 4 weeks old; have a confirmed diagnosis of DMD, BMD, or be a carrier; and meet other inclusion criteria.

For more information, visit [ClinicalTrials.gov](https://www.clinicaltrials.gov) and enter NCT04972604 in the "Other terms" search box. To learn more about the study or inquire about participation, visit [CureDuchenneLink.org](https://www.cureduchennelink.org) or email support@cureduchennelink.org.



Image: Doris/Getty Images

Duchenne muscular dystrophy (DMD)

Rare Pediatric Drug Designation



Image: FatCamera/Getty Images

HuidaGene Therapeutics' investigational gene-editing therapy, HG302, for DMD amenable to exon 51 skipping, has been granted a rare pediatric drug designation by the US Food and Drug Administration (FDA).

This status incentivizes companies to develop treatments for rare and serious or life-threatening diseases affecting people under age 18.

HG302 is expected to improve muscle function by using gene editing to “skip over” a faulty portion of the *DMD* gene called exon 51 when the genetic transcript is being made. This exon skipping allows for the production of a short but functional dystrophin protein. Mutations in the *DMD* gene cause DMD.

HG302 is intended to be a one-time, permanent therapy administered via a single intravenous (into the vein) infusion. The therapy uses an adeno-associated virus (AAV) to carry the gene-editing materials into the body. Approved therapy Exondys 51 works similarly but is an ongoing therapy given as a weekly intravenous infusion.

For more information, visit [HuidaGene.com](https://www.huidagene.com).

Gene Therapy Candidate Receives Orphan Drug Designation

The FDA granted SGT-003, a potential gene therapy for DMD, orphan drug designation. This designation incentivizes companies to develop medications that could prevent, diagnose, or treat diseases affecting fewer than 200,000 people in the United States.

The drug developer, Solid Biosciences, plans to begin a Phase 1/2 clinical trial in 2024. SGT-003 uses a proprietary capsid (protein shell) to deliver a DNA sequence encoding a shortened form of the dystrophin protein (microdystrophin).

In DMD, mutations in the *DMD* gene cause a lack of functional dystrophin, the protein that protects muscles from damage during contractions. Because of SGT-003's unique design and features, it is expected to more potently deliver the microdystrophin to cells and make muscles more resilient.

For more information on the upcoming study, visit [ClinicalTrials.gov](https://clinicaltrials.gov) and enter NCT06138639 in the “Other terms” search box.

Positive Early Data for New Exon-Skipping Drug

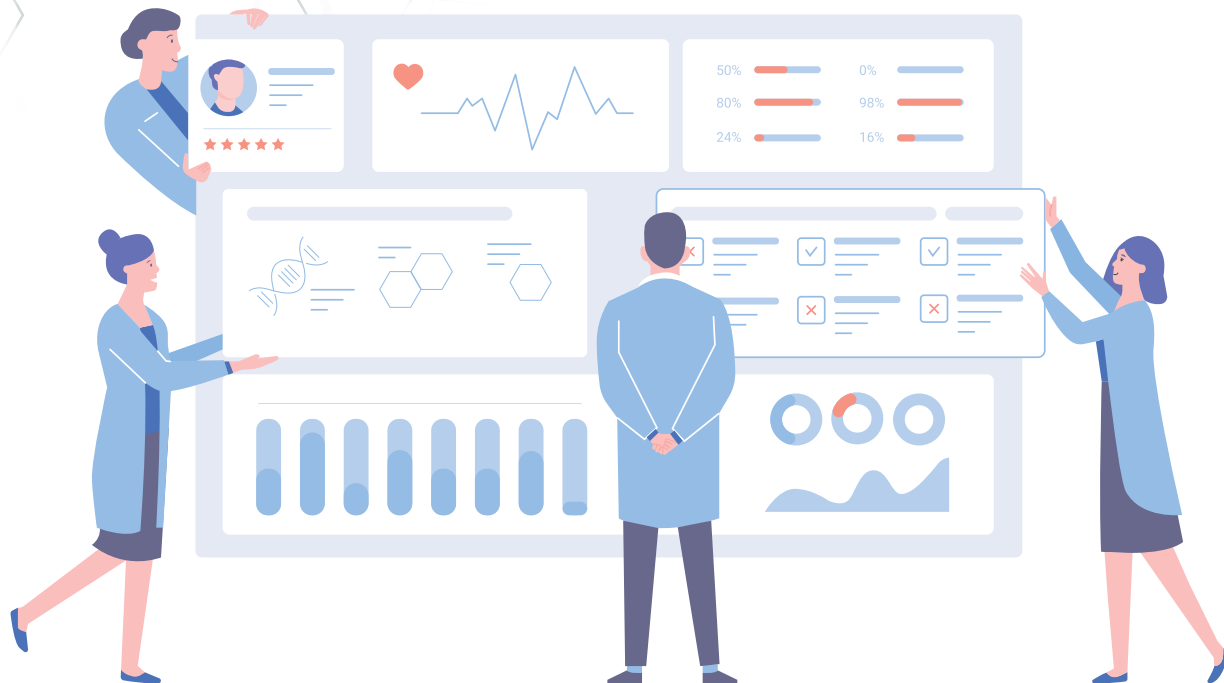


Image: Nadezhda Fedrunova/Getty Images

Dyne Therapeutics announced positive initial clinical data from its phase 1/2 DELIVER trial of DYNE-251 in patients with DMD amenable to exon 51 skipping. Exon skipping is a therapeutic strategy that works in DMD by causing cells to “skip” over faulty or misaligned sections of genetic code (exons) to produce a shortened but still functional dystrophin protein.

The initial efficacy assessment is based on six-month data from six male patients with DMD enrolled in the 5 mg/kg cohort of the randomized, placebo-controlled portion of the trial. These participants received either DYNE-251 or a placebo once every four weeks. At six months,

the results showed that the participants receiving DYNE-251 every four weeks reached levels of dystrophin expression and exon skipping that exceeded levels reported in a clinical

trial for eteplirsen, the current standard of care for DMD exon 51, at six months. In addition, eteplirsen is administered weekly.

DYNE-251 has demonstrated a favorable safety profile. Most adverse events were mild or moderate, and no serious side effects related to the experimental therapy were identified.

Enrollment is complete through the 20 mg/kg cohort of the DELIVER trial, and approximately 275 doses have been administered to date, which means they may try increasing the dose to 40 mg/kg. Dyne expects to provide its next clinical data update from the DELIVER trial in the second half of 2024.

Exon skipping is a therapeutic strategy that works in DMD by causing cells to “skip” over faulty or misaligned sections of genetic code (exons).

+RECRUITING CLINICAL TRIALS

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

For more information on the DELIVER trial, visit ClinicalTrials.gov and enter NCT05524883 in the “Other terms” search box.

Duchenne muscular dystrophy (DMD)

ELEVIDYS Study Continues After Approval

Sarepta Therapeutics announced topline results from EMBARK, the post-marketing confirmatory trial of delandistrogene moxeparvovec-rokl (ELEVIDYS), which was granted accelerated approval for boys ages 4 to 5 with DMD by the FDA in June 2023. The study did not meet its main goal, but results showed notable improvements across multiple measures of physical function in boys with DMD compared to placebo.

EMBARK is a randomized, double-blind, placebo-controlled, phase 3 clinical study of ELEVIDYS in boys with DMD ages 4 to 7 years. The primary endpoint was to evaluate how the therapy affects scores on the North Star Ambulatory Assessment (NSAA). The results showed that NSAA scores improved by an average of 2.6 points among participants given ELEVIDYS, compared with 1.9 points in participants given the placebo. The difference of 0.65 points



Image: ljubaphoto/Getty Images

between treated and placebo groups did not reach statistical significance. However, all other timed functional endpoints — including stride velocity 95th centile (SV95C) and time to ascend four steps — showed consistent benefits from the therapy.

These results have been submitted to the FDA to consider expanding the approval of ELEVIDYS, which is the first gene therapy for DMD.

For more information on the EMBARK trail, visit ClinicalTrials.gov and enter NCT05096221 in the “Other terms” search box.

Facioscapulohumeral muscular dystrophy (FSHD)

Therapy Named Orphan Drug

Epic Bio announced that the US Food and Drug Administration (FDA) granted orphan drug designation to EPI-321 for the treatment of FSHD. This designation incentivizes companies to develop medications that could prevent, diagnose, or treat diseases affecting fewer than 200,000 people in the United States.

EPI-321 is an investigational therapy that aims to address the underlying molecular mechanisms of FSHD by suppressing abnormal expression of the *DUX4* gene. The therapy is delivered to

muscle tissue in a single dose using an adeno-associated virus (AAV) vector.

Epic plans to start a phase 1/2 clinical trial of EPI-321 in the first half of 2024. The multicenter study will assess the safety, activity, and preliminary efficacy of EPI-321 in individuals with FSHD.

For more information, visit epic-bio.com.



Image: koto_feja/Getty Images

Myotonic dystrophy (DM)

Image: Irina_Strelnikova/Getty Images



Initial Data Is Promising

Dyne Therapeutics announced positive initial clinical data from its phase 1/2 ACHIEVE trial of DYNE-101 in patients with DM type 1 (DM1).

DM1 occurs when the *DMPK* gene contains an abnormally expanded section. DYNE-101 is designed to reduce toxic messenger RNA (mRNA) caused by the expanded *DMPK* gene, allowing the processing and translation of normal proteins, potentially stopping or reversing the disease progression.

The initial efficacy assessment is based on data from 32 adult DM1 patients enrolled in the trial's randomized, placebo-controlled multiple ascending dose portion, including cohorts at two different dosing levels. In each cohort, participants were randomized to receive either DYNE-101 or a placebo once every four weeks. A smaller group received two doses of DYNE-101 followed by a placebo.

Dyne reported that DYNE-101 demonstrated early dose-dependent results, including in correction of splicing, the key biomarker for DM1, as well as meaningful improvement in myotonia (difficulty relaxing muscles) at the lowest dose.

Data also indicated that DYNE-101 is decreasing *DMPK* mRNA as designed.

DYNE-101 has demonstrated a favorable safety profile. Most adverse events were mild or moderate, and no serious treatment-related adverse events have been identified.

Enrollment is complete through the 5.4 mg/kg Q8W cohort of the ACHIEVE trial, and approximately 300 doses have been administered to date, which means they may try increasing the dose to 6.8 mg/kg. Dyne expects to provide its next clinical data update from the ACHIEVE trial in the second half of 2024.

DYNE-101 is designed to reduce toxic mRNA caused by the expanded *DMPK* gene, which could lead to the potential stopping or reversing of disease progression.

For more information on the ACHIEVE trial, visit ClinicalTrials.gov and enter NCT05481879 in the "Other terms" search box.

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Understanding Titinopathies

Q&A with Bjarne Udd, MD, PhD

BY MYRNA TRAYLOR



The Neuromuscular Advocacy Collaborative gathered at the 2024 MDA Clinical & Scientific Conference in March. The titinopathy community was represented by Sarah Foye (bottom row, second from left), president and founder of Team Titin.

Titinopathies are a group of neuromuscular diseases caused by variants in the titin (*TTN*) gene. This gene carries instructions for cells to make the large muscular protein, titin, and mutations in the gene are associated with a number of neuromuscular diseases. To better understand titinopathies, we spoke with Bjarne Udd, MD, PhD, a professor at the University of Helsinki in Finland, who leads a research group that specializes in understanding and identifying the genes involved in several neuromuscular disorders, especially those associated with the *TTN* gene. The work of Dr. Udd and his team is in great demand from research teams around the world and supports continued progress toward diagnosis and future treatment of the disorders.

Can you tell us about titinopathies and the gene that causes them?

We currently know of more than 10 different muscle disease types in the group of titinopathies, which can cause disorders both severe and mild, from prenatal death to very mild, late-onset lower leg weakness. *TTN* is enormous — 50 times bigger than most

genes — which means that disease-causing variants in different parts of the gene result in very different types of disease manifestations. Some *TTN* variants may cause only cardiomyopathy (disease of the heart muscle), others cause only skeletal muscle disease, and many titinopathies cause both muscle and heart conditions. Clinical and genetic screening of family members for cardiac and muscle conditions may be indicated.

SUPPORTING TITINOPATHIES

More than 30 neuromuscular advocacy organizations joined MDA's Neuromuscular Advocacy Collaborative at the 2024 MDA Clinical & Scientific Conference in March. One was Team Titin, a titinopathy-focused nonprofit. Founded by Sarah Foye, Team Titin has a mission to support individuals, families, and their health-care providers and serve as a catalyst for academic and industry research to develop a better understanding of Titin-related disorders. To learn more, visit [TitinMyopathy.com](https://www.TeamTitin.org).





