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Quest

MDAQUEST.ORG ISSUE 3 / 2025

75 YEARS AND COUNTING

This special anniversary issue is dedicated to the unstoppable community MDA serves every day.

IMPORTANT SAFETY INFORMATION

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

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

- have an infection or fever.
- have recently received or are scheduled to receive any vaccinations.
- have any history of allergic reactions.
- have kidney (renal) problems.
- are pregnant or plan to become pregnant. It is not known whether VYVGART HYTRULO will harm your unborn baby.
  - Pregnancy Exposure Registry. There is a pregnancy exposure registry for women who use VYVGART HYTRULO during pregnancy. The purpose of this registry is to collect information about your health and your baby. Your healthcare provider can enroll you in this registry. You may also enroll yourself or get more information about the registry by calling 1-855-272-6524 or going to VYVGARTPregnancy.com
- are breastfeeding or plan to breastfeed. It is not known if VYVGART HYTRULO passes into your breast milk.

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

VYVGART HYTRULO can cause side effects which can be serious, including:

- **Infection.** VYVGART HYTRULO may increase the risk of infection. If you have an active infection, your healthcare provider should delay your treatment with VYVGART HYTRULO until your infection is gone. Tell your healthcare provider right away if you get any of the following signs and symptoms of an infection:
  - fever
  - chills
  - frequent and painful urination
  - cough
  - pain and blockage of nasal passages
  - wheezing
  - shortness of breath
  - sore throat
  - excess phlegm
  - nasal discharge
- **Allergic reactions (hypersensitivity reactions).** VYVGART HYTRULO can cause allergic reactions that can be severe. These reactions can happen

VYVGART Hytrulo®

(efgartigimod alfa and hyaluronidase-qvfc)

A self-injection for CIDP that can fit your life, your way

A once-weekly **self-injection** that takes **about 20-30 seconds** can mean more time in your day for the people and things you love—like a day on the golf course with your buddies*

Ask your doctor if VYVGART Hytrulo is right for you

Visit **VYVGARTHytruloSelfinjection.com** to learn more about self-injection with the VYVGART Hytrulo prefilled syringe

*Please see **Patient Information**. Follow appropriate administration steps in the **Instructions for Use**. Monitor for signs and symptoms of an allergic reaction for at least 30 minutes after injection. If an allergic reaction occurs, you should seek medical attention.

during, shortly after, or weeks after your VYVGART HYTRULO injection. Tell your healthcare provider or get emergency help right away if you have any of the following symptoms of an allergic reaction:

- rash
- swelling of the face, lips, throat, or tongue
- shortness of breath
- hives
- trouble breathing
- low blood pressure
- fainting

- **Infusion or injection-related reactions.** VYVGART HYTRULO can cause infusion or injection-related reactions. These reactions can happen during or shortly after your VYVGART HYTRULO injection. Tell your healthcare provider if you have any of the following symptoms of an infusion or injection-related reaction:
  - high blood pressure
  - chills
  - shivering
  - chest, stomach, or back pain

The most common side effects of VYVGART HYTRULO include respiratory tract infection, headache, urinary tract infection, and injection site reactions.

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

What is VYVGART HYTRULO® (efgartigimod alfa and hyaluronidase-qvfc)?

VYVGART HYTRULO is a prescription medicine used to treat adults with chronic inflammatory demyelinating polyneuropathy (CIDP).

It is not known if VYVGART HYTRULO is safe and effective in children.

Please see full Prescribing and Patient Information for VYVGART HYTRULO at VYVGARTHytrulo.com/PI and talk to your doctor.

Dosage form and strength:

VYVGART Hytrulo is available as a single-dose subcutaneous injection containing 200 mg/mL of efgartigimod alfa and 2,000 U/mL of hyaluronidase per prefilled syringe.

Please see Consumer Brief Summary on next page.

US-VYV_HYT-25-00171 V1 09/2025

argenx

CONSUMER BRIEF SUMMARY

Important Information about 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 HYTRULO with your healthcare provider. The US Food and Drug Administration (FDA)-approved product labeling can be found for VYVGART HYTRULO by visiting www.VYVGARTHYTRULO.com/PI or calling 1-833-VYVGART (1-833-898-4278).

What is VYVGART HYTRULO?

VYVGART HYTRULO is a prescription medicine used to treat adults with chronic inflammatory demyelinating polyneuropathy (CIDP).

It is not known if VYVGART HYTRULO is safe and effective in children.

Who should not take VYVGART HYTRULO?

Do not take VYVGART HYTRULO if you are allergic to efgartigimod alfa, hyaluronidase, or any of the ingredients in VYVGART HYTRULO. See the end of this brief summary for a complete list of ingredients in VYVGART HYTRULO.
VYVGART HYTRULO can cause serious allergic reactions and a decrease in blood pressure leading to fainting.

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

- have an infection or fever.
- have recently received or are scheduled to receive any vaccinations.
- have any history of allergic reactions.
- have kidney (renal) problems.
- are pregnant or plan to become pregnant. It is not known whether VYVGART HYTRULO will harm your unborn baby.
  - Pregnancy Exposure Registry. There is a pregnancy exposure registry for women who use VYVGART HYTRULO during pregnancy. The purpose of this registry is to collect information about your health and your baby. Your healthcare provider can enroll you in this registry. You may also enroll yourself or get more information about the registry by calling 1-855-272-6524 or going to VYVGARTPregnancy.com
- are breastfeeding or plan to breastfeed. It is not known if VYVGART HYTRULO passes into your breast milk.

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

What are the possible side effects of VYVGART HYTRULO?

VYVGART HYTRULO can cause side effects which can be serious, including:

- **Infection.** VYVGART HYTRULO may increase the risk of infection. If you have an active infection, your healthcare provider should delay your treatment with VYVGART HYTRULO until your infection is gone. Tell your healthcare provider right away if you get any of the following signs and symptoms of an infection:
  - fever
  - chills
  - frequent and painful urination
  - cough
  - pain and blockage of nasal passages
  - wheezing
  - shortness of breath
  - sore throat
  - excess phlegm
  - nasal discharge
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These are not all the possible side effects of VYVGART HYTRULO. Call your doctor for medical advice about side effects.
You may report side effects to FDA at 1-800-FDA-1088.

What are the ingredients in VYVGART HYTRULO prefilled syringe?

Active ingredients: efgartigimod alfa and hyaluronidase (human recombinant)

Inactive ingredients: arginine hydrochloride, histidine, L-histidine hydrochloride monohydrate, methionine, polysorbate 80, sodium chloride, sucrose, and water for injection, USP

How is VYVGART HYTRULO prefilled syringe administered?

A single-dose prefilled syringe for (under the skin) subcutaneous injection. You or your caregiver should receive training on the right way to prepare and inject VYVGART HYTRULO using the single-dose prefilled syringe. Do not try to inject VYVGART HYTRULO prefilled syringe until you have been shown the right way by a healthcare provider. See the detailed Patient Information and Instructions for Use that come with VYVGART HYTRULO prefilled syringe.

VYVGART HYTRULO is manufactured by: argenx BV, Industriepark 7, 9052 Zwijnaarde, Belgium, U.S. License No. 2217
Halozyme Inc., 12390 El Camino Real, San Diego, CA 92130, U.S. License No. 2187
VYVGART HYTRULO is distributed by: argenx US, Inc., 33 Arch Street, Boston, MA 02110

argenx

Contents

SPECIAL
ISSUE 3 **2025**

SPECIAL SECTION

SPECIAL DEPARTMENTS

Reimagining Our Future


Sharon Hesterlee, PhD

MDA's 75th anniversary isn't just a milestone; it's a product of momentum. This organization was built by people like you who saw unmet need and refused to look away. For three-quarters of a century, MDA has helped change the shape of what's possible for people living with neuromuscular diseases.

That legacy matters. But legacy alone doesn't move us forward. What carries us into the future is your trust and our determination to keep earning it. Families like yours are navigating a landscape that's more tangled than ever, but you're speaking with powerful precision about what you need. You're not asking MDA to do more of the same. You're asking us to think differently.

"What carries us into the future is your trust and our determination to keep earning it."

So we are. We're reimagining what it means to be a true partner on your disease journey. That means staying ahead of regulatory and legislative barriers, designing new pathways to success, and using MDA's role as a connector between researchers, clinicians, agencies, industry partners, advocates, families, and many others in the neuromuscular community to bring fresh ideas to the forefront and turn them into action.

+DONATE TO MDA
Scan this QR code to support MDA's vital work.

In this next chapter of MDA's story, we're listening intently, moving deliberately, and building a future alongside you that reflects what you've told us matters most. This special issue of Quest Magazine honors the decades behind us, while embracing the work still ahead with resolve, creativity, and care.

Sharon Hesterlee, PhD
Interim President & Chief Executive Officer
Muscular Dystrophy Association

MDA 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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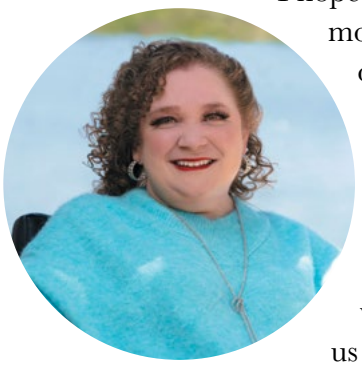
FOLLOW MDA ON

PUBLICATION MANAGEMENT
BackPocket AGENCY

My Journey with MDA

As the Quest Media team produced this special issue of Quest Magazine in honor of MDA's 75th anniversary, I've been swept up in a wave of nostalgia — memories of my lifelong journey with MDA, which began in 1975.



I hope you'll indulge me for a moment, because I can't think of a better community to share these thoughts with.

I was diagnosed with spinal muscular atrophy (SMA) at 15 months, and MDA entered my family's world as a life raft, helping us navigate this unexpected journey. As a child, I proudly served as an MDA Ambassador and attended MDA Summer Camp.

When I was 17, I heard that the gene responsible for SMA had been discovered. I remember the hope and excitement I brought to school the next day. I remember, too, the voice of doubt from a classmate who told me not to expect more progress in my lifetime. Yet here we are. I cried tears of joy and relief during my first dose of Spinraza seven years ago.

MDA has been woven into every chapter of my life. I've spent countless hours at MDA events, met lifelong friends, and gathered memories that make me smile. I first met Jerry Lewis on a football field when I was 5. Later, at 27, I sang on the MDA National Telethon, backed by the telethon orchestra.

Mindy, age 8

And now, working for MDA, I have the honor of watching other people's dreams come true as new treatments are approved, sharing your stories, and learning even more from this incredible community. Looking at where we began, where we are today, and where we could be years from now, I'm struck by one truth: Progress is not a rumor; it's a legacy.

To every scientist in the lab, volunteer at a gala, parent cheering in the stands, and child daring to dream, your courage and perseverance keep us going.

Mindy Henderson
Vice President, Disability Outreach & Empowerment
Editor-in-Chief, Quest Media

ICONS TO WATCH FOR THROUGHOUT QUEST

QUEST MAGAZINE ONLINE

QUEST BLOG

QUEST PODCAST

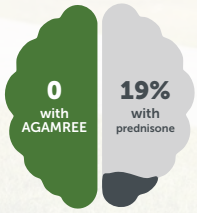
DISEASES A-Z CONTENT LIBRARY

aGamree® (vamorolone) oral suspension 40mg/mL

"Now that we've been on it for 6-ish months...he's happier overall, and he'll tell you that he just feels *better*."*

-Jessica, mother of Gaven, a real AGAMREE® patient

In a 24-week, placebo- and active-controlled clinical study:



Clinically Relevant TEAEs of Psychiatric Disorders[2]

Patients treated with AGAMREE showed no moderate or severe clinically relevant behavior TEAEs[1†]

Additional data are needed to determine the long-term effects of AGAMREE[3]

GAVEN
A real AGAMREE patient

LEARN MORE at **AGAMREEhcp.com**

*Results may vary from person to person.
†Chi-square *P*-value for patient counts: AGAMREE 6.0 mg/kg/day vs prednisone, *P*=0.0074; AGAMREE 6.0 mg/kg/d vs placebo, *P*=0.3215; prednisone vs placebo, *P*=0.0294. Treatment-emergent adverse events related to psychiatric disorders include the following adverse reactions: abnormal behavior, aggression, emotional disorder, mood swings, personality change, sleep disorder, trichotillomania.[1,2]
Clinically relevant AEs were either at least moderate in severity or leading to withdrawal from study or serious event.[1]
TEAE, treatment-emergent adverse event.

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.

Catalyst pharmaceuticals

References: 1. Henricson E et al. Poster presented at: 10th EAN Congress; 2024; Helsinki, Finland.
2. Data on file. Catalyst Pharmaceuticals, Inc. **3.** Guglieri M et al. *JAMA Neurol.* 2022;79(10):1005-1014.

aGamree® (vamorolone) oral suspension 40mg/mL

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.

DOSAGE 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 quadriparesis. 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)

Adverse Reaction	AGAMREE 2 mg/kg/d (N=30) %	AGAMREE 6 mg/kg/d (N=28) %	Placebo (N=29) %
Cushingoid features	7	29	0
Psychiatric disorders[1]	7	21	14
Vomiting	17	14	7
Weight increased	0	11	3
Vitamin D deficiency	7	11	0
Cough	10	7	3
Headache	7	7	3
Diarrhea	3	7	3
Increased appetite	3	7	3
Rhinitis	3	7	3

[1]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.

Catalyst pharmaceuticals

Distributed by Catalyst Pharmaceuticals, Inc., Coral Gables, FL 33134.

AGA-0109-2 April 2024

Progress Now

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

New approvals

New Treatment Approved for gMG

In April, the US Food and Drug Administration (FDA) approved Johnson & Johnson's nipocalimab-aahu (IMAAVY™) for the treatment of people ages 12 and older living with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody-positive. This is a significant advancement for the more than 100,000 people in the United States living with gMG, a chronic autoimmune neuromuscular disease.