What distinguishes titinopathies from other types of neuromuscular diseases?

Many of the different titinopathies have similar clinical symptoms and signs as myopathies caused by other gene variants, such as congenital myopathy (CM), limb-girdle muscular dystrophy (LGMD), distal myopathy, Emery-Dreifuss muscular dystrophy (EDMD), and others. The main distinguishing factor is that the defect occurs in the *TTN* gene. Depending on where you have the disease-causing mutation in the gene and what type of mutation it is, there will be totally different patterns of weakness.

What causes titinopathy?

Titinopathies are inherited and can be either dominant, in which one mutation from only one parent is sufficient to cause the disease in the offspring, or recessive, in which you must inherit the gene mutation from both parents to have symptoms of the disease. An example of a dominant titinopathy is hereditary myopathy with early respiratory failure. An example of a recessive titinopathy is *TTN*-related centronuclear myopathy.

Are there any advances in the diagnosis of titinopathies?

Having the correct genetic diagnosis is currently the most important recent advance. Since the gene is so huge, it could not be sequenced before the new next-generation sequencing technology became available.



Bjarne Udd, MD, PhD

This technique uses massive parallel sequencing (a method to determine the order) either of a set of genes (gene panels), the whole exome (the protein coding parts of genes), or the whole genome (all DNA parts). Before we knew these titinopathies were caused by

TTN gene defects, the various diseases were just called by the way they presented. For instance, if you had foot drop (caused by weakness in the foot and ankle), the disease fell into a group of distal myopathies, and if you had weakness in the girdle muscles, it was called a limb-girdle disease.

TITINOPATHIES*

- + Prenatal lethal myopathy
- + Congenital myopathy with centronuclear pathology
- + Congenital arthrogyrosis
- + Congenital amyoplasia without progressive weakness
- + Early-onset limb-girdle muscular dystrophy (LGMD)
- + Childhood-onset Emery-Dreifuss muscular dystrophy (EDMD)
- + Juvenile recessive distal titinopathy (JRDT)
- + Adult-onset proximal recessive rimmed vacuolar myopathy
- + Adult-onset hereditary myopathy with early respiratory failure (HMERF)
- + Late-onset dominant distal titinopathy (also known as tibial muscular dystrophy or TMD)

**Most of these can appear with cardiomyopathy.*

Are there any promising treatments on the horizon?

Many preclinical attempts to find ways to correct or compensate for the genetic defects are underway. For one dominant form of titinopathy, hereditary myopathy with early respiratory failure (HMERF), we are trying to see if we can neutralize that chromosome with the defective *TTN* gene. People can manage very well with just one healthy protein coding *TTN* gene on one chromosome. So, our theoretical starting point is that if we silence that defective gene, the patient will have enough titin protein from the gene on the healthy chromosome. Studies are underway in fish and mouse models.

Another potential therapy we are looking at is targeting the defective *TTN* gene with a method called exon skipping (currently used in therapies for Duchenne muscular dystrophy), in which small sections of DNA are introduced to overlay the defective exon so the bad protein cannot be produced. Most genes have five to 10 exons, but *TTN* is so massive that it has 364, which makes some treatment approaches challenging. [Q](#)

Myrna Traylor is a writer for Quest Media.



Think Ahead

Take these key steps to plan for your long-term care needs

BY SUSAN JOHNSTON TAYLOR

The progressive nature of neuromuscular diseases means caregiving needs generally increase over time. Therefore, it's important for anyone who lives with a neuromuscular disease — or has a dependent who does — to plan for long-term care.

“Start planning and saving as soon as possible,” recommends Jonathan Greeson, CFP, a financial planner who lives with spinal muscular atrophy (SMA). “I tell clients we have to find a balance between enjoying today and preparing for tomorrow. Healthcare will be a large expense for all people, but even more so for people with disabilities.”

Of course, long-term care includes more than just medical needs. It may also include help with daily living tasks when a person is unable to perform those

tasks themselves, such as dressing, bathing, toileting, housekeeping, and preparing meals.

Due to the pandemic, a shortage of home care workers, and other factors, the cost of long-term care has been rising steeply. The average annual cost of a home health aide has increased 10% since 2022, and the Centers for Medicare and Medicaid Services expects these costs to continue rising.

In this environment, long-term care planning is more important than ever. Here's a look at three strategies.

“I tell clients we have to find a balance between enjoying today and preparing for tomorrow.” — Jonathan Greeson, CFP



Jonathan Greeson, CFP

1. Protect Medicaid eligibility

Medicaid covers basic medical services and long-term care for people with certain incomes and disabilities. However, many people do not qualify because of strict income and asset limits, which vary by state. In

many cases, having more than \$2,000 in countable assets (a bank account, financial investments, or property) disqualifies a single person from collecting Medicaid, while \$3,000 in countable assets disqualifies a married couple.

Many families use a special needs trust to maintain Medicaid eligibility while providing for needs that aren't covered by public benefits.

“If a parent gives a disabled child a nice inheritance, the child will most likely become ineligible for Medicaid,” explains Jonathan. “This can be avoided by putting the inheritance in a trust, so Medicaid cannot count it as a resource.”

Medicaid planning and special needs trusts are complex, so Jonathan recommends consulting a lawyer who specializes in this area. The Special Needs Alliance (SpecialNeedsAlliance.org) has a directory of attorneys who specialize in special needs planning.

2. Choose a savings vehicle

Americans with disabilities that began before age 26 are eligible to set up an Achieving a Better Life Experience (ABLE) account. (In 2026, the age requirement will change to before age 46.)

ABLE accounts are tax-advantaged savings accounts that work similarly to a 529 college savings account. If annual contributions are within \$18,000, the ABLE account does not impact eligibility for Medicaid and other public benefits. An ABLE account owner who works and does not contribute to an employer-sponsored

retirement account may make additional contributions up to an amount that varies by state.

“ABLE accounts are a huge step in financial freedom for people with disabilities, but they still have limits,” Jonathan says. “They are great for saving for a house, car, school, etc. However, for a larger expense like 24-hour care, the limits would be a hindrance. Paying for care alone could empty the entire account in one year.”

Other savings vehicles do not have these contribution limits, but they could impact eligibility for benefits. Use the directory from Special Needs Answers (SpecialNeedsAnswers.com) to find a planner who can provide guidance on savings vehicles.

3. Think about long-term care insurance early

Long-term care insurance policies usually work like auto or home insurance: You pay premiums as long as the policy is in effect and make claims if you ever need home health care, respite care, a residential care facility, or other covered services. Many companies that offer these policies will not medically underwrite someone who has been diagnosed with a serious condition or will charge them higher premiums.

Individuals who know an inherited neuromuscular disease runs in their family may consider purchasing a plan before they have genetic testing or exhibit any symptoms.

Some people may be eligible for an employer-sponsored group policy that does not require medical underwriting. However, if the employee leaves that job, they'll need to pay the premiums on their own or lose the coverage.

An insurance agent or broker who focuses on long-term care planning may be able to help. Find insurance departments and local agents through the National Association of Insurance Commissioners state directory (content.naic.org/state-insurance-departments).

Look forward

Whatever option you choose, the important thing is having a plan for future care needs. With the right support, you'll be prepared as your plans and needs evolve. [Q](#)

Susan Johnston Taylor writes about health and general interest topics.



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AGAMREE is FDA approved for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older³

SELECT IMPORTANT SAFETY INFORMATION

Warnings & Precautions

- **Alterations in Endocrine Function:** Monitor patients receiving AGAMREE for Cushing's syndrome, hyperglycemia, and adrenal insufficiency after AGAMREE withdrawal. In addition, patients with hypopituitarism, primary adrenal insufficiency or congenital adrenal hyperplasia, altered thyroid function, or pheochromocytoma may be at increased risk for adverse endocrine events. Acute adrenal insufficiency can occur if AGAMREE is withdrawn abruptly, and could be fatal.
- **Immunosuppression and Increased Risk of Infection:** Use of corticosteroids, including AGAMREE, increases the risk of new infection, exacerbation of existing infections, dissemination, and reactivation or exacerbation of latent infection and may mask some signs of infection; these infections can be severe, and at times fatal.
- **Alterations in Cardiovascular/Renal Function:** Monitor for elevated blood pressure and monitor sodium and potassium levels in patients chronically treated with AGAMREE.
- **Gastrointestinal Perforation:** Use of corticosteroids increases the risk of gastrointestinal perforation in patients with certain gastrointestinal disorders, such as active or latent peptic ulcers, diverticulitis, fresh intestinal anastomoses, and non-specific ulcerative colitis. Signs and symptoms may be masked.
- **Behavioral and Mood Disturbances:** Potentially severe psychiatric adverse reactions may occur with systemic corticosteroids, including AGAMREE, and may include hypomanic or manic symptoms (eg, euphoria, insomnia, mood swings) during treatment and depressive episodes after discontinuation of treatment.
- **Effects on Bones:** Prolonged use of corticosteroids, such as AGAMREE, can lead to osteoporosis, which can predispose patients to vertebral and long bone fractures. Monitor bone mineral density in patients on long-term treatment with AGAMREE.
- **Ophthalmic Effects:** The use of corticosteroids, such as AGAMREE, may increase the risk of cataracts, ocular infections, and glaucoma. Monitor intraocular pressure if treatment with AGAMREE is continued for more than 6 weeks.
- **Vaccination:** Do not administer live-attenuated or live vaccines to patients receiving AGAMREE. Administer live-attenuated or live vaccines at least 4 to 6 weeks prior to starting AGAMREE.

Please see Brief Summary of full Prescribing Information on the next page.

References

1. Liu X, et al. *Proc Natl Acad Sci USA*. 2020;117(39):24285-24293. 2. Heier CR, et al. *EMBO Mol Med*. 2013;5(10):1569-1585. 3. AGAMREE (vamorolone) Oral Suspension [prescribing information]. Catalyst Pharmaceuticals, Inc.; 2024. 4. Guglieri M, et al. *JAMA Neurol*. 2022;79(10):1005-1014.



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AGAMREE® (vamorolone) oral suspension

BRIEF SUMMARY – See Full Prescribing Information at AGAMREEhcp.com

Initial U.S. Approval: 2023

INDICATIONS AND USAGE

AGAMREE is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

DOSE AND ADMINISTRATION

Dosing Information

The recommended dosage of AGAMREE is 6 mg/kg taken orally once daily preferably with a meal, up to a maximum daily dosage of 300 mg for patients weighing more than 50 kg.

Some patients may respond to a dose of 2 mg/kg daily. Doses may be titrated down to 2 mg/kg/day as needed, based on individual tolerability.

Discontinuation

Dosage of AGAMREE must be decreased gradually if the drug has been administered for more than one week.

CONTRAINDICATIONS

AGAMREE is contraindicated in patients with known hypersensitivity to vamorolone or to any of the inactive ingredients of AGAMREE. Instances of hypersensitivity, including anaphylaxis, have occurred in patients receiving corticosteroid therapy.

WARNINGS AND PRECAUTIONS

Alterations in Endocrine Function

Corticosteroids, such as AGAMREE, can cause serious and life-threatening alterations in endocrine function, especially with chronic use. Monitor patients receiving AGAMREE for Cushing's syndrome, hyperglycemia, and adrenal insufficiency after AGAMREE withdrawal. In addition, patients with hypopituitarism, primary adrenal insufficiency or congenital adrenal hyperplasia, altered thyroid function, or pheochromocytoma may be at increased risk for adverse endocrine events.

Acute adrenal insufficiency can occur if AGAMREE is withdrawn abruptly, and could be fatal. The risk of adrenal insufficiency is reduced by gradually tapering the dose when withdrawing treatment. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.

Immunosuppression and Increased Risk of Infection

Corticosteroids, including AGAMREE, suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic pathogens. Corticosteroids can reduce resistance to new infections, exacerbate existing infections, increase the risk of disseminated infections, increase the risk of reactivation or exacerbation of latent infections, and mask some signs of infection. Corticosteroid-associated infections can be mild but can be severe, and at times fatal.

The rate of infectious complications increases with increasing corticosteroid dosages. Monitor for the development of infection and consider AGAMREE withdrawal or dosage reduction as needed.

Tuberculosis

If AGAMREE is used to treat a condition in patients with latent tuberculosis or tuberculin reactivity, reactivation of tuberculosis may occur. Closely monitor such patients for reactivation. During prolonged AGAMREE therapy, patients with latent tuberculosis or tuberculin reactivity should receive chemoprophylaxis.

Varicella Zoster and Measles Viral Infections

Varicella and measles can have a serious or even fatal course in non-immune patients taking corticosteroids, including AGAMREE. In corticosteroid-treated patients who have not had these diseases or are non-immune, particular care should be taken to avoid exposure to varicella and measles.

- If an AGAMREE-treated patient is exposed to varicella, prophylaxis with varicella zoster immunoglobulin may be indicated. If varicella develops, treatment with antiviral agents may be considered.
• If an AGAMREE-treated patient is exposed to measles, prophylaxis with immunoglobulin may be indicated.

Hepatitis B Virus Reactivation

Hepatitis B virus reactivation can occur in patients who are hepatitis B carriers treated with immunosuppressive dosages of corticosteroids, including AGAMREE. Reactivation can also occur infrequently in corticosteroid-treated patients who appear to have resolved hepatitis B infection.

Screen patients for hepatitis B infection before initiating immunosuppressive (e.g., prolonged) treatment with AGAMREE. For patients who show evidence of hepatitis B infection, recommend consultation with physicians with

expertise in managing hepatitis B regarding monitoring and consideration for hepatitis B antiviral therapy.

Fungal Infections

Corticosteroids, including AGAMREE, may exacerbate systemic fungal infections; therefore, avoid AGAMREE use in the presence of such infections unless AGAMREE is needed to control drug reactions. For patients on chronic AGAMREE therapy who develop systemic fungal infections, AGAMREE withdrawal or dosage reduction is recommended.

Amebiasis

Corticosteroids, including AGAMREE, may activate latent amebiasis. Therefore, it is recommended that latent amebiasis or active amebiasis be ruled out before initiating AGAMREE in any patients who have spent time in the tropics or patients with unexplained diarrhea.

Strongyloides Infestation

Corticosteroids, including AGAMREE, should be used with great care in patients with known or suspected Strongyloides (threadworm) infestation. In such patients, corticosteroid-induced immunosuppression may lead to Strongyloides hyperinfection and dissemination with widespread larval migration, often accompanied by severe enterocolitis and potentially fatal gram-negative septicemia.

Cerebral Malaria

Avoid corticosteroids, including AGAMREE, in patients with cerebral malaria.

Alterations in Cardiovascular/Renal Function

Corticosteroids, including AGAMREE, can cause elevation of blood pressure, salt and water retention, and increased excretion of potassium and calcium.

Monitor blood pressure and assess for signs and symptoms of volume overload. Monitor serum potassium levels.

AGAMREE should be used with caution in patients with congestive heart failure, hypertension, or renal insufficiency. Literature reports suggest an association between use of corticosteroids and left free wall rupture after a recent myocardial infarction; therefore, therapy with AGAMREE should be used with great caution in these patients.

Gastrointestinal Perforation

There is an increased risk of gastrointestinal perforation with the use of corticosteroids in patients with certain gastrointestinal disorders, such as active or latent peptic ulcers, diverticulitis, fresh intestinal anastomoses, and non-specific ulcerative colitis. Signs of gastrointestinal perforation, such as peritoneal irritation, may be masked in patients receiving corticosteroids.

Avoid AGAMREE if there is a probability of impending perforation, abscess, or other pyogenic infections; diverticulitis; fresh intestinal anastomoses; or active or latent peptic ulcer.

Behavioral and Mood Disturbances

Potentially severe psychiatric adverse reactions may occur with systemic corticosteroids, including AGAMREE. Symptoms typically emerge within a few days or weeks of starting treatment and may be dose-related. These reactions may improve after either dose reduction or withdrawal, although pharmacologic treatment may be necessary.

Inform patients or caregivers of the potential for behavioral and mood changes and encourage them to seek medical attention if psychiatric symptoms develop, especially if depressed mood or suicidal ideation is suspected.

Effects on Bones

Decreased Bone Mineral Density

Corticosteroids, such as AGAMREE, decrease bone formation and increase bone resorption both through their effect on calcium regulation (i.e., decreasing absorption and increasing excretion) and inhibition of osteoblast function. This, together with a decrease in the protein matrix of the bone secondary to an increase in protein catabolism and reduced sex hormone production, may lead to inhibition of bone growth in pediatric patients and the development of bone loss at any age. Bone loss can predispose patients to vertebral and long bone fractures.

Consider a patient's risk of osteoporosis before initiating corticosteroid therapy. Monitor bone mineral density in patients on long-term treatment with AGAMREE.

Avascular Necrosis

Corticosteroids may cause avascular necrosis.

Ophthalmic Effects

The use of corticosteroids, such as AGAMREE, may produce posterior subcapsular cataracts. Corticosteroids may also cause glaucoma with possible damage to the optic nerves, and may increase the risk of secondary ocular infections caused by bacteria, fungi, or viruses. Corticosteroids are not recommended for patients with active ocular herpes simplex. Intraocular pressure may become elevated in some patients taking corticosteroids. If treatment with AGAMREE is continued for more than 6 weeks, monitor intraocular pressure.

Immunizations

Administer all immunizations according to immunization guidelines prior to starting AGAMREE. Administer live-attenuated or live vaccines at least 4 to 6 weeks prior to starting AGAMREE. Patients on AGAMREE may receive

concurrent vaccinations, except for live-attenuated or live vaccines.

Effects on Growth and Development

Long-term use of corticosteroids, including AGAMREE, can have negative effects on growth and development in children.

Myopathy

Patients receiving corticosteroids and concomitant therapy with neuromuscular blocking agents (e.g., pancuronium) or patients with disorders of neuromuscular transmission (e.g., myasthenia gravis) may be at increased risk of developing acute myopathy. This acute myopathy is generalized, may involve ocular and respiratory muscles, and may result in quadriplegia. Clinical improvement or recovery after stopping corticosteroids may require weeks to years.

Kaposi's Sarcoma

Kaposi's sarcoma has been reported to occur in patients receiving corticosteroid therapy, most often for chronic conditions. Discontinuation of corticosteroids may result in clinical improvement of Kaposi's sarcoma.

Thromboembolic Events

Observational studies have shown an increased risk of thromboembolism (including venous thromboembolism) particularly with higher cumulative doses of corticosteroids. It is unclear if risk differs by daily dose or duration of use. Use AGAMREE with caution in patients who have or may be predisposed to thromboembolic disorders.

Anaphylaxis

Rare instances of anaphylaxis have occurred in patients receiving corticosteroid therapy.

ADVERSE REACTIONS

The following serious adverse reactions are discussed in more detail in other sections:

- Alterations in Endocrine Function
• Immunosuppression and Increased Risk of Infection
• Alterations in Cardiovascular/Renal Function
• Gastrointestinal Perforation
• Behavioral and Mood Disturbances
• Effects on Bones
• Ophthalmic Effects
• Immunizations
• Effects on Growth and Development
• Myopathy
• Kaposi's Sarcoma
• Thromboembolic Events
• Anaphylaxis

Clinical Trials Experience

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

Common Adverse Reactions in Clinical Studies

Table 1 lists the adverse reactions that occurred in ≥5% of the patients treated with AGAMREE 6 mg/kg/day (N=28) or AGAMREE 2 mg/kg/day (N=30) and that occurred more frequently than in the patients who received placebo (N=29) in Study 1, which was 24 weeks and included patients with DMD between the ages of 4 and 7 years.

Table 1: Adverse Reactions in Patients with DMD that Occurred in ≥5% of Patients Treated with AGAMREE and More Frequently than in Patients Who Received Placebo During 24 Weeks (Study 1)

Table with 4 columns: Adverse Reaction, AGAMREE 2 mg/kg/d (N=30)%, AGAMREE 6 mg/kg/d (N=28)%, Placebo (n=29)%. Rows include Cushingoid features, Psychiatric disorders, Vomiting, Weight increased, Vitamin D deficiency, Cough, Headache, Diarrhea, Increased appetite, Rhinitis.

*Includes the following adverse reactions that occurred more frequently in the AGAMREE group than in placebo: abnormal behavior, aggression, agitation, anxiety, irritability, mood altered, sleep disorder, and stereotypy.

In a separate open-label safety study of pediatric patients aged 2 to less than 4 years (n=16) and pediatric patients aged 7 to less than 18 years (n=16) with DMD, adverse reactions were similar to those seen in the Study 1 pediatric patients.

DRUG INTERACTIONS

Effect of Other Drugs on Vamorolone

Co-administration of AGAMREE with itraconazole, a strong CYP3A4 inhibitor, increases vamorolone exposure. Reduce the dosage of AGAMREE in patients when strong CYP3A4 inhibitors are used concomitantly. No dosage adjustments are required when AGAMREE is concomitantly administered with moderate or weak CYP3A4 inhibitors.

USE IN SPECIFIC POPULATIONS

Pregnancy

Risk Summary

AGAMREE is indicated for use for the treatment of DMD, which is a disease of young male patients. However, corticosteroids in general should be used during pregnancy only if the potential benefit justifies the potential risk to the fetus. Infants born to mothers who have received substantial doses of corticosteroids during pregnancy should be carefully observed for signs of hypoadrenalism. There are no data on the use of AGAMREE during pregnancy.

Animal reproduction studies have not been conducted with AGAMREE.

Lactation

Risk Summary

There are no data on the presence of vamorolone in human milk or the effects on milk production.

AGAMREE is indicated for use for the treatment of DMD, which is a disease of young male patients. However, systemically administered corticosteroids appear in human milk and could suppress growth, interfere with endogenous corticosteroid production, or cause other untoward effects. The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need and any potential adverse effects on the breastfed infant.

Pediatric Use

The safety and effectiveness of AGAMREE for the treatment of DMD have been established in patients 2 years of age and older. Use of AGAMREE in pediatric patients is supported by a multicenter, randomized, double-blind, placebo- and active-controlled study in 121 males 4 to less than 7 years of age. Use of AGAMREE in patients 2 years to less than 4 years of age and 7 to less than 18 years of age is supported by findings of efficacy and safety in patients 4 to less than 7 years of age with DMD, and by pharmacokinetic and safety data from patients 2 to 4 years of age and 7 to less than 18 years of age.

The safety and effectiveness in pediatric patients below the age of 2 years have not been established.

Geriatric Use

DMD is largely a disease of children and young adults; therefore, there is no geriatric experience with AGAMREE.

Hepatic Impairment

Moderate hepatic impairment increases vamorolone exposure. Reduce the AGAMREE dosage in patients with mild to moderate hepatic impairment. There is no clinical experience with AGAMREE in patients with severe hepatic impairment, and a dosing recommendation cannot be provided for patients with severe hepatic impairment.

CLINICAL PHARMACOLOGY

Mechanism of Action

Vamorolone is a corticosteroid that acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which vamorolone exerts its effect in patients with DMD is unknown.

Pharmacodynamics

Vamorolone produced a dose-dependent decrease in morning cortisol levels in the clinical studies. Treatment with corticosteroids is associated with a suppression of endogenous cortisol concentrations and an impairment of the hypothalamus-pituitary-adrenal (HPA) axis function. A dose-dependent increase in leukocyte counts and lymphocyte counts was observed in clinical studies with vamorolone.

Cardiac Electrophysiology

Vamorolone does not cause a mean increase in the QTc interval >20 milliseconds (ms) at 1.6 times the approved recommended dose.

See full Prescribing Information available at AGAMREEhcp.com.



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COMM GOOP

Building with universal design makes life better for everyone — including people with disabilities

BY MATT ALDERTON

The Mizzou Arena in Columbia, Missouri, is home court for the University of Missouri (MU) men’s and women’s basketball teams and serves as an entertainment venue in the off-season. The architects who designed the arena, which opened in 2004, knew that the entrance was important — it would welcome throngs of fans of all ages. They initially designed a showcase entrance plaza with flights of stairs with intermediate landings across the arena’s

width. They ensured it was accessible by placing ramps at the far left and right sides.

Fortunately, Richard Sternadori, Senior Program Coordinator, Researcher, and Director of the Accessible Design Accreditation Initiative for the University of Missouri (MU) Department of Architectural Studies Great Plains ADA Center, was a consultant on the project.