IMAAVY™ is a neonatal Fc receptor (FcRn) inhibitor, designed to reduce the levels of pathogenic autoantibodies that drive MG. By targeting these autoantibodies, IMAAVY™ offers patients a targeted, effective, and potentially life-changing treatment option. Data from the pivotal, ongoing Vivacity-MG3 study support the FDA's approval of IMAAVY™.

While current treatments exist for gMG, many patients experience incomplete symptom relief or challenging side effects. IMAAVY™ approval expands the treatment landscape, offering a novel approach to reducing disease burden and providing a new option for those who have struggled with conventional therapies.

To learn more, visit imaavy.com. For more information on the ongoing Vivacity-MG3 study, go to ClinicalTrials.gov and enter NCT04951622 in the "Other terms" search box.

Image: iStock.com/name

Image: iStock.com/atakan

FDA Expands Use of Eculizumab (Soliris) to Pediatric MG

The FDA's approval of Alexion/AstraZeneca's eculizumab (Soliris) for adults with gMG who are AChR antibody-positive has been expanded to patients ages 6 and older. This landmark approval makes Soliris the first and only treatment available for pediatric patients living with gMG.

The expanded use of Soliris is supported by clinical trial data from adults with gMG, as well as data on drug safety and how it interacts with the body in pediatric populations. A 26-week study of 11 pediatric patients ages 12 to 17 demonstrated that adverse reactions to Soliris were consistent with those observed in adults.

Soliris was first approved in 2007 for the blood disorder paroxysmal nocturnal hemoglobinuria. It works by inhibiting the complement system, part of the body's immune system, to prevent it from damaging tissues. The treatment has since been approved for multiple blood disorders and autoimmune conditions besides gMG, .

To learn more, visit soliris.net/home.

Vyvgart Hytrulo Prefilled Syringe Approved

The FDA approved a new option for patients to self-inject efgartigimod alfa and hyaluronidase-qvfc (VYVGART® Hytrulo) with a prefilled syringe. The therapy is approved for the treatment of adults with gMG who are AChR antibody-positive

While the earlier version of VYVGART® Hytrulo requires administration by a healthcare provider, the new prefilled syringes allow the drug to be administered at home by a patient or caregiver after receiving instruction. According to the drug sponsor, argenx, this option enhances patient independence and reduces the time required for treatment.

VYVGART® Hytrulo is designed to reduce the circulating immunoglobulin G (IgG) autoantibodies in the bloodstream. By lowering the levels of these harmful antibodies, it may ease MG symptoms and overall disease severity.

To learn more, visit vyvgart.com.

CLINICAL TRIAL TERMS TO KNOW

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

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

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

Open-label: Participants know what treatment they are receiving.

Placebo-controlled: Some participants receive the treatment being tested and some receive a placebo that looks like the real treatment but has no active ingredients.

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

Image: iStock.com/DrAfter123

MDA research in action

Supporting Science and Education

In addition to providing a significant number of research grants each year to scientists working in the neuromuscular field, MDA offers conference grants to other organizations to support scientific or educational meetings related to neuromuscular diseases. The goal is to promote connections and knowledge sharing between researchers, clinicians, and the patient community. MDA's 2025 conference grants include:

2025 CMD Scientific & Family Conference
This conference brought together stakeholders in congenital muscular dystrophy (CMD), nemaline myopathy, and titinopathy, including researchers, clinicians, affected individuals and their families, industry representatives, advocacy groups, and government officials.

2025 RYR-1-Related Diseases Patient-Led International Research Workshop
This workshop united international experts in RYR-1-related diseases with affected individuals and family members to share knowledge, form collaborations, and develop strategies for finding therapies.

MDA's conference grants support scientific and educational meetings.

2025 CMTA Patient & Research Summit
The summit included presentations on living well with Charcot-Marie-Tooth disease (CMT) and updates on the Charcot-Marie-Tooth Association's Strategy to Accelerate Research (STAR) programs and initiatives, presented by CMT researchers.

Amyotrophic lateral sclerosis (ALS)

Drug Selected for Platform Trial

Image: iStock.com/Vitalina

Transposon Therapeutics announced that TPN-101 has been selected for testing in the HEALEY ALS platform trial, a groundbreaking research effort aimed at testing multiple therapies simultaneously to find effective treatments for ALS.

TPN-101 is an oral small-molecule drug designed to block an immune response that leads to inflammation and damage to nerve cells, which may contribute to the progression of ALS related to *C9orf72* gene mutations. The decision to include the treatment in the platform trial was based on its unique mechanism of action and final data from a phase 2 clinical trial. The trial involved 42 adults with ALS or frontotemporal dementia (FTD) related to *C9orf72* mutations. The results showed that treatment with TPN-101 significantly reduced the rate of disease progression compared to placebo.

HEALEY is the first ALS platform trial designed to accelerate the path to new ALS therapies by testing multiple treatments at once and comparing the results against a pooled placebo group. This reduces the cost of research, decreases the trial time, and allows more participants to be given an active drug rather than the inactive placebo.

MDA has supported the HEALEY ALS trial with a clinical research grant.

For more information on the phase 2 trial, visit ClinicalTrials.gov and enter NCT04993755 in the "Other terms" search box.

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

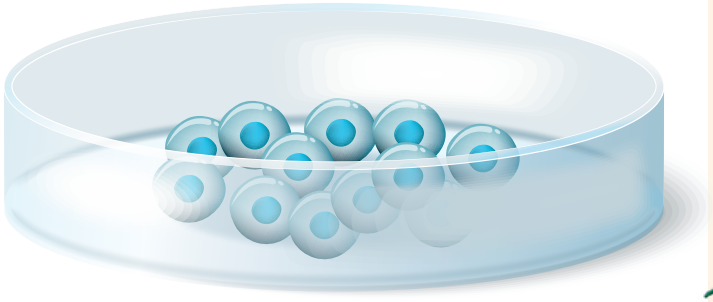


Image: iStock.com/ttsz

Amyotrophic lateral sclerosis (ALS)

Phase 3 Trial Opening Soon

A phase 3b clinical trial to test Brainstorm Cell Therapeutics' debamestrocel (NurOwn), a stem cell-based therapy for ALS, is expected to open soon at multiple sites across the US.

NurOwn aims to slow disease progression and potentially extend survival in people with ALS using a patient's own mesenchymal stem cells (MSCs), which are collected from their bone marrow. In a lab, the MSCs are turned into cells that can promote healthy nerve cell growth and survival. Then they are infused back into the patient.

In a phase 3 clinical trial of 189 adults with rapidly progressing ALS, NurOwn was not shown to significantly slow disease progression compared with a placebo, but a subset of patients with less advanced disease did appear to benefit from the treatment.

Brainstorm announced that it plans to recruit about 200 adults with moderate disease who have been experiencing symptoms of ALS for less than two years for the phase 3b trial, called ENDURANCE. The US Food and Drug Administration (FDA) has reviewed and agreed to the trial protocol, which allows Brainstorm to activate clinical trial sites quickly and potentially supports a future application for approval of NurOwn.

For more information, visit ClinicalTrials.gov and enter NCT06973629 in the "Other terms" search box.

Duchenne muscular dystrophy (DMD)

Positive Data From Gene Therapy Trial

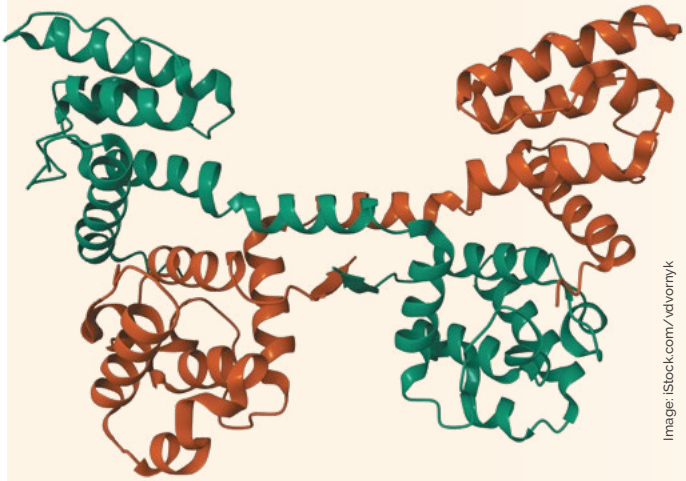


Image: iStock.com/vdvornyk

Regenxbio Inc. announced new positive interim data from a phase 1/2 clinical trial of RGX-202, an investigational gene therapy for DMD.

In DMD, a mutation in the dystrophin (*DMD*) gene prevents cells from making enough functional dystrophin protein, which muscle cells need to function and repair themselves. The AFFINITY clinical trial is testing the safety, tolerability, and efficacy of a one-time intravenous (into the vein) dose of RGX-202 in boys ages 4-12 with DMD. This therapy is designed to deliver a gene with instructions to make microdystrophin (a shortened but functional dystrophin protein) to muscle cells.

New findings suggest that RGX-202 positively impacts the disease trajectory, with participants receiving a specific dose level showing meaningful functional improvement. Regenxbio also reported that the therapy continues to be well-tolerated, with no serious side effects reported.

For more information, visit ClinicalTrials.gov and enter NCT05693142 in the "Other terms" search box.

New Therapy May Improve Muscle Repair

Image: iStock.com/Vladimir Kononok

A new oral small-molecule therapy for DMD, SAT-3247, is safe and shows early signs of increasing muscle strength in a phase 1b clinical trial, according to Satellos Bioscience Inc.

DMD impairs the production or function of dystrophin, a protein that helps protect muscles from damage during movement. In addition, the condition appears to disrupt the body's natural process for repairing muscle damage. SAT-3247 is designed to address progressive muscle loss in people with DMD by restoring muscle regeneration in response to damage.

In the open-label phase 1b trial, five adult male participants took SAT-3247 concurrently with standard-of-care corticosteroid therapy. Data show that average grip strength doubled across participants.

Satellos plans to advance SAT-3247 to a placebo-controlled phase 2 trial.

For more information, visit ClinicalTrials.gov and enter NCT06565208 in the "Other terms" search box.

SAT-3247 is designed to address progressive muscle loss in people with DMD by restoring muscle regeneration in response to damage.

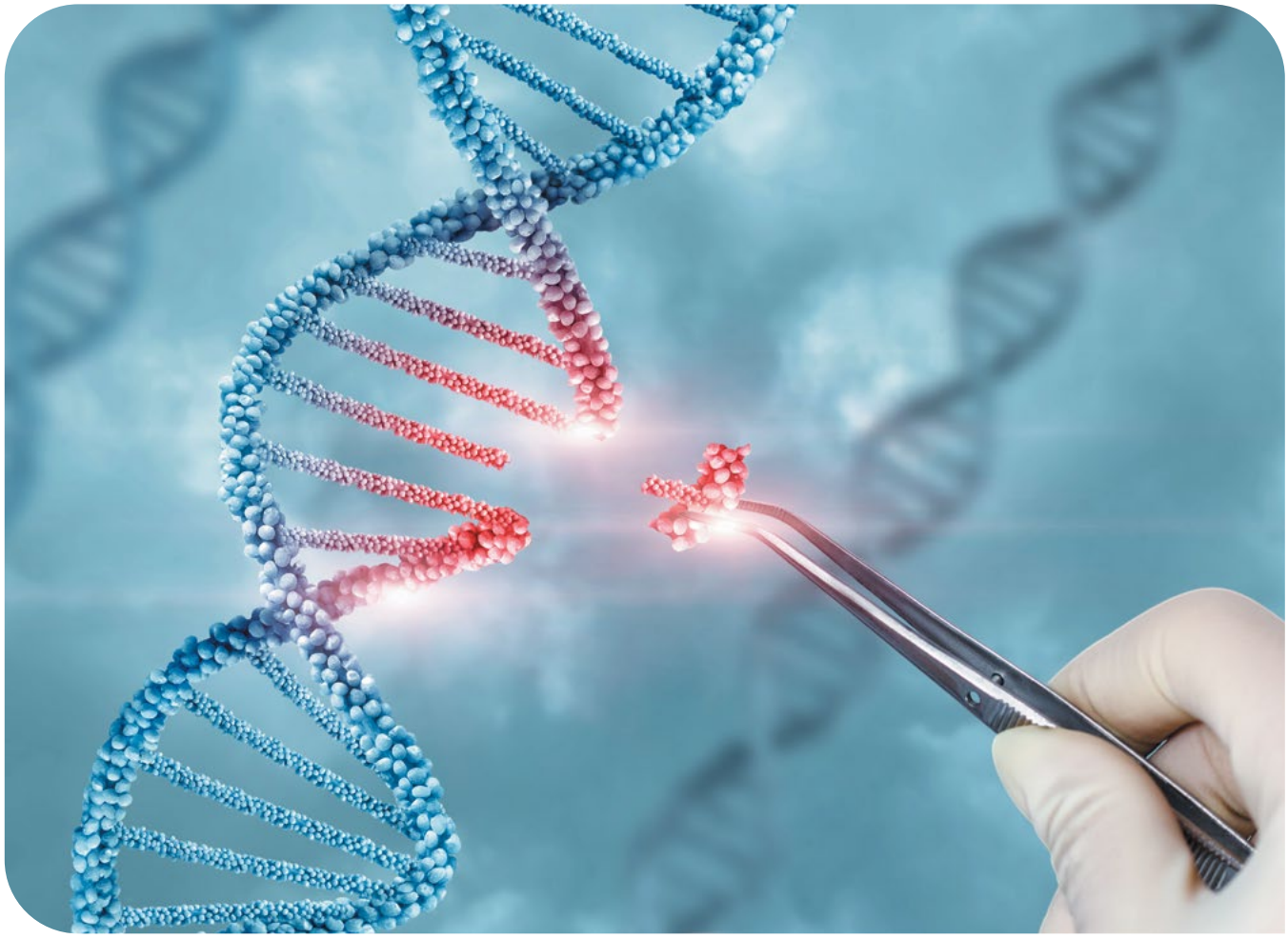


Image: iStock.com/Natali_Mis

Duchenne muscular dystrophy (DMD)

Support for Gene Editing Therapy

The US Food and Drug Administration (FDA) has granted rare pediatric disease designation to Precision Biosciences' PBGENE-DMD, an experimental gene-editing therapy for DMD.