“When the initial drawings came to my office, we suggested a gentle slope from the curb all the way to the

ON

multiple entrances,” Richard says. The result is a large, open concrete promenade that eases the flow of crowds toward the entrance doors and allows families and groups of friends to stay together, whether they are on foot, in wheelchairs, using walkers, or pushing strollers. The effect is more welcoming and no less grand.

This is an example of universal design, which the Center for Inclusive Design and Environmental Access (IDEA Center) at the University of Buffalo’s School of Architecture and Planning defines as



BUILDING BETTER

Listen to a conversation with Dominic Marinelli, a certified Accessibility Specialist/Plans Examiner, about making buildings more accessible at [MDAQuest.org/podcast/architecture](https://mdaquest.org/podcast/architecture).

“a design process that enables and empowers a diverse population by improving human performance, health and wellness, and social participation.”

Increasingly, architects who design everything from office buildings and outlet malls to retail stores and stadiums are recognizing the merits of designing buildings and spaces that are equitable, accessible, and enjoyable for everyone, regardless of age, background, or ability.

What is universal design?

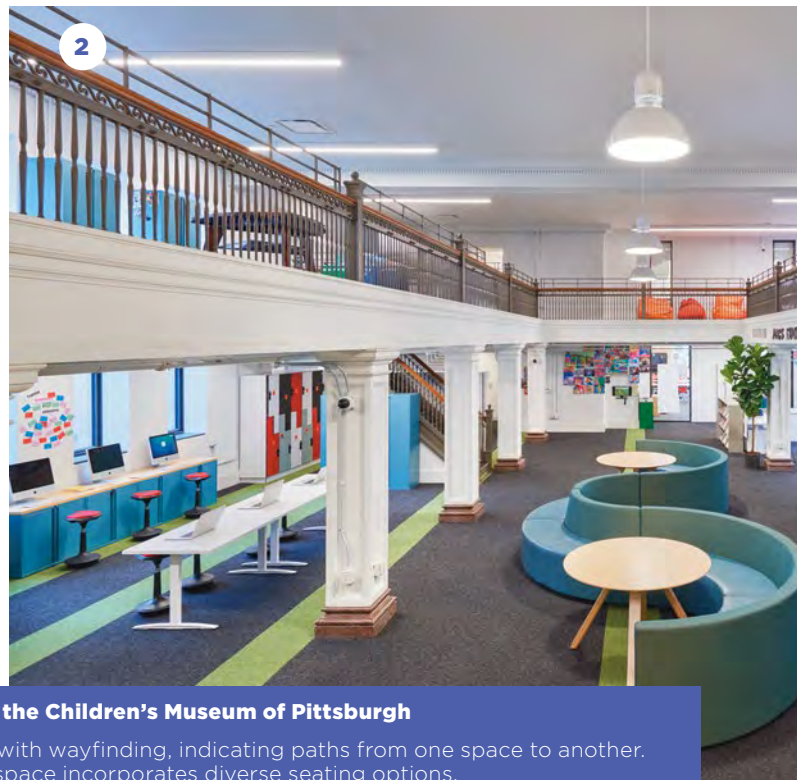
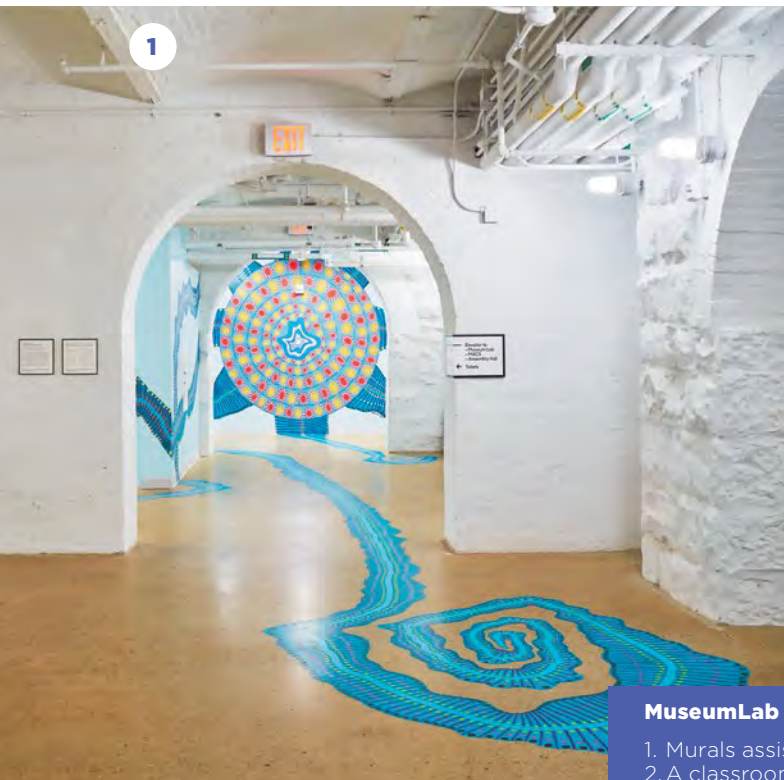
Architect Jonathan White, Director of Design Consulting at the IDEA Center, describes it in even simpler terms: “Universal design makes life easier, healthier, and friendlier for everyone,” he says.



The Mizzou Arena has a large, sloping promenade leading to the entrance doors, rather than stairs.

“It’s not just about accommodating people with disabilities, although that’s certainly a prerequisite. It’s about making the environment usable for everyone, whether that’s parents with small children, older adults, people from different socioeconomic backgrounds, or people from different cultures — whatever the case may be.”

When architect Ron Mace first coined the term “universal design” in 1985, he was reacting to building codes and regulations that he saw as encumbering



MuseumLab at the Children’s Museum of Pittsburgh
 1. Murals assist with wayfinding, indicating paths from one space to another.
 2. A classroom space incorporates diverse seating options.
 3. In the Make Lab, workstations are spaced far apart to allow easy movement around them.



accessibility rather than enhancing it. For example, the Americans with Disabilities Act (ADA) requires developers to design buildings and public spaces in ways that accommodate people with disabilities. In practice, however, it sometimes constrains accessibility by putting an unintended ceiling on it.

“Often, the regulatory approach is perfunctory. People want to know, ‘What’s the minimum I can do? What can I get away with?’” Richard says. “Universal design is better in that respect because it doesn’t take that minimalist approach to design. It takes a broader approach.”

Because that broader approach isn’t focused on ADA compliance, Richard stresses that universal design is not a substitute for accessible design. Rather, the two are complementary. “If it’s done right, universal design serves everyone. That’s the intent,” he says. “Universal design and accessible design should work hand-in-hand.”

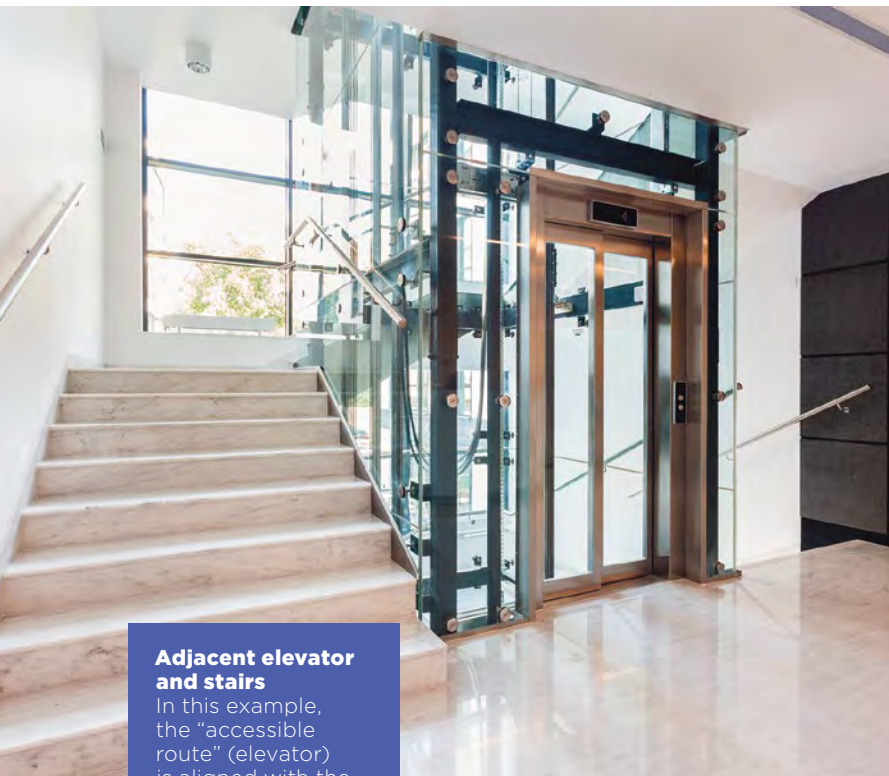
When they do, the results can be transformational for individuals as well as communities, according to architect Erin Peavey, Vice President and Health and Well-being Design Leader at Dallas-based architecture firm HKS. “Social connection is critical to all of life thriving,” says Erin, who points out

UNIVERSAL DESIGN GOALS

The IDEA Center operates according to eight goals for universal design:

1. **Body fit:** Accommodating a wide range of body sizes and abilities
2. **Comfort:** Keeping demands within desirable limits of body function and perception
3. **Awareness:** Ensuring that critical information for use is easily perceived
4. **Understanding:** Making methods of operation and use intuitive, clear, and unambiguous
5. **Wellness:** Contributing to health promotion, avoidance of disease, and protection from hazards
6. **Social integration:** Treating all groups with dignity and respect.
7. **Personalization:** Incorporating opportunities for choice and the expression of individual preferences
8. **Cultural appropriateness:** Respecting and reinforcing cultural values and the social and environmental contexts of any design project





Adjacent elevator and stairs

In this example, the “accessible route” (elevator) is aligned with the “circulation path” (stairs). Keeping these paths adjacent allows people with different abilities, such as wheelchair users and their colleagues or caregivers, to stay together as they navigate a building.

that scientists have linked social connectedness to increased health, happiness, and longevity. In fact, the more isolated someone is, the more likely they are to experience heart disease and stroke, type 2 diabe-

tes, depression and anxiety, addiction, dementia, and early death, the Centers for Disease Control and Prevention (CDC) reports. “And yet, the built environments we’re creating regularly make it so that people are left out with no real option to be included,” Erin says.

Putting it into practice

What does universal design mean in real life? Consider something as simple as a sidewalk with a ramp to street level. “Curb cuts are something that for the longest time we didn’t have, but they are helpful for everyone,” explains Erin. They make navigating sidewalks easier not only for wheelchair users but also for seniors with walkers, injured people on crutches, parents pushing strollers, and travelers pulling luggage.

Applying universal design to a pedestrian crosswalk might go several steps further than curb cuts and add visible crossing warnings and protective posts. “Another approach is to raise the crossing to the sidewalk level — make the cars navigate the hill instead of the people,” Jonathan says. “This also helps drain water away from the crossing instead of allowing a puddle to accumulate.”

Or consider a playground. “I have a 5-year-old daughter, and when we go to parks there are a lot of things that are not inclusive,” Erin says. Many playgrounds have a loose ground cover, such as wood chips, that is difficult to navigate with limited mobility. A better choice is poured-in-place rubber, which makes playgrounds accessible to all children, as well as their parents and grandparents.

“If I were to design a playground, I might make sure there’s a fence with an entrance that can be monitored, and a shelter with seating and bathrooms, so adults can stay

there comfortably all day and watch their kids,” adds Jonathan, who says universal design is as much about comfort and safety as it is about accessibility. (Learn more about accessible playgrounds and how to find them at [MDAQuest.org/accessible-playgrounds](https://mdaquest.org/accessible-playgrounds).)

Jonathan’s favorite example of universal design is the MuseumLab at the Children’s Museum of Pittsburgh, a hands-on space for kids to experiment with art and technology. Opened in 2019 in a renovated historic building, it features spacious elevators and bathrooms with room for wheelchairs to turn around, large gaps between workstations to accommodate kids with mobility devices, lobbies and hallways with generous circulation paths for family groups and strollers, and easy-to-see directional signage at every turn.

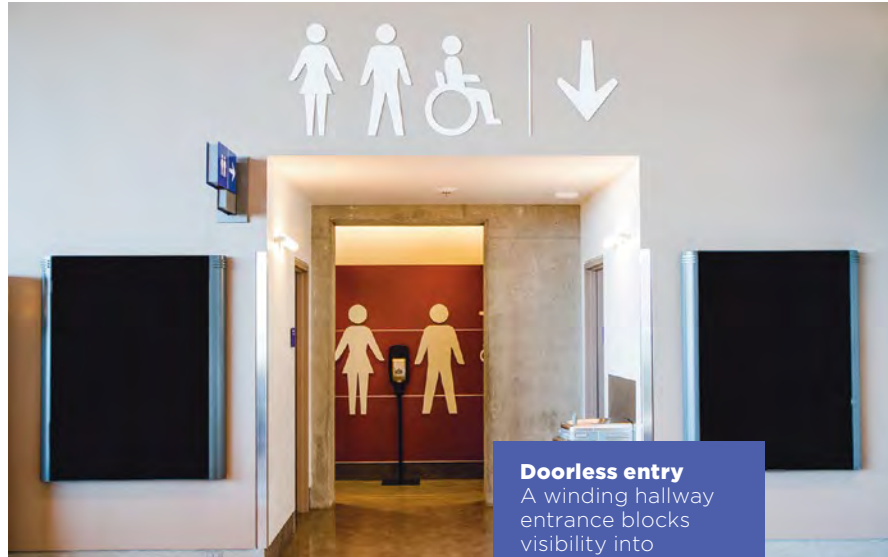
Champion change

For a variety of reasons — budget constraints, lack of awareness among architects and developers, and perceived complexity, just to name a few — universal design is not yet practiced universally. Fortunately, there are things you can do to champion universal design in your community.

Image: Katarzyna Blalawicz/Getty Images

It starts with future architects, according to Richard, who recommends supporting the Accessible Design Accreditation Initiative, which he directs. The initiative's goal is to update architecture and interior design curriculums at colleges and universities to include the latest accessible design standards. Supporters can add their names to a list of co-sponsors online, or they can express their support directly to any of the four national accreditation organizations that assess and validate design programs at universities. The MU Great Plains ADA Center offers more information at GPADACenter.org/accessible-design-accreditation-initiative.

“If we can get schools to take this seriously — to make sure students graduate understanding



Doorless entry

A winding hallway entrance blocks visibility into these restrooms. This doorless design increases accessibility, reduces the spread of disease by eliminating high-touch surfaces, and reduces injuries, construction costs, and maintenance issues associated with doors.



3 WAYS TO FIND ACCESSIBLE SPACES

Use these tools to find accessible spaces in your community or when you visit a new place:

Google Maps: The “Accessible places” feature is available on a phone, tablet, or computer. In the app, tap your profile picture or initials, then tap “Settings” and “Accessibility.” From there, turn on “Accessible places.” With this feature on, wheelchair icons on the map indicate wheelchair-accessible businesses and other places of interest. maps.google.com

AccessNow: This app connects you to a global community of citizen mappers who identify accessible places — like hotels, restaurants, restrooms, and parks — based on firsthand experience. You can search for places with specific accessibility features and contribute to the AccessNow community by ranking and reviewing places you visit. AccessNow.com

Ahoi: To use this app, create a profile with your specific accessibility needs, such as entry ramps or automatic doors. Then, you can find destinations, from retail stores to doctor's offices, ranked by how well they meet your needs, with community-driven reviews that include photos. The app also uses wayfinding technology and sidewalk health data to help you navigate safely to your destination.

AhoiMate.com

how important accessible design is to their clients, to the firms that hire them, to people with disabilities, and to society in general — then we've changed the world and America,” Richard says.

At the local level, find out if your town or city government has an accessibility board, council, or commission, suggests Erin, who says citizens can often join or advise such groups to raise awareness about universal design tenets and opportunities. Also, look for meetings about proposed building projects and open comment periods when developers solicit public feedback.

Jonathan adds that it can be especially persuasive when you make a business case for universal design. “There's a supermarket chain in Germany called Kaiser's. They implemented a bunch of universal design strategies throughout their stores and saw a 30% increase in revenues,” he notes.

The key for everyone — designers, developers, policymakers, and citizens — is education. “If you educate yourself and use your knowledge to educate others, that's what will create change in your community,” Jonathan says. [🔗](#)

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



Leah Zelaya enjoys hand cycling.

REDEFINING

Leah Zelaya, 16, an MDA National Ambassador, is a dancer and athlete. When she was 6, she started taking classes with Dancing Dreams, an organization that offers adaptive dance for children with disabilities in the New York area. In her younger years, a teenage helper acted as her spotter as she practiced. Now, she dances using bilateral leg braces or a wheelchair equipped with special features for dance.

“I was brought up playing sports my whole life,” says Leah, who also skis, hand cycles, and plays pickleball. “My father always inspired me with his passion for sports and how determined he is when he does them.”

Image: Dmytro Aksonov/Getty Images

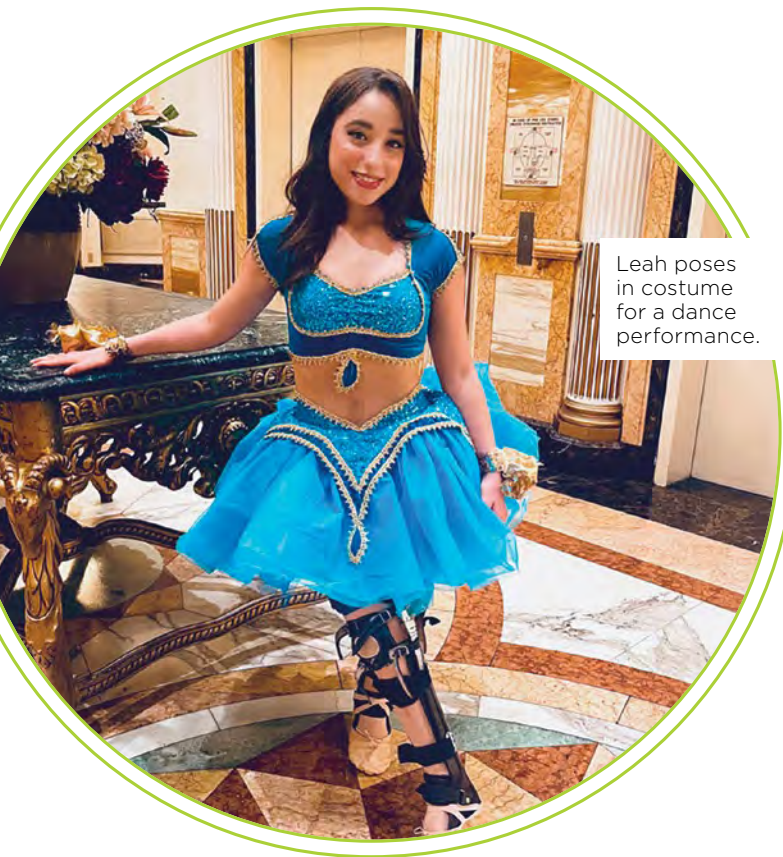
A guide to getting involved in adaptive sports

BY CHARMAINE DYMOND

BEYOND LIMITS



Jaime Zelaya plays wheelchair softball.



Leah poses in costume for a dance performance.

Leah and her father, Jaime, both live with scapulo-peroneal spinal muscular atrophy (SPSMA). Though they share the same disease, Jaime is quick to point out that they don't share a love for all the same sports. "The sports I like she doesn't like, and the sports she likes, I won't even dare to play," he says. "I'm holding my heart and trying not to die of a heart attack when I see her coming down a mountain going almost 25 miles per hour."

His admiration for his daughter is evident. While she credits him for inspiring her love of sports, he credits her with showing him that he could keep playing the sports he loves even after running and then walking became challenging. Leah and her mother, Bevi, connected Jaime to the Wheelchair Sports Federation (WheelchairSportsFederation.org), a national nonprofit that provides adaptive sports opportunities for youth and adults. Now he plays wheelchair basketball, softball, rugby, and tennis.

"I'd never played before in a wheelchair, but I got hooked," says Jaime. "It's given me a fountain of youth in my heart and my soul."

A sport for every ability

Almost every sport can be modified to be adaptive, enabling people with disabilities to participate at a recreational or competitive level. Modifications include specially designed equipment, such as maneuverable wheelchairs for basketball or lighter shot-puts for track and field.

To ski, Leah sits in a bi-ski, similar to a sled on two skis. A sport might also be adapted through changes to the rules or field of play. For example, sitting volleyball uses a low net, and players sit on the floor, while power soccer is played in a gymnasium with players in power wheelchairs equipped with a foot guard they use to pass and shoot a soccer ball.

Competitive adaptive sports programs use classification systems that group athletes based on their physical abilities and challenges. "Adaptive sports are an attempt at fairness," says Clayton Frech, founder and CEO of Angel City Sports (AngelCitySports.org), a Los Angeles-based nonprofit providing adaptive sports for people of all ages with physical disabilities. "When my son, Ezra, was younger, he would measure himself against boys his age in the track-and-field event he was doing with his disability, which is an above-knee amputation. So, he might run next to a kid with a below-knee amputation, but their times would not be compared. This keeps young kids engaged in sport, and some may end up competing nationally or even internationally." Ezra is now on the US Paralympics team and is the world champion in his high jump classification.



Leah recently discovered wheelchair pickleball.

ADAPTIVE SPORTS RESOURCE GUIDE



National Adaptive Sports Organizations

Challenged Athletes Foundation
ChallengedAthletes.org

Move United
MoveUnitedSport.org

American Association of Adapted Sports Programs
AdaptedSports.org

Achilles International
AchillesInternational.org

US Paralympic Sport Development
usopc.org/paralympic-sport-development

Wheelchair Sports Federation
WheelchairSportsFederation.org

Adaptive Sports Programs and Leagues

Adaptive Track & Field USA
atfusa.org

National Wheelchair Basketball Association
nwba.org

USA Climbing — Paraclimbing
USAClimbing.org/compete/paraclimbing

USA Hand Bike Circuit
USAHandBikeCircuit.com

USA Hockey — Sled Hockey
USAHockey.com/SledHockey

US Electric Wheelchair Hockey Association
PowerHockey.com

US Power Soccer Association
PowerSoccerUSA.org

US Wheelchair Rugby Association
uswra.org

Directories of State and Local Adaptive Sports Programs and Resources

- + ChallengedAthletes.org/adaptive-sport-organizations
- + USAadaptive.net/resources-for-adaptive-athletes
- + DASASports.org/contact/athlete-resources
- + usopc.org/grants-and-equipment

Grants for Equipment, Programs, or Travel

Athletes Helping Athletes
RoadRunnerSports.com/content/aha-home-page

Challenged Athletes Foundation
ChallengedAthletes.org/grants

GoHawkeye Foundation
GoHawkeye.org/grants

IM ABLE Foundation
IMableFoundation.org/grants

Move United
MoveUnitedsport.org/grants

Semper Fi & America's Fund
SemperFiFund.org/our-programs/service-member-family-support-program/specialized-adaptive-equipment

Wheels for Wheels
WheelsForWheels.com/grants



Adaptive Sports and Disabled Athletes in the Media

Watch

2024 Paralympic Games on NBC
NBCOlympics.com/paralympic-games

“Murderball” (documentary) on Prime Video
amazon.com

“Stumped” (documentary) on Vimeo
vimeo.com/ondemand/mostumped

Read

“4 Lessons I Learned from Adaptive Sports,” by Chad Wilson (Quest Magazine online)
MDAQuest.org/4-lessons-i-learned-from-adaptive-sports

“Letters from Leah: The Importance of Physical Activity,” by Leah Zelaya (Quest Blog)
MDAQuest.org/letters-from-leah-the-importance-of-physical-activity

“Limitless,” by Mallory Weggemann with Tiffany Yecke Brooks (biography)
ThomasNelson.com/p/limitless

Move United Magazine
MoveUnitedSport.org/news-media/move-united-magazine

“Tenacious,” by Patty Cisneros Prevo (children’s book)
LeeAndLow.com/books/tenacious

Listen

Redefining Disability (podcast)
MoveUnitedSport.org/adaptive-sports-podcast

Sports Saved My Life (podcast)
AngelCitySports.org/sports-saved-my-life-the-podcast





Brayden Tiernan plays wheelchair basketball.

Beyond sports

For people with neuromuscular diseases, participating in adaptive sports offers a range of physical, emotional, and social benefits, such as improving strength, cardiovascular health, self-esteem, healthy habits, and social connections.

“With adaptive sports, like any sport, you learn about teamwork, testing your boundaries, and putting yourself in uncomfortable positions and realizing you can do things you didn’t think you could — and that translates over into the rest of your life,” says Glenn Merry, Executive Director at Move United ([MoveUnitedSport.org](https://www.MoveUnitedSport.org)), a network of more than 225 organizations offering adaptive sports across the United States.