Gene mutations that cause DMD occur in specific sections of the *DMD* gene, called exons. These exons are numbered, and more than 60% of people with DMD have mutations between exons 45-55.

PBGENE-DMD is designed to remove exons 45-55 with one administration, using an adeno-associated virus (AAV) to deliver the therapy to cells in the body. According to Precision, this approach will permanently edit the person's DNA sequence, resulting in naturally produced, functional dystrophin protein.

Gene mutations that cause DMD occur in specific sections of the *DMD* gene, called exons.

Rare pediatric disease designation incentivizes companies to develop treatments for rare and serious or life-threatening diseases affecting people under age 18. Precision may use this award to support a request for FDA priority review of PBGENE-DMD.

To learn more, visit precisionbiosciences.com.

Image: iStock.com/akinbostanci


WHERE anythi
2024

ng IS POSSIBLE

Former campers reflect on 70 years of MDA Summer Camp magic

BY KELLY BERGER

In 1955, MDA held its first Summer Camp in Sussex, New Jersey. Sixteen campers from New York boarded a bus and headed to a rustic yet accessible campsite, where they explored and built camaraderie, free from the typical physical and societal limitations.

One of the first to be led by a voluntary health organization, this innovative summer camp experience was offered exclusively for kids living with muscular dystrophy.

The pilot program was a success. MDA Summer Camp is now celebrating its 70th year. Today, at MDA's nationwide network of camps, youth with neuromuscular diseases can have traditional camp experiences — enjoying outdoor adventures, discovering new interests, and making lifelong friends — in a barrier-free environment. Many former campers credit their time at Summer Camp with helping them build the self-confidence and independence they need to take on everyday challenges well beyond their camp years.

MDA Summer Camp *is a week-long overnight camp for kids and young adults ages 8-17 who have been diagnosed with neuromuscular diseases.*

Camper Kelly Berger with counselor Tyler Snow in 2006

As one of those former campers, I have such fond memories of the five years I attended Summer Camp in Louisville, Kentucky. I'm 36, living with collagen VI muscular dystrophy, and I still reminisce about Summer Camp with the friends I made there more than two decades ago. It was a week where I lived freely, felt like a star, and celebrated my achievements, big and small. It was a welcome break from focusing on my physical limitations — at camp, anything is possible.

What's special about Summer Camp?

MDA Summer Camp is a week-long overnight camp for kids and young adults ages 8-17 who have been diagnosed with neuromuscular diseases. From the beginning, MDA has provided Summer Camp at no cost to families.

Each camp location is unique in its features and facilities, but all are safe and accessible for campers with neuromuscular diseases. In addition, volunteer medical professionals and counselors are trained to meet every child's healthcare needs and provide physical and emotional support, so campers can fully enjoy the camp experience.

"We are able to utilize equipment and technologies that are specifically designed for campers with disabilities to be able to enjoy the magic and freedom of being a kid at summer camp," explains Kelsie Andreska, MDA's Director of Recreation Programs.

This allows them to participate in quintessential summer camp activities, like fishing, swimming,

Summer Camp 2024

Virtual Summer Camp 2021

Sophia Dipasupil (front row, third from left) with camp friends in 2015

and horseback riding. But being at Summer Camp means so much more than trying archery or ziplining. It means getting to know your cabinmates and giving your cabin a creative nickname. It means showing your camp spirit with flags and spirit sticks, dressing up for theme days, and joining campfire sing-a-longs — with s'mores, of course.

Camper favorites vary from epic talent shows to Sponsor Days featuring exciting visitors to the unforgettable dance on the final night. Experiencing these activities while surrounded by other youth living with disabilities and embracing a new level of independence creates a magic that is unique to MDA Summer Camp.

Alicia Dobosz, MDA's Executive Vice President of Community Engagement, has witnessed first-hand how campers transform, even in the few short days after their arrival, learning to advocate for themselves and create new dreams for the future.

"This incredible change is undoubtedly due to the support, camaraderie, and connection that is almost palpable from the minute you enter camp," she says. "There's a certain magic about it that's hard to describe unless you've experienced it."

Sory Rivera (right) and a friend at a camp dance in the 1990s

The best week of the year

Campers, both new and seasoned, have one echoing parallel: Attending MDA Summer Camp is their favorite week of the year.

Sophia Dipasupil, 18, of Minnesota, who lives with juvenile dermatomyositis and attended Summer Camp for 10 years, found that "being around others who could understand me made it easy to be far from home. I got to experience things I never had the opportunity to do before, like ziplining, pontoon boat rides, and horseback riding," she says.

That rings loudly for Sory Rivera, 37, of Texas, a 17-year Summer Camp veteran who lives with spinal muscular atrophy (SMA). She adored the week-long getaway that brought her out of her shell. "I remember having the time of my life and bawling my eyes out when I had to leave," she says.

Brothers Caden Sanchez, 21, and Cody Sanchez, 18, of Wisconsin, who both live with LAMA2-related muscular dystrophy (LAMA2-RD), attended nine years of Summer Camp together. "It's a week to be free and have fun and not feel different than anyone else," Cody says.

Caden and Cody were often in different cabins, and one of their favorite activities was being rivals

Camper favorites *vary from epic talent shows to Sponsor Days featuring exciting visitors to the unforgettable dance on the final night.*

in adaptive sports like basketball or baseball. "We would always compete against each other's cabins and try to win. We were very competitive," Caden says.

The Sanchez brothers also enjoyed swimming, and Cody particularly liked building pinewood derby cars. He raced the finished cars in tournaments and received medals for winning. Both brothers looked forward to Sponsor Day, when local fire fighters would visit and play baseball with the campers.

Cody (left) and Caden (right) at camp with their sister Cassidy, a counselor, around 2015

Finding a community

For Caroline LeMay, 27, of Massachusetts, who lives with collagen VI muscular dystrophy and attended camp for eight years, her fondest Summer Camp memories involved bonding with her cabinmates and counselors. Those years were formative in building friendships with the women she calls sisters. "It was the first time I met people who could understand the disability experience," she says.

Caroline LeMay (standing, second from left) and friends dressed up for the camp dance in 2013

Even when camp friends are separated most of the year, the distance is no barrier to longevity, according to Sophia. "To this day, my friends from camp inspire me in so many ways. The memories we made together and the impressions they've made on my life will last forever," she says.

These strong friendships formed a secure base that helped her build self-confidence. "The sense of community I felt helped me realize that I'm not alone and that we are all stronger because of each other," she says. "It helped me see and believe that my opportunities and life experiences are not limited."

Building confidence

Feeling empowered seems to come naturally for campers when they are surrounded by others like them.

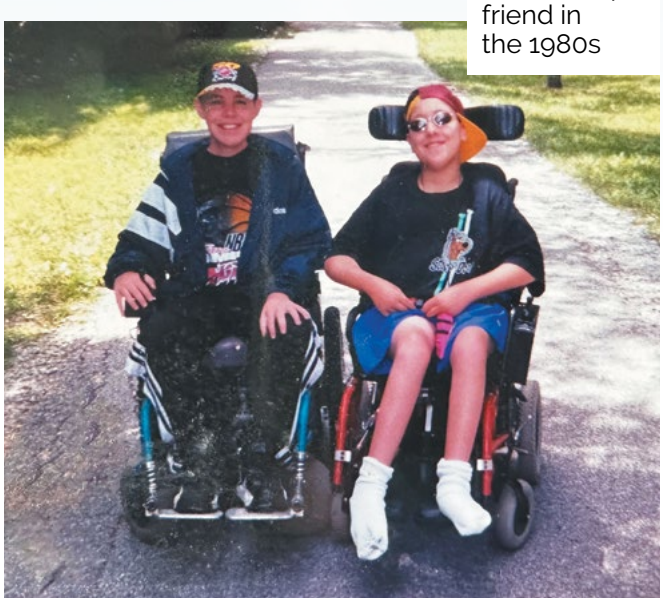
To Sory, Summer Camp was a safe place where she fully embraced herself. "If I hadn't gone to MDA Summer Camp, I wouldn't have blossomed in the way I have with my confidence," she says.

At Summer Camp, for the first time, Sory didn't feel like her 400-pound power wheelchair was a barrier to inclusion. She participated in fishing, boating, and even climbed a three-story

wall — all activities she couldn't do outside of camp. Sory still remembers the tough, brave kid she was at camp and uses that to carry her through adverse times in her adult years.

Isaac Banks, 41, of Illinois, who lives with limb-girdle muscular dystrophy (LGMD) and attended camps for 10 years, claims his first camp fears easily faded once he was surrounded by people like him. "No worries about people staring at my wheelchair — we were all in one. No concern about not being able to play the games — they were all designed for our needs," he says. "My first time going to Summer Camp made me realize there is a big world, and there is room for me in it."

Summer Camp is where Isaac first saw others in wheelchairs successfully navigating life with disabilities. Eventually, he became one of those influential camp veterans. "My desire to be a motivational speaker came from talking with the younger kids as I tried to motivate them for greatness the way that I was motivated," Isaac says. "I can honestly say that Summer Camp is the reason I am who I am."



Top: Camp group photo
Bottom: Isaac Banks (left) and a camp friend in the 1980s

Volunteers feel the magic

The magic of Summer Camp would not be possible without the generous efforts of volunteers who make camp a reality year after year.

"The sense of **community** *I felt helped me realize that I'm not alone and that we are all stronger because of each other. It helped me see and believe that my opportunities and life experiences are not limited."*

— SOPHIA DIPASUPIL

Many volunteers, moved by this transformative week, say they get as much out of the experience as the campers themselves.

"Medical team members tell us that it changes the way they provide care and interact with their patients," Alicia says. "Some volunteers choose new career paths based on their time at camp. It's life-changing for all involved."

Tyler Snow, 45, of Indiana, who volunteered as a counselor for more than 16 years, credits Summer Camp with giving him a new perspective on life.

"My main job was to make sure the campers had the best week of their lives," he says. He would often go above and beyond to entertain the campers in his care. Whether it was taking a whipped cream pie to the face or stuffing his mouth with marshmallows for a "chubby bunny" challenge,

A Day at MDA Summer Camp

Each day at MDA Summer Camp is carefully crafted to give campers of all ages and abilities opportunities to try something new, participate in team building with counselors and fellow campers, and bond with cabinmates. Here's an example of what can happen in a day at Summer Camp.

MORNING

Breakfast & Announcements
Cabins gather in the main dining area for the first meal of the day. Camp leaders share camp news and announce upcoming activities.

Fishing or Archery
Cabins are grouped together to head to the ramped boat dock to cast their lines or venture to the archery range to see who can hit the bullseye.

AFTERNOON

Lunch & Announcements
Cabins return to the main dining area. Camp leaders share any weather updates that may alter planned activities.

Rest Time
Campers head back to their cabins to rest or hang out with their cabinmates out of the summer sun and heat.

Swimming or Arts & Crafts
Campers may cool off in the accessible swimming pool or gather in a craft cabin to make friendship bracelets, tie-dyed T-shirts, or nature paintings.

he embraced it, so campers could leave with epic stories to tell and photos to show all the fun that took place.

"Camp fills my cup, fills my soul, and revives my faith in humanity," says Naomi Sullivan, RN, 45, of Illinois, a 12-year camp medical staff volunteer. Summer Camp has reminded her not to take life or her abilities for granted.

Volunteer Naomi Sullivan (back row, second from right) joined campers for a good prank in 2023

Alexander Fay, MD, PhD, 49, a pediatric neurologist in San Francisco, started volunteering at Summer Camp because he wanted to learn what his patients could do for fun outside of a clinical setting. He was moved by the selflessness of the counselors and the joy created at camp and went on to serve for five years as a camp doctor at multiple locations. "I try to carry the spirit of MDA Summer Camp with me through the rest of the year, because it reminds me that our calling is to serve each other," he says.

EVENING

Dinner & Announcements
Cabins regroup in the main dining area. Camp leaders make their last announcements about evening activities and what's to come the next day.

Talent Show
In a night of friendly competition and connection, cabins may band together to perform a song or dance, or campers cheer on friends doing karaoke, juggling, or even a comedy or magic set — the creativity soars!

Fireside Hangout
Campers gather for s'mores, sing-alongs, and storytelling. It's a cozy way to end the night after a long day of activities.

Curfew & Lights Out
Campers wind down in their cabins, surrounded by newfound friends, dreaming of the next day's antics.

Adapting to the changing landscape

As the world has changed in the last 70 years, MDA Summer Camp has evolved with it, incorporating new programming and exciting sponsors, enhancing safety protocols and policies, and, most importantly, expanding access to more youth with neuromuscular diseases.

Since 2020, MDA Virtual Summer Camp has provided an engaging and meaningful experience for kids and young adults to enjoy the feeling of Summer Camp from the comfort of their homes. Virtual campers join in on arts and crafts, STEM projects, and cooking activities and spend time connecting with their fellow campers and volunteers during Cabin Chat video calls.

While much has changed over the decades, one thing remains constant: Summer Camp is a place where campers find joy, independence, and newfound freedom as they try new things, forge new friendships, and embrace a week where the world is fully accessible.

For those of us who have had the pleasure of experiencing Summer Camp, the spirit of camp lives on forever in our hearts — a cherished time of camaraderie, connection, and maybe even some pranks. Q

+FEEL THE MAGIC
To learn more about becoming a camper or volunteer, visit **mda.org/SummerCamp**.