That’s been 16-year-old Brayden Tiernan’s experience. Brayden, who lives with Charcot-Marie-Tooth disease (CMT) and competes in wheelchair basketball and track and field, says that playing adaptive sports has taught him to be more independent and willing to try new things. Along with the physical benefits of sport, he’s gained practical skills, like wheelchair maintenance, and is mentoring younger athletes.

“My favorite part about adaptive sports is you’re not just learning how to play a sport, you’re also learning how to live life with disabilities,” he says.

Brayden’s parents have seen his confidence grow, and they are grateful for the friendships that have flourished. “We get to see him play sports, and we love the camaraderie he has with his teammates,” says Brayden’s father, Patrick. “And then we get to share our experiences with the other parents and give each other tips.”

Their school district in Louisville, Kentucky, has helped make this possible. “They started a whole adaptive program and are working on trying to get more events,” says Brayden, who plays on his high school wheelchair basketball team and in a competitive league with the Louisville Junior Mustangs.

“We get to see him play sports, and we love the camaraderie he has with his teammates. And then we get to share our experiences with the other parents and give each other tips.”

— Patrick Tiernan



ACTIVITIES FOR ALL

Sports aren’t the only way to enjoy being active. Find out what keeps others in our community moving at [MDAQuest.org/tag/staying-active](https://www.MDAQuest.org/tag/staying-active).

Overcoming obstacles

Despite the growth of adaptive sports through organizations such as Move United and in schools, a lack of local opportunities in some areas remains an obstacle. Athletes might need to travel considerable distances for adaptive programs, which is time-consuming and expensive, putting these activities out of reach for many.

League play often requires traveling for games and tournaments. Thankfully for Brayden, the Junior Mustangs cover hotel and gas expenses, but his track-and-field program does not cover these costs.

Getting equipment can be another obstacle for adaptive athletes. For example, some wheelchair sports require specially designed wheelchairs, which may need to be customized with seatbelts and cushions. Then there are the added costs of maintaining and repairing equipment. “It can build up pretty quickly,” says Patrick. “I don’t know where we would be if it weren’t for people and organizations donating their time and money.”

A dose of creativity and resourcefulness is helpful when looking for ways around these barriers. Organizations such as Challenged Athletes Foundation (ChallengedAthletes.org) and Move United offer grants to offset some of the burden, while others, such

as Angel City Sports, loan equipment to athletes. Brayden recently applied for a grant through Challenged Athletes Foundation to replace the basketball wheelchair he outgrew. He also used crowdfunding to purchase a used racing chair so he could compete at regional and national track competitions.

Jaime has been the grateful recipient of donated sports wheelchairs. “That’s how it is in the disability community; we get a new chair, and we donate the other one,” he says.

Get moving

Leah’s biggest piece of advice for people considering playing an adaptive sport — especially if they’re feeling nervous about the idea — is to check it out in person.


“Seeing people with different conditions doing all these sports is truly what continues to motivate me,” she says. “When you see other people with different types of disabilities continue to do what they love, it’s such a special thing.”

Angel City Sports offers events in Southern California throughout the year where new and experienced athletes from around the country come together for adaptive sports clinics, recreational programs, and competitive sports. Their flagship event, the Angel City Games, is held in Los Angeles each summer.

“It’s really good for people who want to learn and be exposed to sports because we offer 15 to 20 different sports, and it’s a very welcoming environment for new athletes,” Clayton says.

Brayden, who admits he didn’t think he’d like wheelchair basketball when he first tried it, encourages people to give adaptive sports a chance and to be open-minded about what activity might be a good fit for them. “I’m not going to be able to do wheelchair basketball or wheelchair track forever because my disability progresses in all four limbs. But as my disability progresses, I can do power soccer and other sports that are still really fun,” he says. [Q](#)

Charmaine Dymond is a freelance writer in Halifax, Canada.



Jaime has a wheelchair customized for sports like tennis.

CLINICAL GO HIGHER

Mindy Leffler admits that having her son participate in clinical trials for Duchenne muscular dystrophy (DMD) has been both a privilege and a sacrifice.

Her son, Aidan, now a 20-year-old student at the University of Washington in Seattle, participated in several clinical trials starting at 8 years old. She recalls years of missed birthdays, skipped holiday celebrations, and hours spent in transit. For one trial, Mindy and her family flew from Seattle to Vancouver every Wednesday for weekly clinic visits.



TRIALS

New digital health technologies can bring more participants into clinical trials and speed up research

BY ANDREW ZALESKI



Image: elenabs/Getty Images

“Families like mine have given up literal flesh and blood, sweat and tears, and the one thing we don’t all have, which is time, in pursuit of answers.”

— **Mindy Leffler**

It was hard, but they did it to contribute to the search for DMD treatments that might stem the disease progression and, ultimately, find a cure.

“Families like mine have given up literal flesh and blood, sweat and tears, and the one thing we don’t all have, which is time, in pursuit of answers,” Mindy says.

Over the course of this journey, she thought that there must be a better way. That led Mindy, a former information architect, to create the Duchenne Video Assessment. It allows families to record video of their

child performing certain tasks at home and submit that footage to researchers. These videos give researchers valuable data on the child’s functional abilities in a real-world environment and save the family from having to travel to a clinical trial site for functional tests.

Today, more clinical trials are incorporating digital health technology, like Mindy’s video assessment, wearable devices, and telehealth visits. These technologies are improving the experience for clinical trial participants, increasing access to clinical trials, and speeding up research.

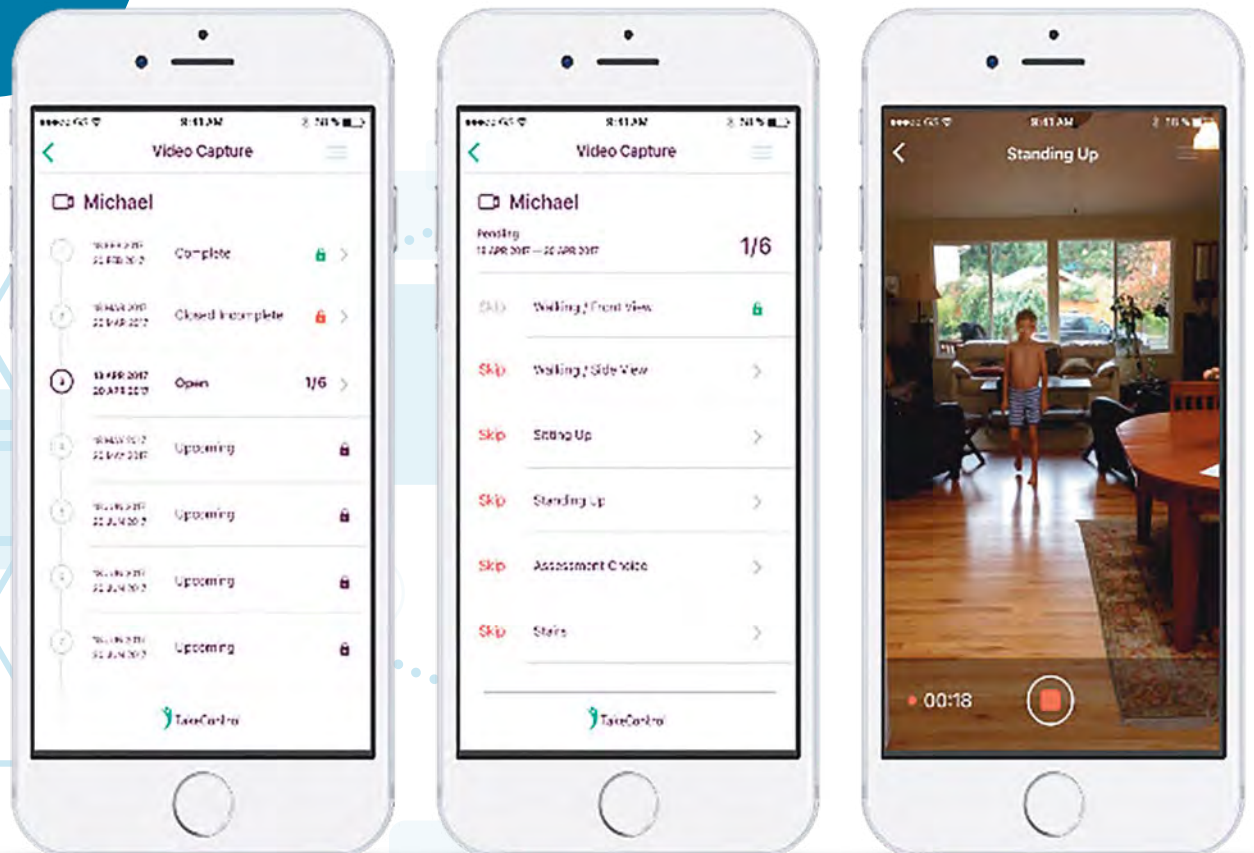
Increasing access

Taking time away from work and family and traveling to clinical trial sites are all-too-common barriers to clinical trial participation.



Mindy Leffler

The Duchenne Video Assessment allows families to record their child performing certain tasks at home.



“It really narrows down the pool of people who can participate in clinical trials, which is hugely inequitable. It’s not fair,” says Mindy.

Beyond logistical considerations, members of the neuromuscular disease community may find they do not qualify for a trial as their disease progresses. Many DMD trials, for example, require participants to complete a six-minute walk test.

“As a neuromuscular disease progresses, certain movements often become more challenging, or an individual is unable to do them at all,” says Paul Melmeyer, MDA’s Vice President of Public Policy and Advocacy. “Consequently, they’re locked out of not only one specific clinical

trial — they’re locked out of all clinical trials.”

New digital health technologies can reduce or eliminate the visits to clinical trial sites that are commonly required in clinical trials.

This increases access to clinical trials, bringing in a more diverse group of participants.

Wearable devices, telehealth services, and software tailored to people with mobility challenges can also provide additional outcomes beyond more limited tests, like the ability to walk or run, that make up the endpoints for many trials today.

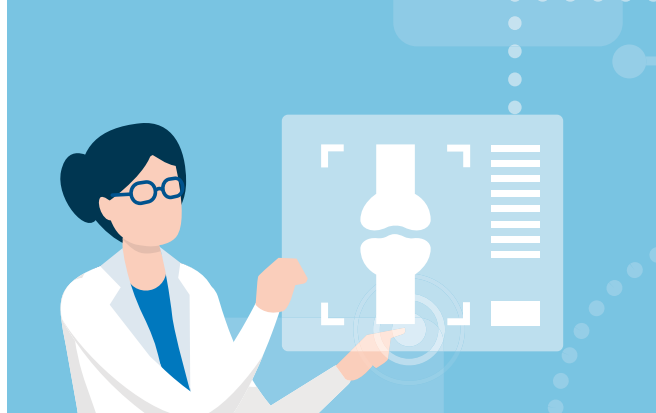


Paul Melmeyer

A better way

Mindy co-created the Duchenne Video Assessment, which she now oversees as Managing Director of Qualitative Research and Psychometrics at Emmes Endpoint Solutions, to address the issues she sees with the typical clinic-based study design. As she shuttled her son to various clinical trials, she wondered why there wasn’t a better way to measure decline or improvement for kids with DMD. After all, her son wasn’t worried about passing a fitness-oriented six-minute walk test — he just wanted to get from the classroom to the cafeteria on his own.

The Duchenne Video Assessment comprises 16 tasks: For younger boys, there are active tasks such as jumping forward; for young men with less function, tasks include eating 10 bites, reaching across a table to flip



WHAT TO KNOW ABOUT CLINICAL TRIALS

If you’re considering a clinical trial, find out how the study is designed and what it requires before you join.

It is important to understand what is involved in a study, including if digital health technology is used, if there are in-person visits, and if visits can be conducted via telehealth. You should also know the potential risks and end goal.

Many trials have a study coordinator who can answer questions and help address barriers to participation.

Read “9 Questions to Ask Before Joining a Clinical Trial” at [MDAQuest.org/9-questions](https://mdaquest.org/9-questions).

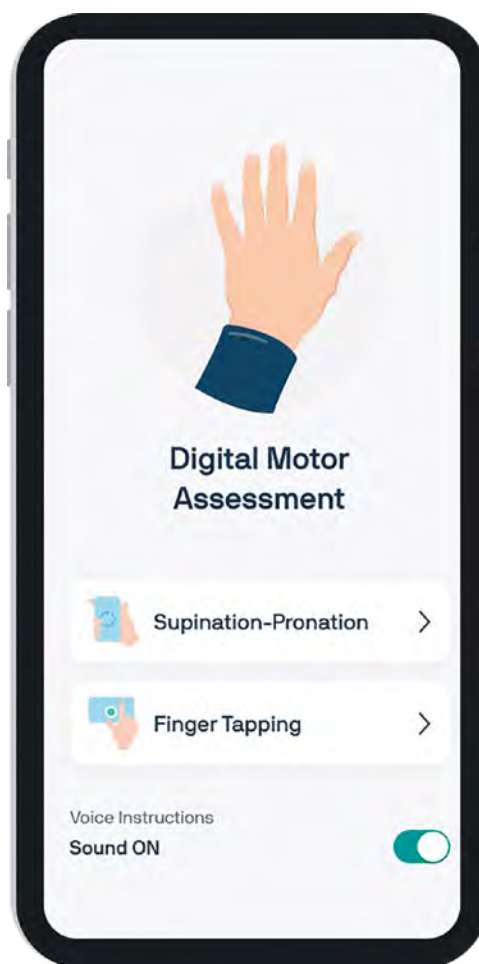
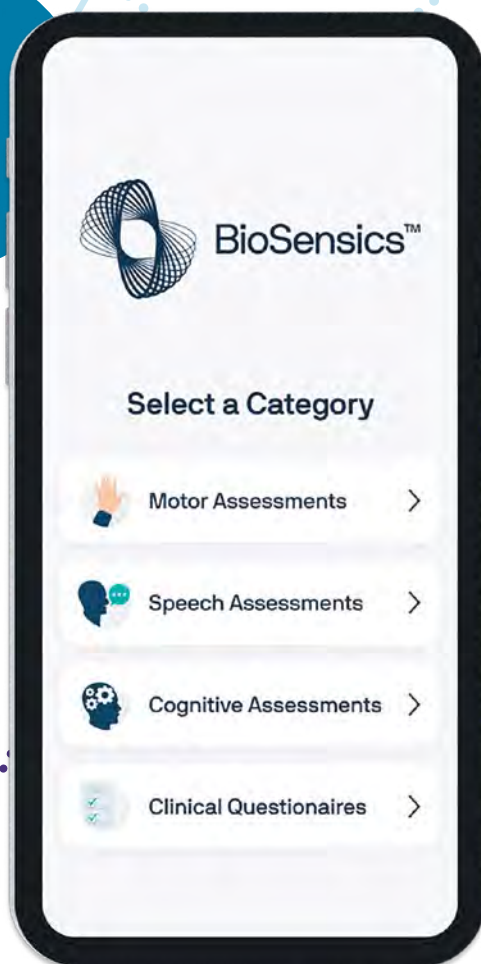
over a cell phone, pushing buttons on a remote control, and driving a power wheelchair. Through a HIPAA-compliant app, families can record their child going through each task in as little as 15 minutes. Those videos are then scored by trained, certified physical therapists who can quantify disease progression. The outcome being studied, then, is neither ambulatory nor nonambulatory; instead, it’s a series of movements applicable across the entire functional spectrum of people with DMD.

So far, this video assessment tool has been used in eight clinical trials.

New digital health technologies can reduce or eliminate the visits to clinical trial sites that are commonly required in clinical trials.

BioDigit Home uses wearable sensors and a mobile app to collect patient data at home.

Digital health technology also allows studies to recruit nationally instead of locally or regionally, giving researchers a larger, more diverse pool of participants.



Getting results

Digital health tools can also capture more objective data, according to Ashkan Vaziri, the founder and CEO of Massachusetts-based BioSensics, which develops wearable devices and digital assessments to help quantify a range of movement in people with amyotrophic lateral sclerosis (ALS), multiple system atrophy (MSA), and other neuromuscular and neurological conditions. The company has created wearables to

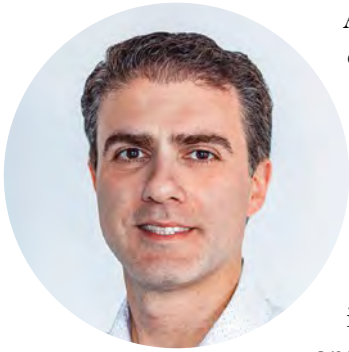
monitor gait and balance, as well as digital tools to measure speech impairment and fine motor control, all of which have been used in dozens of studies so far.

“The current method of assessing various treatments in clinical trials involves patients visiting the clinic, performing specific tasks such as walking, and receiving a score based on that performance. However, this approach captures only a snapshot of a singular activity,” he says. “These advancements allow for data collection at home, providing real-world data on a regular basis. This enables a more comprehensive evaluation of drug effects, yielding more meaningful insights.”

Digital health technology also allows studies to recruit nationally instead of only locally or regionally, giving researchers a larger, more diverse pool of participants. Trials that are less accessible and less diverse are less likely to quickly determine whether a new therapy delivers safe, effective treatment.

+FIND A TRIAL

Use MDA’s Clinical Trial Finder to search by condition and other criteria at mda.org/clinical-trials.



Ashkan Vaziri

And because trial sponsors don't have to coordinate clinic visits, enrollment is streamlined.

Faster enrollment leads to faster data collection, which leads to faster drug development, says Ashkan — this, in turn, can lead to quicker answers on whether an experimental drug or therapy works.

Despite their promise, such tools' use in clinical research is in the early stages. "I believe the percentage of clinical trials that use digital health technologies is still very small," says Ashkan, although he expects to see significant growth in the use of remote technologies and telehealth in clinical trials in the coming years.

Improving clinical trials


To streamline data collection, MDA's Neuromuscular Observational Research (MOVR) Data Hub assembles anonymized clinical and genetic information from individual patients across the MDA Care Center Network, creating a trove of data to help researchers.

Improving clinical trial design is a priority for MDA, and it's an effort that involves getting digital health technology into more clinical trials. MDA has partnered with Mitsubishi Tanabe Pharma for an ALS study in which data collected remotely (using Fitbit and iPad) will be paired with the participants' MOVR data entered from their clinic visits. This arrangement allows investigators to see how the remotely collected data compares to data collected at the clinic, with the ultimate goal of conducting fully remote studies.

In addition, Paul says MDA regularly communicates with the US Food and Drug Administration (FDA) and biotechnology partners to encourage trial designs that

Improving clinical trial design is a priority for MDA, and it's an effort that involves getting digital health technology into more clinical trials.

shift away from endpoints that rely on walking tests and motor ability, so studies are more open to people with diminished mobility — and test what is actually important to those living with neuromuscular diseases.

"Digital health technology, wearables, and other innovations have great promise," Paul says. "If more individuals are eligible to participate, that means the therapy will get to our community that much more quickly." 

Andrew Zaleski is a journalist who lives near Washington, DC, and wrote about living with myotonic dystrophy type 1 for GQ magazine.



MY STUDY STORY

Lacey Woods details her experience in a natural history study for limb-girdle muscular dystrophy (LGMD) at [MDAQuest.org/my-experience](https://mdaquest.org/my-experience).



Mission in Focus at 2024 MDA Clinical & Scientific Conference



Brooke Eby (left) and Donald S. Wood, PhD, MDA President and CEO, spoke at the 2024 MDA Clinical & Scientific Conference.

Brooke Eby, a social media phenomenon who has been chronicling her journey with amyotrophic lateral sclerosis (ALS) since her diagnosis at the age of 33, delivered the keynote speech, reminding the clinicians and researchers present of the urgency of their work.

“[ALS] forced me to live life in dog years; I have to make one equal seven,” she said. “I live at the speed of ALS. Therefore, you need to operate at the speed of ALS.”

With humor, she shared her perspective on the importance of their work and how it is improved by keeping patients at the center of all they do. “I am your ultimate stakeholder. I am the person living with the disease that you’re trying to end,” she reminded them.

Thousands of researchers, healthcare providers, industry professionals, and people living with neuromuscular diseases from around the world gathered in Orlando, Florida, in March for the largest annual global meeting of the neuromuscular disease community at the 2024 MDA Clinical & Scientific Conference.

After an opening address by Donald S. Wood, PhD, MDA’s President and CEO, these esteemed minds found themselves listening raptly to somebody far outside of their professional sphere: a TikTokker.

Brooke’s keynote was followed by three days of sessions that provided conference participants with the latest information on topics ranging from newborn screening to streamlining care for pediatric neuromuscular diseases to preparing for new gene therapies.

This annual conference is an important opportunity for drug researchers working in laboratories, doctors and therapists caring for people with neuromuscular diseases, and community members living with these conditions to mingle and share their experiences and visions for the future.

MDA Awards \$140,000 in Advocacy Grants

MDA is excited to announce the latest recipients of Advocacy Collaboration Grants. Seven organizations were awarded a combined \$140,000, spanning various aspects of neuromuscular disease advocacy and community support, including

engagement with healthcare professionals, grassroots advocacy, more inclusive clinical trials, and more.



Learn more about the Advocacy Grants at MDAQuest.org/advocacy-grant-projects.



Ben Willis and his camp counselors

Get Ready for Camp!

Leaving home to spend a week at Summer Camp is a big deal for kids and parents, so MDA works hard to make sure families are as prepared as possible. After being accepted, families will receive plenty of helpful information, including packing lists, arrival and departure procedures, detailed directions to the site, an overview of the week's activities, and any other pertinent information specific to the camp.

"While I don't think you ever feel truly prepared as a parent, MDA went above and beyond to prepare us for Summer Camp," said Jay Willis, whose son Ben, 17, lives with spinal muscular atrophy (SMA) and is getting ready for his seventh time at Summer Camp. "We attended a panel discussion where individuals with muscular dystrophy, all highly successful, independent adults, shared their success stories. Nearly all of them attributed their independence and achievements to MDA Summer Camp. Hearing that firsthand was incredibly reassuring. When we decided to let Ben attend, MDA provided detailed information packets and pre-camp orientation sessions, which helped ease our anxiety and ensure Ben's needs would be met."

He adds this advice: "Take this time to relax and enjoy some activities you wouldn't normally do when your child is with you. Embrace the opportunity to recharge. And when it's time to pick up your camper, be prepared for them to have had the time of their lives and possibly be disappointed to leave!"

Ready to sign up for camp? Visit mda.org/SummerCamp for more information.

Praise for the Gene Therapy Community Support Groups

MDA's Gene Therapy Community groups offer peer-to-peer support for those eligible for currently approved therapies and their parents or caregivers. Here is what a couple of current support group members have to say about their experiences:

Being a part of the support group has been so reassuring! It is nice to hear stories and get advice from people whose children have had their diagnosis longer than ours. Knowing that they've had the same feelings after getting the diagnosis makes me feel less alone.
—Jami, Denver, CO, son with spinal muscular atrophy (SMA)

The gene therapy community group was the first time I was truly able to see that I am not alone in this experience. This has been so powerful for me.
—Isabel, Mount Vernon, WA, son with SMA



Next Steps Seminar Offers Connection and Information

More than 25 participants with various neuromuscular diseases or their parents gathered in January for MDA's Next Steps Seminar: Newly Diagnosed Pediatrics. This seminar covered crucial topics such as early intervention and K-12 education, insurance and waivers, social/emotional well-being, durable medical equipment, home modifications, and more. Family panel discussions and Q&A sessions with experts in various fields were also offered.

Upcoming seminars will cover topics for newly diagnosed adults, transitioning to adulthood, and individuals newly diagnosed with ALS.

To learn more and to register, visit mda.org/seminars.



Advocacy Efforts Take Flight



Front row, left to right: Senator Tammy Duckworth (D-IL), Mindy Henderson, VP, Disability Outreach & Empowerment and Editor-in-Chief, MDA Quest Media. Back row, left to right: Michele Erwin, President and Founder, All Wheels Up; Paul Melmeyer, Executive Vice President, Public Policy & Advocacy, MDA; Pete Buttigieg, Secretary of Transportation, US Department of Transportation; Alexandra Bennewith, Vice President, Government Relations, United Spinal Association; Michael Lewis, Vice President of Policy, American Association of People with Disabilities

Improvements to Air Travel

MDA continues to celebrate major progress to improve air travel for people living with disabilities. First, the US Department of Transportation released a monumental proposal rule that, if finalized, would improve the safety, rights, and dignity of air travelers with disabilities. Provisions in this rule include:

- + Creating new standards to ensure that passengers using wheelchairs can promptly board and deplane
- + Mandating training for airline personnel and crew members who assist passengers with disabilities
- + Providing onboard wheelchairs to be available on most commercial flights
- + Offering greater transparency to wheelchair users regarding the size constraints of aircraft cargo holds
- + Requiring airlines to provide passengers with options to repair and replace mishandled wheelchairs and mobility devices

MDA's Paul Melmeyer, Vice President of Public Policy, and Mindy Henderson, Vice President of Disability Outreach & Empowerment and Quest Editor-in-Chief, went to the White House to celebrate this proposal.