Kelly Berger lives in Cincinnati and freelances in writing and digital marketing. She's an avid music festival and concertgoer. Follow her journey at **thekellyberger.com**.

The Best Week of the Year

A look at MDA Summer Camp through the eyes of the campers and volunteers who are transformed by it

2025

2005

"*For those of us who have had the pleasure of experiencing Summer Camp, the spirit of camp lives on forever in our hearts.*" — KELLY BERGER

2017

1986

"*At camp, I no longer felt alone because I was surrounded by a welcoming, supportive community. They taught me that I can be proud of my disability and achieve the same things that others can.*" — SOPHIA DIPASUPIL

Circa 1975

"My years at MDA Summer Camp are among my fondest memories. I even wrote my college admissions essay about people I met at camp who helped me discover new insights about myself."
— CAROLINE LEMAY

"Camp changed me for good and for the better." — NAOMI SULLIVAN

Circa 1970

2024

"Summer Camps were a highlight of my youth, providing a space where I felt truly understood and accepted." — SORY RIVERA

"I found my people. I finally had a community that I was able to fully embrace, connect, and enjoy life without judgment from anyone else in the world."
— OLIVIA HOLLER

1995

Edgewise® THERAPEUTICS
Leaders in Muscle Disease Science

What is the North Star Ambulatory Assessment?

The North Star Ambulatory Assessment (NSAA) is a widely used tool that measures changes in muscle function and mobility in individuals who are able to walk (with or without assistive devices).[1,2] It can also be used in clinical trials to measure a person's functional abilities before and after receiving an investigational drug to assess the potential impact of the therapy.[1,3]

NSAA Activities[2]

- Hop left leg
- Hop right leg
- Stand on Heels
- Rise from Floor
- Run
- Jump
- Lift Head
- Descend box step (L)
- Descend box step (R)
- Climb box step (L)
- Climb box step (R)
- Stand one leg (L)
- Stand one leg (R)
- Get to sitting
- Rise from chair
- Walk
- Stand

The NSAA helps healthcare providers understand how diseases affect movement.

The **NSAA** helps healthcare providers understand how diseases, like Becker muscular dystrophy (Becker), affect movement by regularly assessing a set of physical activities (like climbing steps, or getting up from a chair) that are needed to perform everyday activities.[4,5] It can also be helpful for an individual living with Becker to better understand their disease and notice how their abilities related to certain activities may change over time. Each activity is scored on whether it can be completed:

+2 points: With no adaptation (activity can be completed with no assistance or change to how it was previously done)

+1 point: With an adaptation due to weakness (activity is still possible, but requires assistance or a change, such as using hands to stand up)

0 points: Not at all (activity cannot be completed)

The maximum score is **34 points**. A lower score indicates Becker is having an impact on movement in daily activities.[2]

TO LEARN MORE ABOUT THE NSAA and how it is used in clinical research and ongoing care and support, watch this short, animated video on the Edgewise YouTube Channel.

FOR MORE INFORMATION ABOUT EDGEWISE, our development pipeline, and our efforts in the community, visit us online or reach out to us at studies@edgewisetx.com

1. Scott E, et al. Physiother Res Int. 2012;17(2):101-9. **2.** Mercuri E, et al. PLoS One. 2016;11(8):e0160195.
3. Mazzone ES, et al. Neuromuscul Disord. 2009;19(7):458-61. **4.** Lowes L, et al. Poster presented at World Muscle Society; Oct 2024. #322P.
5. Bello L, et al. Sci Rep. 2016;6:32-39.

The NSAA is also used as a tool to assess changes in a disease over time.

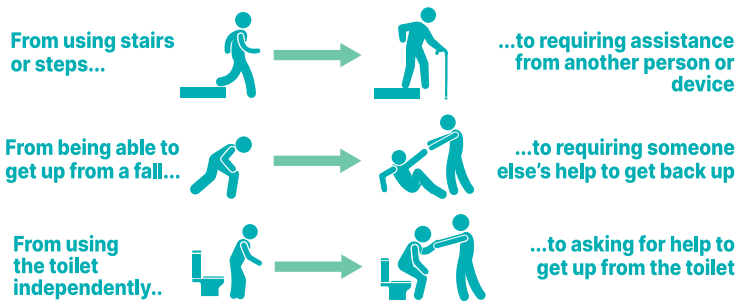
Becker is a genetic disorder causing muscle damage that can impact everyday life.[1] The natural history of Becker (health information gained from monitoring a group of individuals living with Becker with no intervention over a longer period of time) uses the NSAA to track functional decline and understand how the disease progresses.[2-7]

In individuals with Becker, natural history studies have observed an average NSAA score decline of 0.9 - 1.7 points every year.[2,3,4,5] This decline in function can begin at any age and continues throughout the lifespan.

- For example, in one study almost half of those living with Becker lost the ability to run in their early 30's[2]
- In another study, some people living with Becker started using a wheelchair, either part-time or full-time, in their mid 30's[6]
- Several studies suggest about 30-40% of individuals living with Becker were unable to walk at all in their 40's and 50's[2,7]

This change in physical ability is demonstrated by the individual's change in score using the NSAA.

Day by day, there may not be a noticeable change, but over a year, the difference can become more apparent. Losing 1 or 2 points can have a big impact, like being able to climb up a step or a curb or rise from a chair.



The NSAA is also used in Becker research and clinical trials. It is considered a "clinically meaningful outcome measure," which means it can demonstrate a real, important change in a someone's health or ability that matters in their daily life.[8]

When the NSAA is used in clinical trials, it can help to check if someone fits the physical ability requirements to enroll in the trial. It can also be used throughout the trial period to determine if physical ability is improving, staying the same, or declining. This can then be compared to natural history studies. This comparison can indicate how the therapy may or may not be changing the course of the disease compared to if there was no intervention.

Learn More

FOR MORE INFORMATION ON BECKER MUSCULAR DYSTROPHY, building your care team, and other resources to help manage the disease, visit beckermusculardystrophy.com

TO SEE HOW THE NSAA WAS USED TO MEASURE IMPACT IN THE EDGEWISE GRAND CANYON STUDY, view this webinar hosted by the Muscular Dystrophy Association featuring Tina Duong, MPT, PhD from Stanford University and Joanne Donovan, MD, PhD, Chief Medical Officer at Edgewise.

> **"Having to avoid steps and stairs now keeps me from visiting family and friends."**
>
> -Individual living with Becker

> **"Struggling to get up from a chair or toilet in a restroom has created anxiety. I am now reluctant to go to restaurants, movies, and extra worried if I need to use the restroom."**
>
> ~ Individual living with Becker

1. Magot A, et al. J Neurol. 2023;270(10):4763-4781. **2.** Bello L, et al. Sci Rep. 2016;6:32-39. **3.** De Wel B, et al. Eur J Neurol. 2024;31(7):e16282.
4. Van de Velde NM, et al. Neurology. 2024;97(5):e513-e522. **5.** Bello L, et al. Poster presented at World Muscle Society; Oct 2024. #358P.
6. Nakamura A, et al. Ann Clin TransINeurol. 2023:10(12);2360-2372. **7.** Clemens PR, et al. Muscle Nerve. 2020:62(3);269-276.
8. Lowes L, et al. Poster presented at World Muscle Society; Oct 2024. #322P

For 75 years, MDA has been dedicated to standing by your side, offering hope, connection, and community. From the earliest days, our mission has been centered around ensuring that you have the resources, care, and support you need to live stronger and more independently.

How we accomplish our mission has evolved through the years — as have so many aspects of life in the last three quarters of a century. What hasn't changed is our commitment to supporting and empowering you, our community, to navigate the complexities of living with a neuromuscular disease.

"When I think of MDA, I think of hope," says Lily Sander, an MDA National Ambassador from Charlotte, North Carolina, who lives with Charcot-Marie-Tooth disease (CMT). "It's a powerful hope for brighter days where treatments, cures, and an end to these conditions are not just dreams, but a tangible reality. MDA embodies this hope by focusing on advancements today that lead to breakthroughs tomorrow. But their impact goes beyond research; they also provide vital patient support and everything in between, ensuring that families receive the care and resources they need right now."

Here, we take a look at the many MDA programs, services, and resources that directly impact members of our community, and how we meet you wherever you are in your neuromuscular disease journey.

Specialized neuromuscular care

MDA Care Centers started in 1953 with two locations in New York. Now, the MDA Care Center Network (**mda.org/CareCenters**) comprises more than 150 Care Centers providing comprehensive, specialized neuromuscular care at top medical institutions nationwide.

From the early days, MDA recognized the importance of coordinated care, bringing together experts in various fields to offer patients a holistic approach to treatment. This approach is called multidisciplinary care.

Research shows that multidisciplinary care is especially beneficial for people with rare diseases and complex care needs. Bringing together a range of skills and knowledge improves the quality of care and provides patients and their families with more resources and support.

It also adds convenience. "Multidisciplinary care means one journey, one team, one place," says Nora Capocci, MDA's Executive Vice President of Healthcare Services. "By bringing multiple specialists together under one roof, we turn a series of appointments into a single, supportive experience — giving families more time for what truly matters."

Healthcare providers also appreciate the opportunity to coordinate and collaborate on their patients' care. "MDA has provided the opportunity to engage in a multidisciplinary team approach," says Mercedes Medeiros, a social worker at the MDA Care Center at Rhode Island Hospital in Providence, Rhode Island. "This allows us to address not only our patients' neuromuscular needs, but their basic needs as well. We also have access to a local representative who is knowledgeable regarding MDA resources available to our patients."

Through personalized care plans, access to clinical trials, and state-of-the-art treatments, MDA Care Centers have dramatically improved patient outcomes. Patients benefit from the expertise of leading clinicians and the support of a multidisciplinary team dedicated to their well-being. The network has become a lifeline for families, offering hope, continuity of care, and the latest advancements in treatment.

MDA's community programs support and empower people in every stage of life with a neuromuscular disease

BY AMY BERNSTEIN

Full HOPE

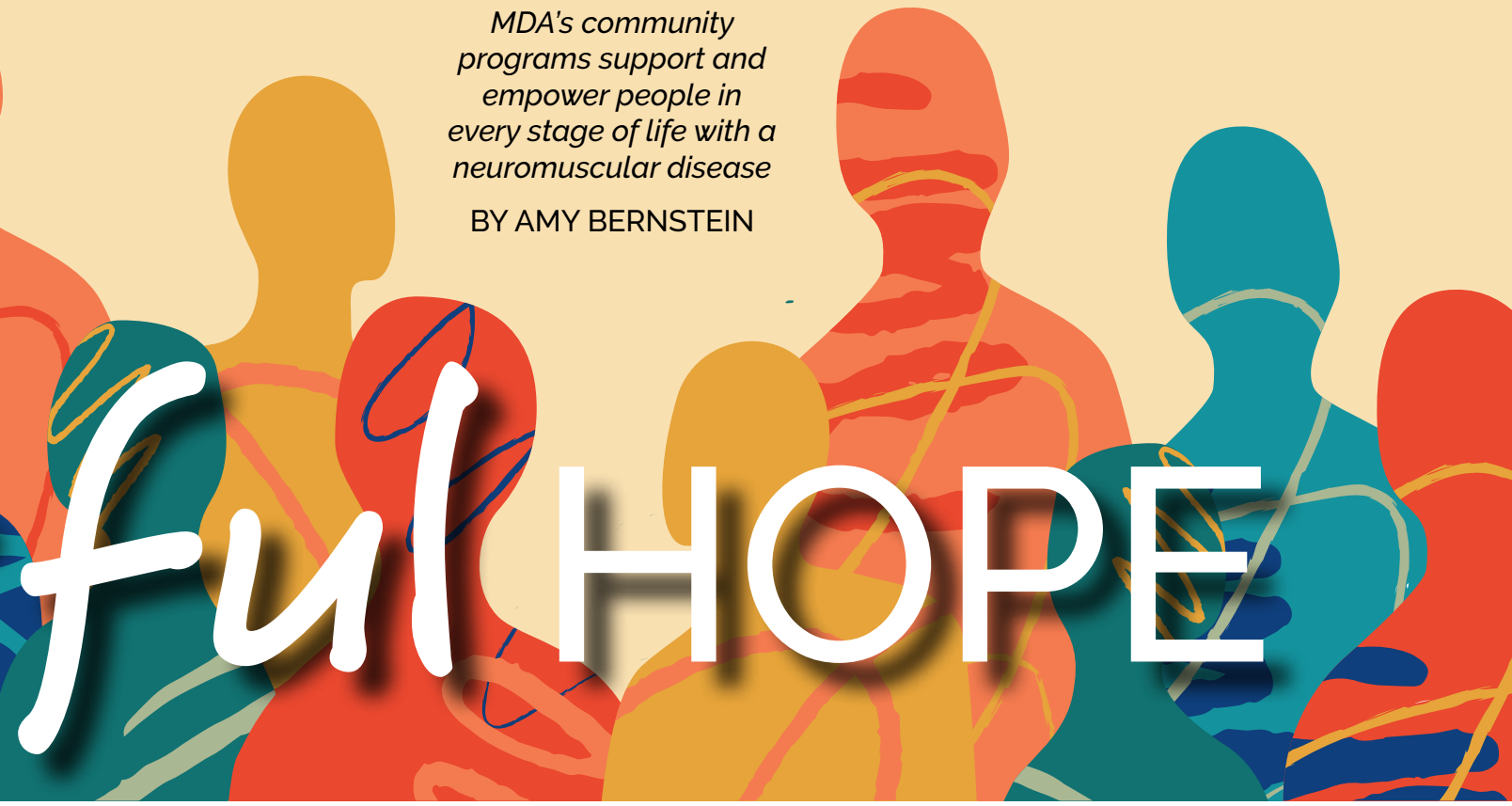


Image: iStock.com/Pandagolik

"The clinics are a lifesaver, having all the people on your care team in one place. It can be isolating having muscular dystrophy. Knowing that there is a whole organization that has your back really helps." — *Adrian, Becker muscular dystrophy (BMD), Rutland, VT*

"I was able to get a clearer understanding of my diagnosis and speak with doctors who knew and understood my concerns and what I was going through," says Henryne Dillard, who lives with limb-girdle muscular dystrophy (LGMD) in Stockton, California.

Moving into the future, MDA's Care Center Network will continue to lead the way in providing access to exceptional care that adapts to the needs of the neuromuscular community.

Support on your journey

We know that families across the country rely on MDA for guidance, support, and access to vital services, so we offer several ways 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.

The Resource Center (**mda.org/Resource Center**) is known as the "front door to MDA."

"MDA has been my source of hope, helping me thrive with my neuromuscular condition. Their support has guided me through challenges and helped me become the best version of myself." — *Ira, spinal muscular atrophy (SMA), West Palm Beach, FL*

It helps individuals, family members, and caregivers find answers to their questions and discover MDA programs and local resources. The Resource Center is staffed by a team of dedicated and caring staff who are available to assist by phone or email.

MDA Connect (**mda.org/connect**) gives community members the opportunity to have a 30-minute one-on-one video call with an MDA Support Specialist. These specialists can provide information on education, careers, accessibility, caregiving, transportation, community engagement, general disease education, and more. MDA also has Support Specialists with experience living with neuromuscular diseases and healthcare backgrounds who can help individuals and families navigate MDA Care Center visits.

MDA's Gene Therapy Support Network (**mda.org/GeneTherapySupport**) offers education and support to community members who are eligible to receive gene therapy or who want to learn more about it. Gene Therapy Support Specialists can help you access gene therapy or navigate insurance, answer general questions, and share resources. You can connect with a Gene Therapy Support Specialist by phone, email, or video call.

Whether through our Resource Center or online support, we've helped countless people manage their conditions and find hope in the face of challenges. Our resources have empowered families to make informed decisions and take control of their healthcare journeys.

"MDA meets people where they are and has a variety of options, so they don't need to navigate things alone," says Alicia Dobosz, MDA's Executive Vice President of Community Engagement.

Practical, actionable education

"Community Education is a staple of MDA because it embodies MDA's mission, which is to support the independence of those we serve," says Marissa Lozano, MDA's Director of Community Education (**mda.org/community-ed**). "That's exactly what our programs and print materials do — give people tools and resources to help them feel empowered."

Offerings range from in-person symposiums to online webinars and workshops to printable fact sheets. They span disease-specific topics and new therapies, as well as issues related to daily living, social-emotional well-being, caregiving, and more. All are offered at no cost.

Program speakers include clinicians, healthcare providers, and researchers who are leaders in the neuromuscular field, as well as advocates, community members, and others who understand the experience of living with or caring for someone with a neuromuscular disease.

"Informing people and empowering them to make decisions in their care and in their lives is a big part of Community Education. It's not just the information, but what you can do with that information," Marissa says.

In addition to providing timely, actionable information, many Community Education programs create opportunities for community members to meet and connect. Marissa points to the Next Steps Seminars (**mda.org/seminars**), which focus on navigating major life transitions, as an example. "Our Next Steps Seminars are not your traditional webinar, where you just listen to a presenter," she says. "We encourage people to unmute, turn on their cameras, and interact. You get to have conversations and share resources."

Conversations between community members are a valuable aspect of in-person programs (**mda.org/symposiums**). "We had a family who traveled from Ohio to Atlanta for one of our in-person Engage Symposiums, and it was the first time their son met somebody with the same diagnosis. It was extremely powerful," Marissa says. "I think that's where the impact of education comes in."

"I attend every MDA webinar that I can to learn from all the expertise of the presenters as well as the experience of the participants. During one MDA webinar, I learned of a research team and reached out to them. I am now part of their research to determine the LGMD subtype that I have." — *Michael, limb-girdle muscular dystrophy (LGMD), Chatham, NJ*

"MDA has impacted me in so many ways. They were a guiding light when I first got my diagnosis. They provided multiple opportunities for me to immerse myself in advocating not only for myself, but for many others living with neuromuscular diseases. Having the great honor of serving as an MDA National Ambassador for two years was truly life-changing for me. I grew more in confidence and community than I ever had before." — *Amy, Bethlem myopathy, Noblesville, IN*

Raising our voices

"MDA has always excelled at elevating awareness for neuromuscular diseases," says Paul Melmeyer, MDA's Executive Vice President of Public Policy and Advocacy.

This legacy began in 1950, when a group of families affected by muscular dystrophy established MDA. It continued with the MDA Labor Day Telethon, hosted by the legendary Jerry Lewis from 1966 to 2011. Today, it is carried on by MDA's Public Policy and Advocacy team (**mda.org/advocacy**), which works tirelessly to champion issues important to people with neuromuscular diseases. Along with promoting access to care, disability policy, and accelerating drug development, the team also organizes and empowers MDA's grassroots advocates. These community members contact lawmakers and work in their neighborhoods to raise awareness of issues that affect the neuromuscular disease community.

MDA's advocacy has led to increased research funding for neuromuscular diseases and

innovations in drug development for rare diseases, as well as important legislation, such as the MD-CARE Act, Newborn Screening Saves Lives Act, ACT for ALS, and accessible air travel reforms in the Federal Aviation Administration (FAA) Reauthorization Act.

"None of these accomplishments would have been possible if it wasn't for our advocates," Paul says. "Members of our community are leading the way with their voices. It's their experiences that drive lawmakers to enact transformative policy reforms."

That's why MDA Ambassadors (**mda.org/ambassadors**) are an essential part of the effort to raise neuromuscular disease awareness. Every year, MDA selects two National Ambassadors from the hundreds of general MDA Ambassadors who share their stories and perspectives at MDA events and on platforms such as Quest Media (**MDAQuest.org**).

"It's the people who are delivering those stories that actually make things change," Paul says.

"When I first think of MDA, I think of a community of people who live with neuromuscular diseases, like me, but also as a place where there is empathy and respect for me. As a kid, I used to be afraid of my disabilities because I was bullied. Five years ago, I became an MDA Ambassador, and never did I imagine how many doors it would open for me. Probably the biggest door MDA opened for me was the door of independence."
— Rodrigo, congenital muscular dystrophy (CMD), Dallas, TX

Empowering lives

The impact of MDA's recreation programs is evident in the way they transform lives. Kids who go to MDA Summer Camp gain lifelong friends. MDA College Scholarship winners pursue their academic dreams. Young adults in MDA's Mentorship Program gain confidence and discover new opportunities.

MDA Summer Camp (**mda.org/SummerCamp**), the first program of its kind, started in 1955 to give youth with neuromuscular diseases a true camp experience. (Turn to page 16 to learn more.)

"MDA Summer Camp is a perfect example of a program that equips individuals to live independent lives," Alicia says. "Spending a week at camp and trying new things while learning how to advocate for their needs with new caregivers — every aspect of the program is designed to build confidence, expand thinking, and create opportunities to feel excited about the future. This beloved program is life-changing for all involved and is central to our mission."

In recent years, MDA has created new programs to support young people with neuromuscular diseases beyond the camp years.

"Given the immense progress made in the last decade, many individuals are pursuing higher education, careers, and independent living," Alicia says. "We created programs that not only financially support the cost of higher education but provide additional support for people pursuing their dreams."

The MDA College Scholarship (**mda.org/scholarship**) program grants merit-based scholarships of up to $5,000 to students living with neuromuscular diseases who demonstrate leadership qualities. Scholars are eligible to apply for additional support for up to four years. In 2024, MDA provided scholarships to 10 promising students.

Individuals with disabilities are often underrepresented in career fields. Yet, research shows diversity in the workforce drives innovation. MDA's Mentorship Program (**mda.org/mentorships**) aims to increase the number of people living with neuromuscular diseases in the workforce by connecting youth to mentors in a variety of fields. These five-week virtual programs are open to young adults with neuromuscular disease ages 14-21. An application is required, but there is no cost to participate.

"The Mentorship Program connects individuals with peers and mentors who have navigated similar journeys and exposes youth to a variety of career options to consider," Alicia says. "It's powerful to have a role model who can help you see yourself in a career."

The ripple effect of these programs extends beyond individual participants to strengthen families and communities.

Building community

Community members formed MDA, and we still consider it our mission to foster connections and create a sense of belonging among people living with neuromuscular diseases. That's why we've developed programs that bring individuals and families together to have fun, like Let's Play and Family Getaways.

Let's Play (**mda.org/lets-play**) is an online community for gaming and camaraderie. The centerpiece of Let's Play is a custom Discord channel, a group messaging platform popular among youth and the online gaming community. Through the Discord channel, individuals, families, and MDA supporters can play games together and interact. Let's Play also has a channel on Twitch, a live-streaming platform, where gamers share their gameplay, or people can gather virtually for special events like movie nights, talent shows, and more. It's a safe and welcoming place to play, connect, and make friends.

Family Getaways (**mda.org/family-getaways**) were created to offer an accessible outdoor recreation experience for families to enjoy together. Provided at no cost, this program offers a weekend for families to spend time in nature, participate in activities, and gather around a campfire with other MDA families.

"Summer Camp is an invaluable experience and core program for MDA, but we felt that we could expand the impact of a recreation experience to engage the whole family," Alicia says.

MDA also recognizes that our community members are a vital source of support for one another. The MDA Peer Connections Program (**mda.org/peer-connections**) helps members of

"MDA has impacted my family in a positive way. I gained lifelong friends and a support system. MDA has helped me embrace my disability to my fullest capacity."
— Olivia, CMD, Liberty, MO

MDA recognizes that our community members are a vital source of support for one another.

the neuromuscular disease community build bonds with one another — across the country or in their neighborhoods. By request, MDA Support Specialists will make introductions between individuals with similar diagnoses or interests, and the individuals can decide when and how to connect. The program is open to individuals with neuromuscular diseases or their caregivers, parents, spouses, or siblings and has made more than 300 connections since it started.

MDA Community Support Groups (**mda.org/CommunityGroups**) are supportive online groups that provide safe spaces to interact, gather resources, and exchange valuable information with others in the neuromuscular disease community. MDA has established several groups for people in specific circumstances, such as parents of kids with neuromuscular diseases and families living with ALS.

MDA's community programs have always been about more than support; they've been about fortifying families.

Pursuing progress

Looking ahead, MDA is expanding and enhancing our resources to meet the evolving needs of the community. We're continuing to invest in digital platforms, virtual support networks, and innovative outreach programs to ensure that every family, regardless of location, has access to the care and support they need.

"Our programming truly is constantly evolving to ensure we are offering a variety of options and connection points for all individuals," Alicia says.

MDA National Ambassador Ira Walker, who lives in Florida with spinal muscular atrophy (SMA), says it best: "When I think of MDA, the first thing that comes to mind is community. MDA is truly the community that those with neuromuscular conditions need. It's the community that listens, understands, and unites us while propelling us toward our very best days." **Q**

Amy Bernstein is a writer and editor for Quest Media.

Every Step of the Way

How MDA meets you where you are

While each neuromuscular disease journey is unique, they all have one thing in common: MDA can be an invaluable guide and resource. Here are some of the many ways MDA touches lives at different points in the journey.

FINDING THE RIGHT CARE

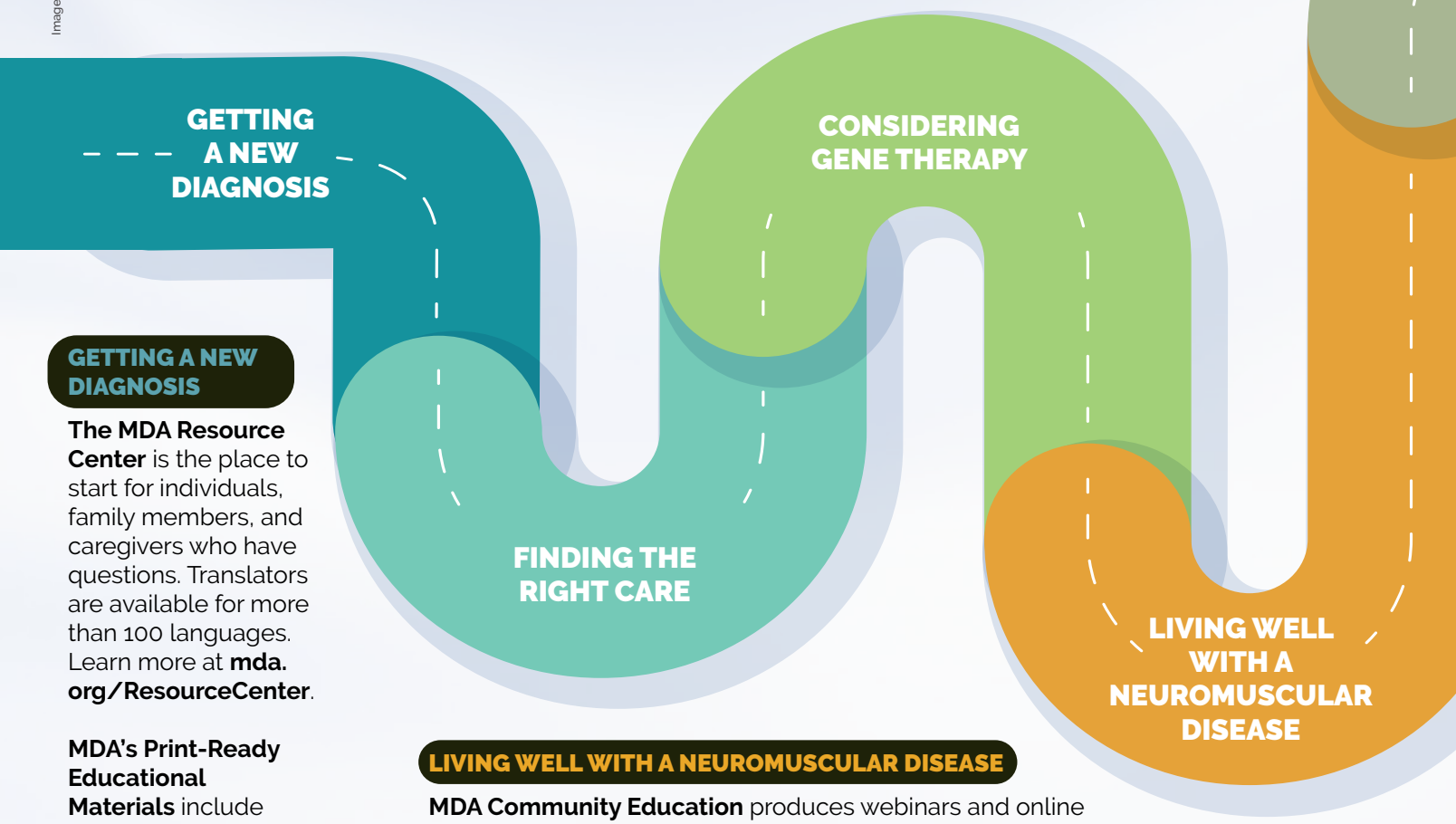
MDA Support Specialists can assist with finding MDA Care Centers and navigating visits. You can schedule a video call or an in-person meeting at an MDA Care Center. Learn more at **mda.org/connect**.