In February, the Senate Commerce Committee also unanimously passed its version of FAA Reauthorization out of committee. The provisions in this bill represent the most impactful reforms to improve air travel for the disability community in nearly 40 years. The US House and Senate must come together on a joint bill before sending it to the president.

Join MDA's campaign to improve air travel at mda.org/AirTravel.

MDA Celebrates Rare Disease Day at the White House



In February, MDA Vice President of Public Policy and Advocacy

Paul Melmeyer joined the White House Office of Science and Technology Policy to participate in the White House Rare Disease Forum. The panel convened individuals living with rare diseases, their family members and caregivers, providers, researchers, and administration officials to discuss current rare disease policy challenges.

MDA Joins Fellow ALS Organizations on Capitol Hill

MDA joined forces with the ALS Association, I AM ALS, and the Les Turner ALS Foundation for a Day of Action on Capitol Hill to support the ALS Better Care Act. This bill would improve access to multidisciplinary care for those living with ALS.

Quest Marketplace

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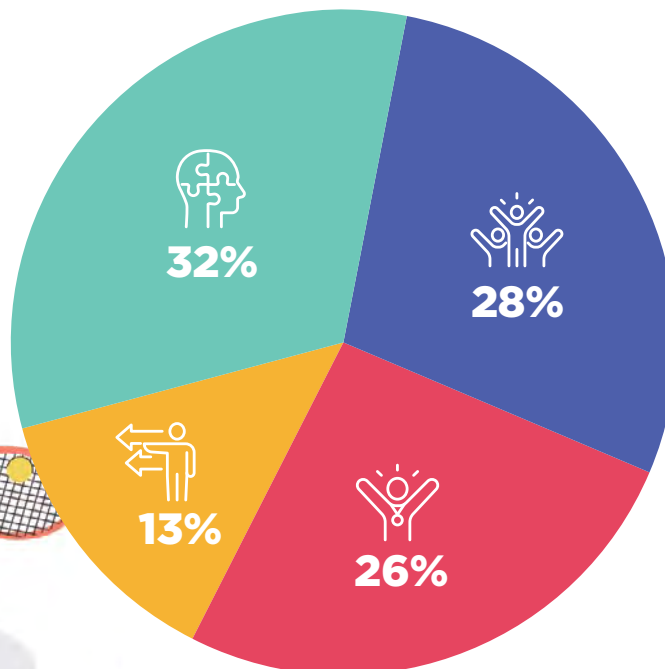
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Quest Poll: Adaptive Sports

Have you or your child ever participated in adaptive sports? (487 responses)



- Yes, team sports (such as power soccer or sled hockey)
- Yes, individual sports (such as skiing or cycling)
- No, I/my child is not interested in playing adaptive sports.
- No, but I would be interested in learning about adaptive sports.



A Welcome Change

A new writing pursuit leads to unexpected benefits

BY CHRIS ANSELMO

Over the 2022 holiday break, I was at a crossroads. I was browsing my personal blog and realized that I hadn't posted in several months. For many years, my blog was where I shared updates about my journey living with limb-girdle muscular dystrophy type 2B (LGMD2B). Although I still loved to write, I longed to share lessons learned from my experience instead of just writing life updates. More specifically, I wanted to help others going through adversity by sharing strategies and resources that have helped me endure tough times — wisdom acquired through trial and (lots of) error.

In January 2023, I started a Substack newsletter, Hello, Adversity (HelloAdversity.substack.com). It was one of the best decisions I ever made.

Getting started

When I began Hello, Adversity, I didn't know what to expect. Would anyone want to read it? At first, I was afraid that my only subscribers would be my parents. Fortunately, this worry was unfounded. I let my family and friends know about the newsletter, and after a few weeks I hit 100 subscribers. It was encouraging to see that people wanted to hear what I had to say and, more importantly, that the content was resonating. Early feedback indicated that readers enjoyed learning resilience strategies to navigate their day-to-day lives.

Writing about resilience and adversity was a welcome change from my former blog. Although I had never written on a set schedule before, I was able to



Chris Anselmo started a newsletter to share resources and strategies for handling life's challenges.

generate new posts without it feeling like a burden. I knew that this was a good sign for Hello, Adversity's long-term prospects.

Content calendar

After some initial experimentation, I settled on writing one new post per week. Whereas I used to write

Hello,
Adversity



2,500- to 3,000-word posts, I now aim for 1,500 to 2,000 words, which is much more manageable, and only takes a few hours to write from beginning to end.

I like to standardize my writing process as much as possible. For example, after my post publishes on Wednesday morning, I start the next post to keep the momentum going. I scope out a draft by Wednesday night, refine it more on Thursday, then take Friday to Sunday off. On Monday, I edit the draft and add any supporting material, then make final tweaks Tuesday night before scheduling the post for the next morning. Having an idea of what I need to do each day saves me from unnecessary anxiety.

I also keep a content calendar where I can quickly see what resilience strategies are on the schedule for the next five to 10 weeks. That way, I don't have to waste time each week wondering what to write about.

Building an audience

I have primarily used two tactics to build my audience: sharing my newsletter with my network and building relationships with other Substack writers. I've found that many people discovered my site because a friend or family member of mine forwarded them one of my posts. Word-of-mouth is a powerful form of marketing. I have also benefited from Substack's internal recommendation tools where other writers can recommend my work to their audience, leading to new subscribers. Most of my subscribers have come from recommendations.

I must admit, it is easy to get wrapped up in metrics like subscriber count, open rate, etc. There were times when I could have promoted my newsletter in spammy or inauthentic ways to grow my email list but decided against it. At the end of the day, the quality of a reader is more important to me than the number of readers.

“The best part of writing Hello, Adversity — besides writing about a topic I enjoy — has been connecting with readers, meeting new people, and growing relationships.”

— *Chris Anselmo*



FINDING FULFILLMENT

Listen to a Quest Podcast conversation with Isaac Banks, an author and public speaker, about finding personal fulfillment at MDAQuest.org/podcast/isaac-banks.

I want people to learn from and utilize the content rather than open the email then immediately delete it.

The best part of writing Hello, Adversity — besides writing about a topic I enjoy — has been connecting with readers, meeting new people, and growing relationships. This was an unexpected benefit of writing a newsletter. I never considered the importance of these connections until I got started and saw how much value there was in developing a consistent audience of regular readers invested in my story and what I had to say. That has made it so much more enjoyable.

The quality of a reader is more important to me than the number of readers. I want people to learn from and utilize the content rather than open the email then immediately delete it.

Share your interests

The reason I am sharing my experience writing a newsletter is not to toot my own horn but rather to inspire you to share your passions with the world. Whether it's telling your story, shedding light on pressing advocacy issues, raising awareness, teaching your expertise, or discussing your hobbies, there is a community of people out there eager to engage with what you have to say. My preferred form of communication is writing, but you can also do public speaking, take up an artistic pursuit, or something else.

Whatever passion you bring to the table, there is an audience for it. Putting yourself out there can positively impact your life and open doors you never knew existed. I am living proof of that. [Q](#)

Chris Anselmo lives in Connecticut with LGMD2B, an adult-onset neuromuscular disease. You can read and subscribe to his newsletter Hello, Adversity at HelloAdversity.substack.com.



Room With a View

Painting a mural in her home helped Heather Nightingale reconnect with nature



Heather Nightingale feels a sense of peace near her nature mural.

Heather Nightingale, 61, of Griffin Creek, Oregon, has loved outdoor adventure since she was a child. “I’ve spent much of my life hiking trails that beckoned me to explore,” says Heather, who lives with myotonic dystrophy type 1 (DM1). “I’ve always found peace there in the midst of the busy world we live in.”

When disease symptoms, such as rapid heart rate and muscle soreness and fatigue, made long hikes more difficult for Heather, she decided to bring nature to her by painting a mural in her bedroom. Creating the mural — an item on Heather’s bucket list — was a labor of love. Heather sat and painted the lower parts of the

wall, and her husband, Doug, helped her reach the high parts.

“My loving husband literally held me up — sometimes five minutes at a time — so I could complete this 92-square-foot wall painting,” says Heather.

After a year of effort, the mural was complete, and Heather once again felt connected to nature.

“This mural encompasses all the wonderful adventures with family and friends in the great outdoors, like picnics in the snow, the laughter of my children, fun tales of Bigfoot encounters — the list is endless,” Heather says. “I lie in my bed at night and see these beautiful mountains before I close my eyes. It’s so peaceful.” [📺](#)



Heather painted the mural over the course of a year.



Getting to Know Retired L58 Dallas Fire Fighter: Ken Sutcliffe

You may have seen Ken Sutcliffe, a U.S. Army veteran and a retired fire fighter from IAFF Local 58 Dallas Fire Fighters Association, in Answering the Call, a new PSA from the Muscular Dystrophy Association, (MDA). After spending his career answering the call to save others, Ken was diagnosed with amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig’s disease.

“As a fire fighter, my job was about saving lives. I never thought one day I’d be fighting for mine.”

— Ken Sutcliffe, Retired Fire Fighter, Living with ALS

Prior to retirement, Ken joined fire fighters across the country collecting funds in the community – one dollar at a time – as part of the Fill the Boot program to raise money for MDA. Thanks to MDA and Fill the Boot, he and many others living with neuromuscular disease are receiving the treatment and care that they need to live longer and love stronger.



MDA’s Wings Over Wall Street to Celebrate 24 Years

As one of MDA’s most iconic fundraisers dedicated to ALS research, this event has raised over \$13 million since its inception. Donations directly benefit ALS research at the Eleanor and Lou Gehrig ALS Center at Columbia University Medical Center, and the Robert Packard Center for ALS Research at Johns Hopkins.

2024 Marks 40 Years with Price Chopper/Market 32

With over \$30 million raised, Price Chopper/Market 32’s long-standing partnership with MDA has helped accelerate breakthroughs for ALS. We’re grateful for their associates and community’s dedication and are proud to honor their invaluable contribution to the ALS community.

MDA Golf Classic: A Tradition for 14 Years

Since teaming up with Dutch Bros Arizona in 2017, this memorable event has helped MDA invest millions in ALS research and provide support and advocacy for the entire ALS community. Last year, the tournament welcomed Doug Clough, a local avid golfer living with ALS, who enjoyed his return to the greens.



Do you have Becker Muscular Dystrophy? Are you interested in participating in a clinical trial? Have you considered the GRAND CANYON Trial?

The GRAND CANYON Trial

Edgewise Therapeutics is seeking individuals living with Becker for the pivotal cohort of the GRAND CANYON trial of EDG-5506, an investigational treatment for Becker. The GRAND CANYON trial aims to evaluate safety and effects on function and biomarkers of muscle damage in adult males with Becker. Participation is for approximately 19 months and will require up to 7 site visits over the duration of the trial.

The Investigational Therapy

EDG-5506 is an investigational therapy in the form of a daily oral pill. EDG-5506 is designed to prevent contraction-induced muscle injury that occurs with daily activity in Becker. EDG-5506 is designed to limit this damage and help prevent the functional decline that accompanies disease progression in Becker.



Can I join the GRAND CANYON trial?

Approximately 120 adults living with Becker are expected to be enrolled in the trial. To participate you must fit the following criteria:

- Genetic diagnosis of Becker Muscular Dystrophy
- Male, ages 18-50
- Ambulatory with the ability to complete physical function activities (i.e., North Star Ambulatory Assessment, 100-meter timed test*)
- Able to meet other criteria as specified

Travel and other resources will be coordinated and provided for participants

*Select assistive devices such as orthotics or a cane can be used during the 100-meter timed test

Sites across the United States began enrolling for the GRAND CANYON trial in 2023. Sites in up to 10 additional countries are expected to open enrollment for GRAND CANYON in 2024. For more information, please go to clinicaltrials.gov (NCT05291091) or scan the QR code to access the GRAND CANYON study website.