Access Workshop: Access to Medical Care is an engaging online learning module that covers the essentials of neuromuscular care. Find it at **mda.org/AccessWorkshops**.

CONSIDERING GENE THERAPY

The Gene Therapy Support Network offers education about gene therapy and support to anyone eligible to receive a gene therapy. Visit **mda.org/GeneTherapySupport**.

Gene Therapy Support Specialists are available for video calls, phone calls, or emails to talk about anything gene therapy-related. Learn more at **mda.org/GeneTherapySupport**.



GETTING A NEW DIAGNOSIS

The MDA Resource Center is the place to start for individuals, family members, and caregivers who have questions. Translators are available for more than 100 languages. Learn more at **mda.org/ResourceCenter**.

MDA's Print-Ready Educational Materials include Disease Fact Sheets and helpful guides on topics related to neuromuscular diseases. Visit **mda.org/education**.

LIVING WELL WITH A NEUROMUSCULAR DISEASE

MDA Community Education produces webinars and online workshops on topics of daily living, social-emotional well-being, and caregiving. To find programs, visit **mda.org/community-ed**.

MDA's Mental Health Hub provides mental health resources tailored to individuals with neuromuscular diseases and their families and caregivers. Visit **mda.org/MentalHealth**.

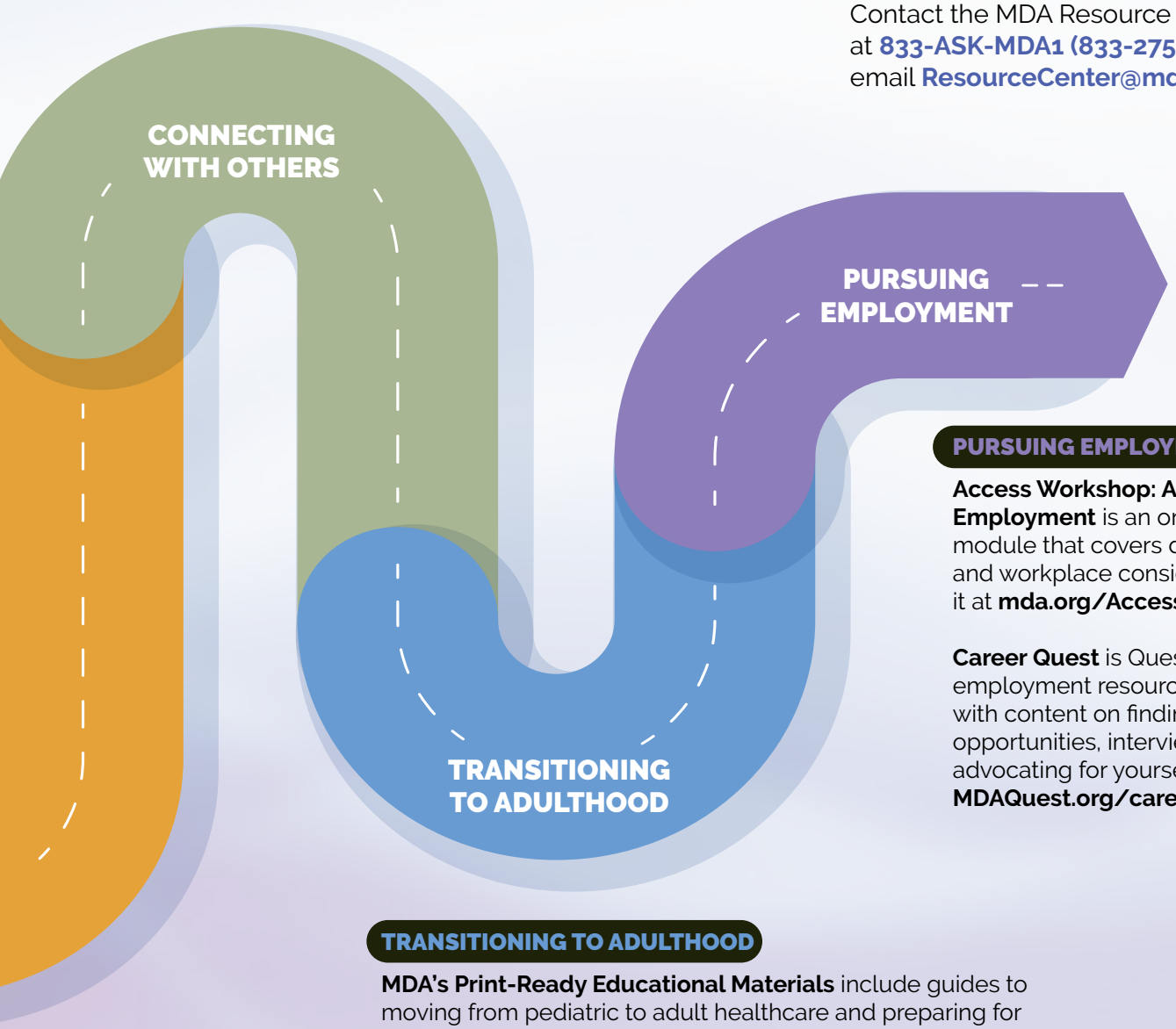
CONNECTING WITH OTHERS

Family Getaways are group experiences at accessible destinations, provided at no cost, for families affected by neuromuscular diseases. Learn more at **mda.org/family-getaways**.

Community Support Groups are safe places to get to know others with similar experiences and learn from each other. Visit **mda.org/CommunityGroups**.

+HAVE QUESTIONS?

MDA's Resource Center can provide support, guidance, and resources at every stage of your journey. Contact the MDA Resource Center at **833-ASK-MDA1 (833-275-6321)** or email **ResourceCenter@mdaUSA.org**.



PURSUING EMPLOYMENT

Access Workshop: Access to Employment is an online learning module that covers disability rights and workplace considerations. Find it at **mda.org/AccessWorkshops**.

Career Quest is Quest Media's employment resource hub, with content on finding job opportunities, interviewing, and advocating for yourself. Visit **MDAQuest.org/career-quest**.

TRANSITIONING TO ADULTHOOD

MDA's Print-Ready Educational Materials include guides to moving from pediatric to adult healthcare and preparing for college. Select "Transition to Adulthood" at **mda.org/education**.

Next Steps Seminar: Transition to Adulthood is a live, interactive online seminar for people ages 14-26 living with neuromuscular diseases. Learn more at **mda.org/seminars**.

SPONSORED CONTENT

Disease Doesn't **Define You**

Kamiron Kraft, 21, lives with thymidine kinase 2 deficiency (TK2d), a rare genetic mitochondrial disease defined by weakness in the muscles closest to the center of the body. Often, people with TK2d lose the ability to walk, eat, and breathe independently.

Kamiron — who was diagnosed at 15 after experiencing childhood symptoms including weak joints, difficulty breathing, and heart issues — uses a wheelchair for mobility and equipment to support his breathing. We spoke to this determined young man, who works as a disability consultant in Ithaca, New York.

How do you advocate for people with rare diseases?

Kamiron: The most important thing I do is talk with people at expos, in online forums, or on speaker panels. This summer, I'm running a four-day wheelchair skills camp, and I recently met someone who offered to send adaptive bikes for campers. She asked if I would help plan the next Achilles International paratriathlon.

How did you get involved in adaptive sports?

Kamiron: I grew up doing sports, but as I got older and my body changed, a lot of those fell off. Today, I play wheelchair basketball and do adaptive rock climbing.

What advice would you give to someone diagnosed with a disorder like TK2d?

Kamiron: When an opportunity comes, try it. The worst-case scenario is you hate it and don't do it again. The best-case scenario is you love it and find a new hobby, or even a new group of friends.

I never want to look back and be disappointed that I didn't live every minute, good or bad, as fully as I could have.

What kind of future do you hope to see for people with TK2d?

Kamiron: Medically, I hope we advance to the point that we can detect rare diseases better and treat, if not cure, them. But I also hope people with any rare disease can be content that this is a part of you, but it doesn't have to define you. You don't have to be 'John with TK2d.' You can just be John.

Learn more about TKd2 and hear other stories at TK2d.com.*

Kamiron Kraft

* TK2d.com is a site owned and operated by UCB. All information and materials on this site are provided by UCB and are subject to UCB's terms and conditions and pertain to the US only, unless otherwise indicated.

Inspired by **patients.**
Driven by **science.**

GOING S

MDA was founded on the hope for a better future for people with neuromuscular diseases. 75 years later, we continue to drive progress.

BY ANDREW ZALESKI

Is hope foolish? For 75 years, MDA has been answering with a resounding, "No."

When New York businessman Paul Cohen founded MDA in 1950, very little was known about neuromuscular disorders, which, at the time, were usually referred to

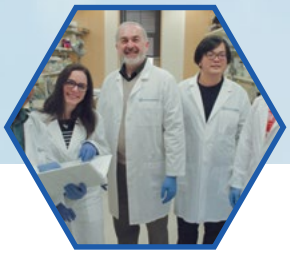


Image: iStock.com/laddawan punna; Krafla

simply as muscle disease. Only one person was interested in studying these diseases: Ade T. Milhorat, MD, a doctor at New York Hospital-Cornell Medical Center. Paul, who lived with facioscapulohumeral muscular dystrophy (FSHD), invited several other families affected by muscular dystrophy to join him in founding MDA and raising money for Dr. Milhorat's research. This led to a better understanding of muscle diseases and the founding of MDA Care Centers in 1953.

The same hope and determination can be traced through all the major discoveries and breakthroughs in neuromuscular medicine over the last 75 years. Thousands of researchers and clinicians now dedicate their efforts toward treating and, perhaps one day, eliminating neuromuscular diseases that affect millions of people worldwide — including this article's author. I live with type 1 myotonic dystrophy (DM1), and I hope that MDA's investments in research might one day lead to a method that blocks the mutated *DMPK* gene that causes my condition.

Angela Lek, PhD, Interim Chief Research Officer, MDA

Thousands *of researchers and clinicians now dedicate their efforts toward treating and, perhaps one day, eliminating neuromuscular diseases that affect millions of people worldwide — including this article's author.*

Since MDA's founding, the organization has poured more than $1.1 billion into supporting research on my own disease and many others, moving closer to answers. "We planted the seed that has now blossomed into an active field of research and drug development in 2025 and beyond," says Angela Lek, PhD, Interim Chief Research Officer at MDA.

With 20 neuromuscular disease therapies approved in the last 10 years, and a robust research pipeline with thousands of preclinical studies and clinical trials, there is every reason to believe that the extraordinary progress the field has made will continue.

Hope in action

Over the decades, MDA has supported research in areas that other organizations deemed impossible.

Much of the early study into muscular dystrophy focused on the muscle itself, aiming to understand how muscle tissue formed and functioned. A leader in this area was physiologist Don Wood, PhD, MDA's Immediate Past President and CEO.

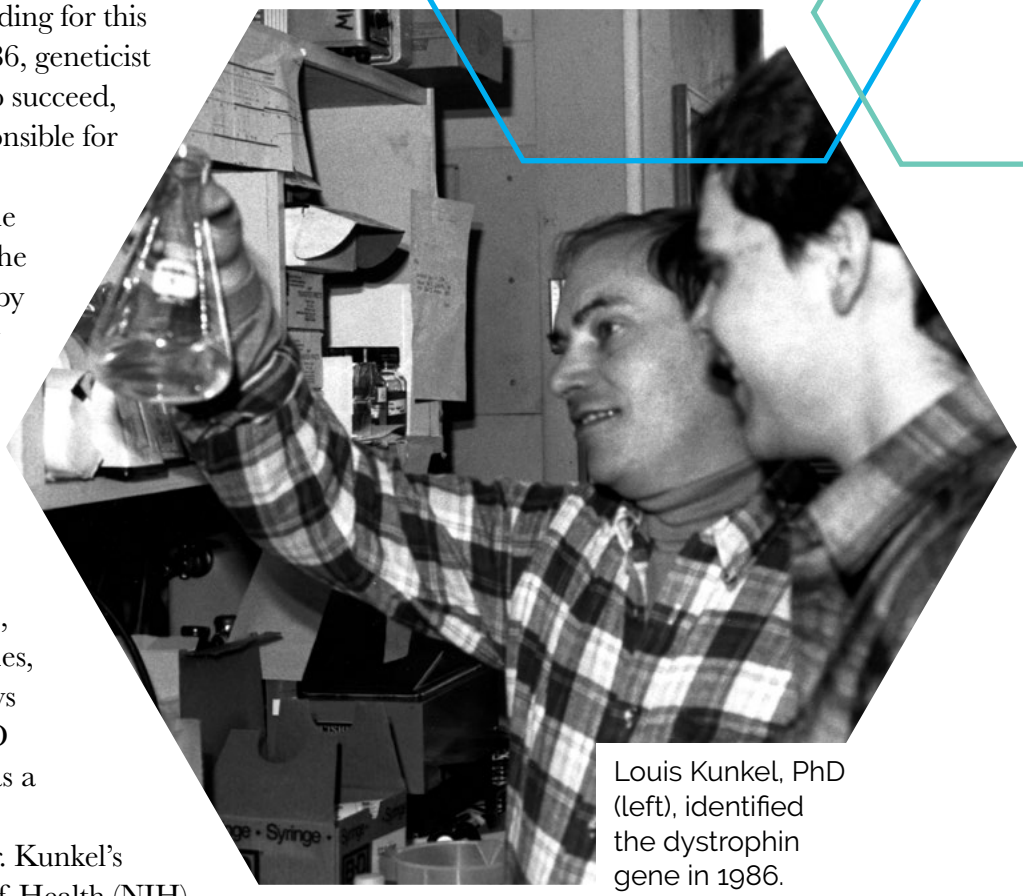
In 1976, Dr. Wood developed the technology to measure the strength of muscle fibers from muscle biopsies. This method was commonly used to measure strength in people with Duchenne muscular dystrophy (DMD), Becker muscular dystrophy (BMD), and myotonic dystrophy (DM). Grants from MDA helped fund this work.

In 1983, while serving as MDA's head of research, Dr. Wood established a task force on genetics. Because many neuromuscular diseases are hereditary, he concluded, it was vital to identify their

genetic causes. MDA awarded funding for this research to several labs, and in 1986, geneticist Louis Kunkel, PhD, was the first to succeed, identifying the genetic defect responsible for DMD and BMD.

It was a landmark discovery, one that still resonates, transforming the field of neuromuscular medicine by demonstrating that treating many neuromuscular diseases may be possible at the genetic level.

"At the time, we knew a lot about the symptoms of neuromuscular diseases, and we knew they're genetic because we could see the inheritance patterns, but until we began to find the genes, it was hard to make progress," says MDA Interim President and CEO Sharon Hesterlee, PhD. "That was a big breakthrough."



Louis Kunkel, PhD (left), identified the dystrophin gene in 1986.

Dr. Wood recalls that, before Dr. Kunkel's discovery, the National Institutes of Health (NIH) rejected his application for a research grant. The NIH told him the technology didn't exist to look for the genetic defect. Without MDA's funding, the discovery might not have happened until much later.

It's a sentiment that Jeffrey Chamberlain, PhD, knows well. Now the director of the Senator Paul D. Wellstone Muscular Dystrophy Specialized Research Center in Seattle, he recalls being at his own lab in the early 1990s when peers told him that gene therapy for muscle diseases would never work. He pressed on.

Dr. Chamberlain's research led to the creation of the first microdystrophin, a modified version of the gene that causes DMD, as well as the adeno-associated virus (AAV) mechanism for delivering genetic material to muscles throughout the body. These innovations have been used in gene therapies approved by the US Food and Drug Administration (FDA), as well as many currently in clinical trials.

"MDA believed in my ideas," Dr. Chamberlain says. "Without their support, the AAV microdystrophins would have never been developed."

"At the time, we knew a lot about the symptoms of neuromuscular diseases, and we knew they're genetic because we could see the inheritance patterns, but until we began to find the genes, it was hard to make progress. That was a big breakthrough."

— SHARON HESTERLEE, PHD

Building momentum

MDA's approach has succeeded in attracting scientists to the field of neuromuscular medicine not only

Image: iStock.com/laddawan punna

MDA's approach *has succeeded in attracting scientists to the field of neuromuscular medicine not only by believing in pioneering research work, but also by funding researchers through grants and fellowship programs.*

by believing in pioneering research work, but also by funding researchers through grants and fellowship programs.

"MDA was the first to train and recruit scientists and clinicians to study muscle disease," Dr. Wood says. "The fellowships provided financial security, so people had, in effect, scientific jobs to study muscular dystrophy."

To date, MDA has supported more than 9,000 scientific investigators focused on various neuromuscular diseases.

But MDA hasn't just been a funder — it's also provided inspiration. One of Dr. Chamberlain's enduring childhood memories is turning on the television during Labor Day weekend to watch comedian Jerry Lewis host the annual MDA Telethon, which ran from 1966 to 2011. The stories shared by people living with neuromuscular diseases influenced his professional path.

Dr. Chamberlain earned his doctorate degree while working with Stephen Hauschka, PhD, a biochemist at the University of Washington.

Always Advancing
Neuromuscular medicine keeps evolving. Stay up to date and find articles to help you understand the latest scientific advances at MDAQuest.org/science-research.

In Dr. Hauschka's laboratory, Dr. Chamberlain and his colleagues were among the first to clone muscle genes and identify the on-off switches that help muscle tissue form as people grow.

"Years later, it turns out that those muscle on-off switches are critical for the development of gene therapy approaches for DMD and many other types of muscular dystrophy," he says.

MDA provided funding to Dr. Hauschka's lab, and when Dr. Chamberlain established his own lab at the University of Michigan in 1990, his first grant also came from MDA.

"They really helped me hit the ground running and start understanding more about DMD and what it might take to come up with a treatment," Dr. Chamberlain says.

But it wasn't solely in the field of research where MDA made a difference. Stanley Appel, MD, recalls moving to Houston in 1977 to work at Methodist Hospital while heading the Baylor College of Medicine neurology department. There, he had the keen insight that patients with amyotrophic lateral sclerosis (ALS) needed comprehensive evaluations — encompassing walking, breathing, speech, swallowing, and muscular function — and these evaluations should happen in one place, at one time, so patients wouldn't spend anxious days lining up different appointments. That led to the formation of the ALS Research and Clinical Center at Houston Methodist in 1982, with MDA's support.

"MDA was there in the beginning, supporting the concept of multidisciplinary care, and we've been doing it ever since," Dr. Appel says. "That is where we've made a big difference in quality of life for our patients."

MDA's impact

For 75 years, MDA has invested in hope by supporting talented researchers and clinicians who looked beyond what seemed possible to forge an entirely new field of medical science.

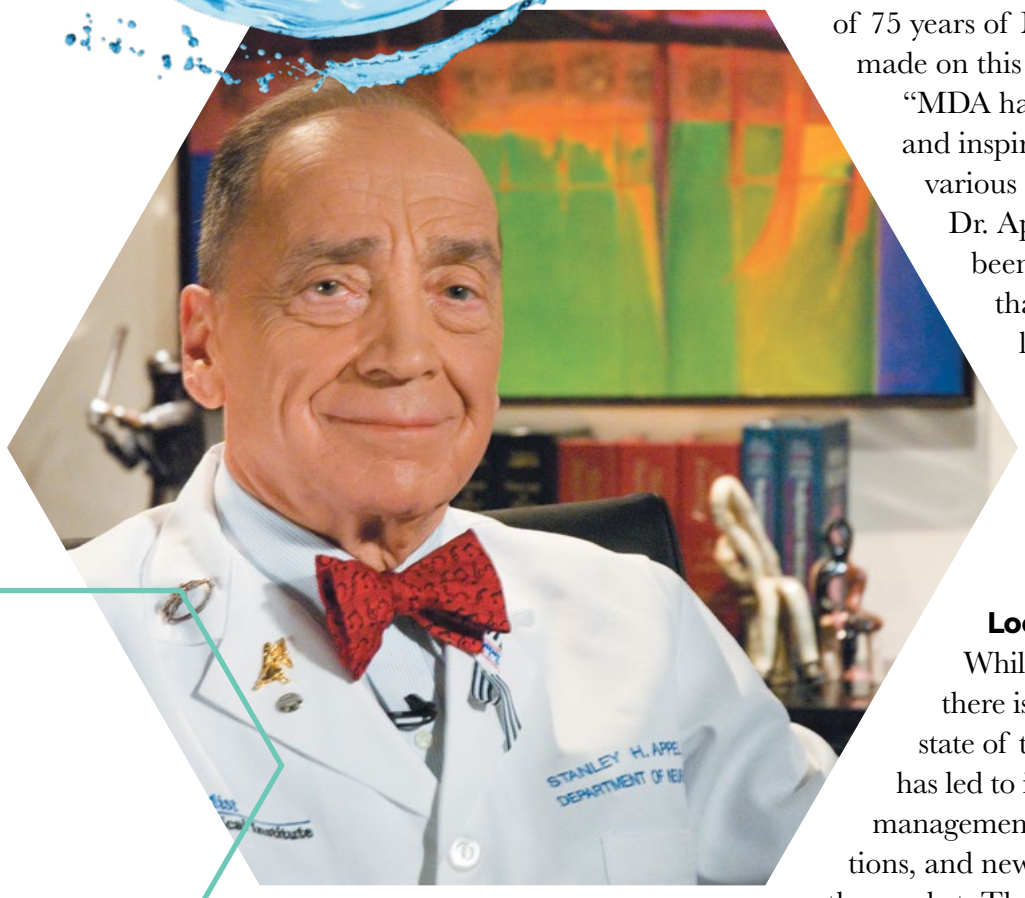
A key to this effort is MDA's coveted Development Grants, which award three years' salary and additional funding for supplies and equipment to promising early-career scientists pursuing neuromuscular research.

Łukasz Sznajder, PhD, an assistant professor of biochemistry at the University of Nevada, Las Vegas, used his MDA Development Grant to develop a research program for type 1 and type 2 DM. This

"[MDA] really ***helped me*** *hit the ground running and start understanding more about DMD and what it might take to come up with a treatment.*

— JEFFREY CHAMBERLAIN, PHD

Jeffrey Chamberlain, PhD



Stanley Appel, MD

year, Dr. Sznajder published a scientific paper on a surprising topic: the molecular connection between autism spectrum disorder (ASD) and DM1. This finding is significant because it demonstrates a complete mechanism for how ASD can be caused by a genetic mutation.

MDA's commitment to funding research and supporting clinical care has always been about pushing the boundaries of what's possible — while keeping in mind that our mission is centered on the individuals and families affected by neuromuscular diseases. Perhaps the best way to take stock of 75 years of MDA history is through the impact made on this community.

"MDA has played a very important, unique, and inspirational role in helping people with various neuromuscular diseases," says Dr. Appel. "All the experts who have been trained and all the disciplines that have grown under MDA's leadership take second place to the impact it's had on patients, their families, and the quality of life for many individuals who might never have had that quality without MDA's presence."

Looking forward

While there's more work to be done, there is reason to celebrate the current state of the neuromuscular field. Research has led to improved treatment and symptom management for many neuromuscular conditions, and new therapies continue to come on the market. Those investigating rare diseases — scientists, doctors, and organizations like MDA — are now turning to a frontier once thought unimaginable: regenerating functional muscle tissue.

By applying modern biomedical knowledge and technology to the science of how muscle tissue forms and functions, researchers hope to develop methods to restore muscle after it is lost to a neuromuscular disease. To this end, MDA held the first international muscle regeneration summit in 2024, bringing together leading researchers on the topic.

The progress doesn't end there. Current breakthroughs — life-saving gene therapies, rapid

"All the experts who have been trained and all the disciplines that have grown under MDA's leadership take second place to the impact it's had on patients, their families, and the quality of life for many individuals who never might have had that quality without MDA's presence." — STANLEY APPEL, MD

diagnostic advances, first-in-class drug approvals — were made possible by the decades of foundational work done by MDA. And we're only beginning to reap the rewards. According to MDA Interim Chief Research Officer Dr. Lek, the number of drugs approved to treat neuromuscular diseases is expected to grow exponentially over the next decade.

"We envision a future where every person living with neuromuscular disease can benefit from timely diagnosis, individualized care, and transformative therapies," she says. "Looking back, MDA changed the trajectory of entire lives and disease categories, through decades of partnership with scientists, clinicians, industry, and, most importantly, our patient community." Q

Andrew Zaleski is a journalist who lives near Washington, DC. He wrote about living with type 1 myotonic dystrophy (DM1) for GQ magazine.

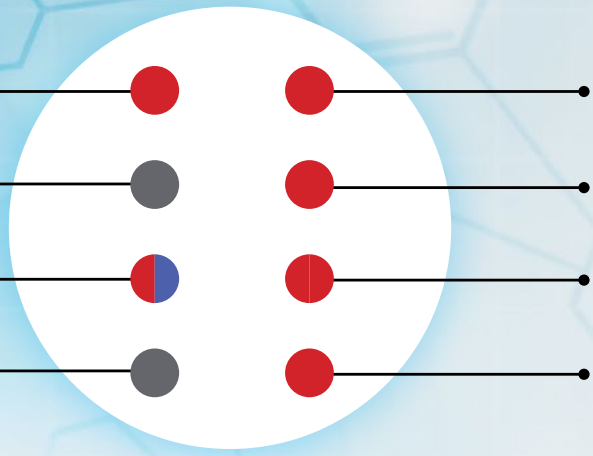
Across the Spectrum

MDA's mission encompasses individuals and families living with hundreds of different neuromuscular diseases

When MDA was formed in 1950, "muscular dystrophy" was used to describe most conditions that involved progressive muscle weakness. Today, thanks to advances in genetics and diagnostics, we know that neuromuscular diseases encompass multiple types of muscular dystrophies and hundreds of other conditions that affect the muscles, nerves, or neuromuscular junction, leading to progressive or intermittent weakness. MDA covers more than 300 neuromuscular diseases. Here's a peek at MDA's broad scope.

CLINICAL TRIALS **APPROVED TREATMENTS** **RESEARCH IN PROGRESS**

Muscular Dystrophies



Becker muscular dystrophy (BMD)

Congenital muscular dystrophies (CMD)
- 17 subtypes

Duchenne muscular dystrophy (DMD)
- DMD affects about 15,000 people in the US

Emery-Dreifuss muscular dystrophy (EDMD)
- 8 subtypes

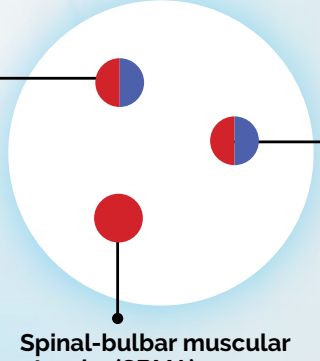
Facioscapulohumeral muscular dystrophy (FSHD)
- 3 subtypes
- FSHD affects about 20,000 people in the US

Limb-girdle muscular dystrophies (LGMD)
- 35 subtypes

Myotonic dystrophy (DM)
- 3 subtypes
- DM affects about 150,000 people in the US

Oculopharyngeal muscular dystrophy (OPMD)

Motor Neuron Diseases

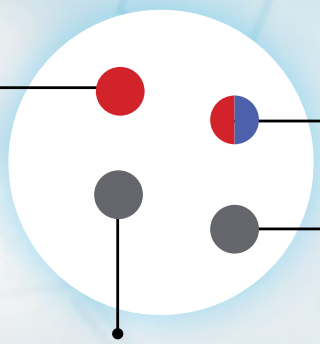


Amyotrophic lateral sclerosis (ALS)
- 24 subtypes
- ALS affects about 30,000 people in the US

Spinal muscular atrophy (SMA)
- 13 subtypes
- SMA affects about 20,000 people in the US

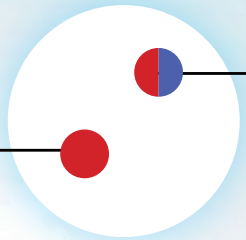
Spinal-bulbar muscular atrophy (SBMA)

Ion Channel Diseases



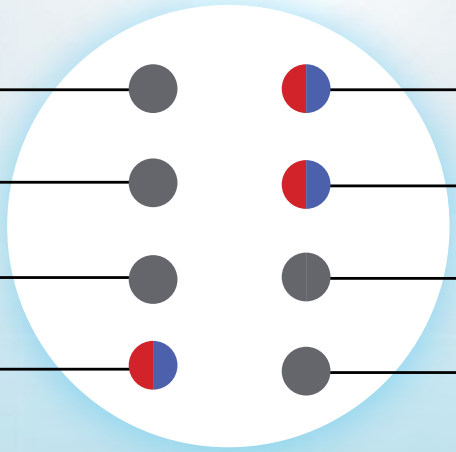
- **Andersen-Tawil syndrome**
- **Periodic paralysis**
  - 5 subtypes
- **Paramyotonia congenita**
- **Myotonia congenita**
  - 3 subtypes

Mitochondrial Diseases



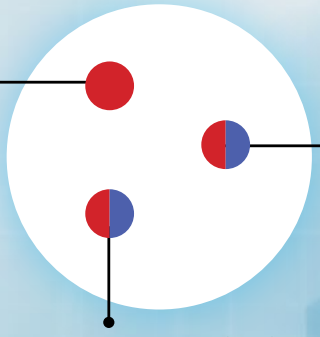
- **Friedreich ataxia (FRDA or FA)**
  - FRDA affects about 5,000 people in the US
- **Mitochondrial myopathies**
  - 19 subtypes

Myopathies



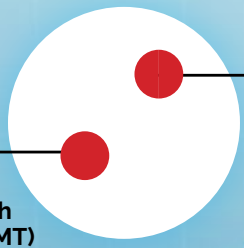
- **Centronuclear myopathy (CNM)**
  - 7 subtypes
- **Congenital myopathy**
  - 11 subtypes
- **Distal myopathy**
  - 21 subtypes
- **Glycogen storage disease (GSD)**
  - 18 subtypes
- **Inflammatory myopathy**
  - 5 subtypes
- **Metabolic myopathies**
  - 2 subtypes
- **Myofibrillar myopathy (MFM)**
  - 11 subtypes
- **Nemaline myopathy (NEM)**
  - 12 subtypes

Neuromuscular Junction Disorders



- **Congenital myasthenic syndrome (CMS)**
  - 40 subtypes
- **Lambert-Eaton myasthenic syndrome (LEMS)**
- **Myasthenia gravis (MG)**
  - 9 subtypes
  - MG affects about 70,000 people in the US

Peripheral Nerve Diseases



- **Giant axonal neuropathy (GAN)**
- **Charcot-Marie-Tooth disease (CMT)**
  - 54 subtypes
  - CMT affects about 150,000 people in the US

Diseases A-Z

Visit the Quest Media content library to find stories about community members living with specific conditions, as well as information about diseases and updates on research, at MDAQuest.org/diseases-a-z.

Image: iStock.com/alexaldo

In Their Words

Community members share their thoughts on MDA's past, present, and future

From our earliest days, MDA's mission has been centered on ensuring that individuals and families living with neuromuscular diseases have the resources, care, and support they need to live stronger and more independently. Our community's resilience and strength have propelled us to continually improve and find new ways to offer hope, connection, and community.

We asked community members what comes to mind when they think of MDA and what they hope to see in the future. Here's what they have to say.

Top of mind

"I think of MDA's wonderful events, giving me a chance to connect with other families. Knowing you are not alone in your diagnosis is huge for me, especially since I was diagnosed in my early twenties."

— Emily lives with late-onset Tay-Sachs disease in St. Louis, Missouri

"I remember taking my son Benjamin to the Fill the Boot events in our area, and having him out there helping with the cause was an amazing feeling."

— Valerie has a son living with Duchenne muscular dystrophy (DMD) in Farmville, North Carolina

"When I think of MDA, the first thing that comes to mind is a strong community. It is an organization that promotes people supporting people. It is a place for people who live with rare diseases to find 'their people.' People who understand exactly what they are going through and can understand each other on a level that can be hard to find in our everyday lives."

— Amy lives with Bethlem myopathy in Noblesville, Indiana

"MDA is an incredible resource that helps you with answers to questions you didn't even know to ask."

— John lives with amyotrophic lateral sclerosis (ALS) in Seabrook, Texas

Why I'm involved

"MDA completely transformed my life as a small child, and its impact continues to this day. It was through MDA that I finally received my diagnosis and accessed state-of-the-art care. Beyond the medical support, MDA helped me learn, both mentally and socially, how to live with my new reality after diagnosis. It's also where I began my journey in fundraising, advocacy, and connecting with other patients and families. MDA has truly been there for me through every stage and every need."

— Lily lives with Charcot-Marie-Tooth disease (CMT) in Charlotte, North Carolina

"To be able to do things inclusively and be proactive! Summer Camp, Fill the Boot, Shamrock pin ups — I look forward as an MDA Ambassador to being at these events and helping others learn, as I also learn, about muscular dystrophy."

— Dakota lives with myotonic dystrophy (DM) in Waterbury, Connecticut

"Since getting involved with MDA, I've seen advancements that either didn't exist or I didn't have access to when I was a kid. I believe one very important thing that still needs to be worked on is bringing awareness."

— Rodrigo lives with congenital muscular dystrophy (CMD) in Dallas, Texas

Looking to the future

"From when I was born to now, the field of neuromuscular disease has progressed so much through MDA's support ... This brings me hope I will see change in my lifetime."

— Justin lives with LAMA2 CMD in Concord, Massachusetts

"I hope and pray that neuromuscular disease continues to be better understood and that effective treatments, preventions, and cures are found that are reasonably priced and readily accessible to those impacted by neuromuscular diseases."

— Michael lives with limb-girdle muscular dystrophy (LGMD) in Chatham, New Jersey

"We are now seeing the realization of our long-held hopes for muscular dystrophy treatment options. There are numerous treatments available for various forms of this condition. My hope and desire is that just around the corner, we will arrive at the day when there will be treatments for all conditions, allowing everyone in the MDA community to live their best lives."

— Ira lives with spinal muscular atrophy (SMA) in West Palm Beach, Florida

+ENTER THE QUEST PHOTO CONTEST!

We want to recognize our readers and the meaningful moments in your lives. Share a great photo of you or a loved one with a neuromuscular disease, and it could be selected to appear in a future issue of Quest Magazine. Submit your photo by Sept. 14, 2025, at **surveymonkey.com/r/QuestPhoto25** or scan this QR code.

MDA Milestones

Take a journey with us through some of the foundational moments in MDA's history

1950

MDA Is Founded

On June 6, 1950, New York businessman Paul Cohen, living with a form of muscular dystrophy, assembles a group of people with personal connections to the disease to raise funds for critical research. This meeting marks the founding of the Muscular Dystrophy Association of America, igniting a movement to fight neuromuscular diseases.

1953

First MDA Care Center Opens

The first MDA Care Centers are established at NYU Langone Health and University of Rochester Medical Center, providing specialized neuromuscular disease support and care — a foundation of MDA's Care Center Network.

1955

MDA Summer Camp Begins

The first MDA Summer Camp welcomes children living with neuromuscular diseases, creating a unique space for friendship, fun, and independence.

1971

MDA Hosts the First National Labor Day Telethon

After years of local telethons and radio shows, the Jerry Lewis Labor Day Telethon expands to a nationally televised broadcast, inspiring unprecedented support for MDA's mission.

1986

Dystrophin Gene Is Identified

MDA-funded researcher Louis Kunkel, PhD, discovers the dystrophin gene, marking a watershed moment in research, ushering in a new era of gene discovery and paving the way for breakthroughs in gene therapy.

Continued on page 52

Edgewise THERAPEUTICS
Leaders in Muscle Disease Science

EDGEWISE IS COMMITTED TO DEVELOPING THERAPIES FOR INDIVIDUALS LIVING WITH MUSCLE DISEASE

Edgewise is developing an investigational therapy, **sevasemten,** which aims to protect muscle from injury caused by muscle contraction that occurs in individuals living with Becker.

WATCH OUR MUSCLETOWN VIDEO

Learn more about how muscles work (and why they sometimes don't in Becker and Duchenne muscular dystrophy), their role in our bodies, the proteins involved, and more by watching **this short video**.

EDGEWISE BECKER RESEARCH

PHASE 1

ARCH

Completed Phase 1 in healthy adults with Becker muscular dystrophy.

Open-label Study in Becker muscular dystrophy (NCT05160415)

PHASE 2 & Extension Studies

CANYON — Phase 2 in adults and adolescents with Becker muscular dystrophy (NCT05291091)

GRAND CANYON — Pivotal cohort in adults with Becker muscular dystrophy (NCT05291091)

MESA — Open-label extension that assesses the long-term effects of sevasemten on safety, biomarkers and functional measures of individuals in previous studies (NCT06066580)

DUNE — Advanced Phase 2 exercise challenge to evaluate the effects of sevasemten in adults with Becker, Limb Girdle muscular dystrophy 2i or McArdle disease (EU CT #2022-500215-39)

For more information about Edgewise and our development pipeline, **visit us online** or reach out to us at **studies@edgewisetx.com**

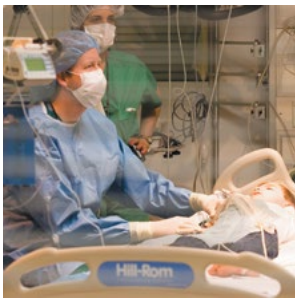
Check out our community resources page here to learn more about Becker and our efforts in the community.

MDA Milestones

2001

MD-CARE Act Is Passed

Passage of the MD-CARE Act, championed by MDA advocates and allies, expands federal support for muscular dystrophy research and healthcare resources nationwide.



2006

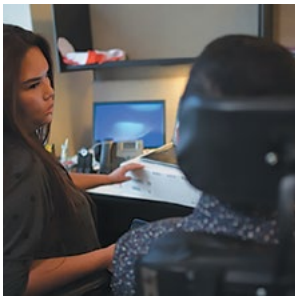
DMD-specific Gene Therapies Emerge

MDA funds the first-ever gene therapy trial for Duchenne muscular dystrophy (DMD), setting a new course for treatment possibilities in neuromuscular diseases.

2015

MDA Resource Center Opens

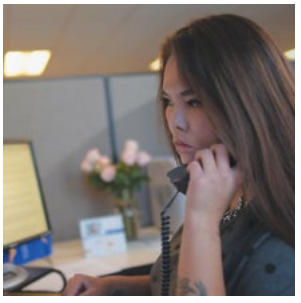
MDA establishes the MDA Resource Center, connecting families with trusted resources and knowledgeable support to navigate life with neuromuscular disease.



2023

MDA Launches the Gene Therapy Support Network

MDA creates the Gene Therapy Support Network to provide up-to-date information and guidance as groundbreaking gene therapies emerge for neuromuscular diseases.



2024

A New Therapeutic Era Begins

A new era of treatments emerges, with more than 20 FDA-approved therapies available for neuromuscular diseases — including the first treatments for DMD and spinal muscular atrophy (SMA) — thanks in part to research supported by MDA.

+75 YEARS OF HISTORY

Scan this QR code for a full timeline of MDA's impact over 75 years.

Expressing Our Gratitude

To our readers and all MDA community members, parents, clinicians, volunteers, friends, and more:

Through your passion, collaboration, and invaluably diverse expertise and effort, the MDA community has and will continue to change the world for people living with neuromuscular diseases. Quest Media is honored to stand alongside you on this journey.

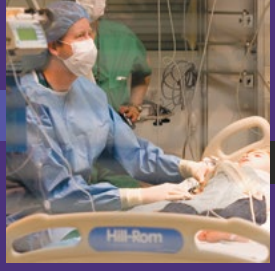
On MDA's 75th anniversary, we celebrate you and all we have accomplished together.

One community. A world of impact.
75 years and counting.

Quest Media
mdaquest.org

75TH

JERRY LEWIS
TELETHON



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