THE OUTLOOK

THE MYOSITIS ASSOCIATION®

Martha Arnold at the top of Mount Cardigan in New Hampshire. "And yes, I did hike up," she says. "It's a 2.8-mile loop with 712 feet elevation gain."

This themed issue on clinical trials sponsored by AstraZeneca, Bristol Myers Squibb, Chugai Pharmaceuticals, and Nkarta

SUMMER 2025
Quarterly Magazine

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THE OUTLOOK

A quarterly publication of The Myositis Association

THE MYOSITIS ASSOCIATION®

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TMA's mission is to improve the lives of persons affected by myositis, fund innovative research, and increase myositis awareness and advocacy.

TMA's vision is a world without myositis.

Published by:
THE MYOSITIS ASSOCIATION
6950 Columbia Gateway Drive
Suite 370
Columbia, MD 21046
E: tma@myositis.org
W: www.myositis.org

Seeing the Other Side: On Being a Clinical Trial Participant

By Martha Arnold

Martha Arnold at Zealand Falls on the Adirondack Trail in NH

Before I retired, I worked in the pharmaceutical industry, helping drug development teams make their strongest case to the FDA for approval of their compounds across a broad range of therapeutic areas. Now as a person living with IBM, I had been looking for opportunities to participate in a clinical trial, eager to "see it from the other side" as a participant rather than someone charged with interpreting the results.

When I heard about the Phase 2/3 trial of Abcuro's ABC008 (now known as ulviprubart), I was all in. My decision to participate was easy, as I suspect it would be for most of us with IBM. We have no treatment options. What do we have to lose? Being on placebo isn't a major concern, as that would be the same nothing we currently have. And earlier tests of the drug raised no significant safety concerns.

My first task was to determine whether I would qualify. The listing of "eligibility criteria" on ClinicalTrials.gov very helpfully laid out the minimum requirements. Sometimes these criteria may seem arbitrary and restrictive. But I know these criteria help ensure that enrollees can perform the activities—such as standing up from a chair—that are used to evaluate the drug.

Eligibility criteria also help enroll a relatively similar group of participants. This is important for the interpretation of trial results. If the groups are similar, differences between those getting the drug and those getting placebo can be seen as the drug's effect and not some other factor.

The second task: which of the trial sites might consider me? With a total of 18 visits over 80 weeks, the site would need to be reasonably close to home. Honestly, we underestimated the time and burden of travel on me and my family. Still, I am retired, and the flexibility in my schedule made it possible.

The third step: to find out if the sites convenient for me were considering new enrollees. If so, what information would they need to see and how could my records be transferred? Results of my muscle biopsy were especially important as this is used to confirm the diagnosis prior to enrollment in nearly all IBM trials.

I was offered an appointment to begin screening at a trial site in Boston. I made several visits to complete the tests and confirm that I qualified. These early visits also educated me about what to expect: the time and activity commitments I would be making, what was known so far about the drug's effect, and what side effects I might experience.

All aligned for me, and I was invited to enroll. I signed the **informed consent** and received my first dose of test medication. I was on my way for the 80-week study entitled "Randomized, **Double-blind**, **Placebo-controlled**, Multicenter Trial to Determine the Efficacy and Safety of ABC008 in the Treatment of Subjects with Inclusion Body Myositis."

After an initial flurry of visits, I settled into traveling to the clinic every eight weeks, where various tests were performed and I received a dose of the test medication. It may have been placebo, or a low dose (0.5 mg/kg) or high dose (2.0 mg/kg) of ulviprubart. This was a "blinded study," which means no one involved with the study knows whether I received drug or placebo.

At the end of the trial, I was offered enrollment in an "open label" extension study where everyone knows that we are receiving the drug at the highest dose. This type of trial is designed to understand what happens as patients stay on the product for longer periods of time.

People ask me whether this second study is being done because the first study showed evidence of **efficacy**. Importantly, no one knows the results of the double-blind trial until the last patient in that initial study completes their final study visit and a lengthy analysis process begins.

To obtain regulatory approval, the drug needs to show in a clinical trial that it changes how patients feel, function, or survive. The hope is that the first study will show that ulviprubart slows or stabilizes disease progression as measured by the IBM Functional Rating Scale and that the change is statistically and clinically different from what is seen with placebo.

When will we know? The initial read of the **primary endpoint** of the placebo-controlled trial is currently planned for the first half of 2026. Until then, all of us living with IBM are eagerly awaiting the results.

Martha Arnold is a former member of TMA's Board of Directors. She was diagnosed with IBM in November 2014. She lives in Pennsylvania with her husband Mark.

MyoCon™
TMA'S GLOBAL MYOSITIS PATIENT CONFERENCE

For thirty years, TMA has hosted the world's largest and most popular myositis patient conference. This annual gathering offers an unforgettable opportunity to learn about your diagnosis, connect with others who live with myositis, and consult with the world's leading myositis experts.

This year's event takes place in Dallas, Texas, on September 18–21

Here is just a glimpse of what you can expect:

- Preconference virtual presentations
- Sightseeing, scientific, and medical off-site excursions (separate ticket required)
- Dozens of breakout sessions with expert speakers
- Ask the Expert sessions
- Lots of networking opportunities
- Heroes in the Fight Awards Ceremony and Gala (separate ticket required)
- An extra special celebration of World Myositis Day on Sunday that you don't want to miss!

Registration is now open, but hurry!
Early bird rates end June 30.

Clinical Trials Challenges

As a patient with necrotizing myopathy, a difficult condition to treat, Dr. Lisa Christopher-Stine's patient was doing just okay on subcutaneous immune globulin therapy. It was holding his condition at bay—sort of—but he was still getting weaker.

Dr. Christopher-Stine, his rheumatologist and Director of the Johns Hopkins Myositis Center, wanted to help her patient with a more effective treatment. With decades of experience as both a myositis clinician and researcher, she knows that for rare conditions with few available treatments like myositis, finding more effective treatments often means being part of a clinical trial. But she also knows there are challenges.

"We stopped his IVIG for the trial, but within a month he became wheelchair bound. He couldn't walk, so he was too weak for the trial," Christopher-Stine says. "It was so frustrating. For him, for me, for everybody."

Dr. Lisa Christopher-Stine

Inclusion/exclusion experience

Inclusion and **exclusion criteria**, also called **eligibility criteria**, are often the cause of such frustration. These are the rules that define who can participate in a **clinical trial** and who can't. Drug developers create these criteria in order to control trial parameters to better reveal the effects of the therapy, often basing the study requirements on FDA guidance. Drugs intended to treat dermatomyositis, for example, are required by the FDA to show both muscle and skin improvement, so clinical trial participants must have both.

This excludes patients who might have active skin involvement only, for instance, which is what has happened to Kaniah Gunter, coleader of TMA's Women of Color Affinity Group, a patient who participated in the dermatomyositis Externally Led Patient Focused Drug Development (EL-PFDD) meeting with the FDA in June 2024.

"Despite my severe calcinosis and other symptoms," Kaniah says, "I was often disqualified from clinical trials because I didn't meet the muscle weakness criteria. It was frustrating. My condition was debilitating, but I didn't have the right kind of symptoms to qualify."

The EL-PFDD, conducted in partnership between TMA and MSU, was intended to help the FDA better understand myositis so they can improve the clinical trial process.

"These patient-facing sessions let the patients speak directly to the FDA," Christopher-Stine says. "That's powerful."

Treatment-related concerns

When a patient agrees to participate in a clinical trial, they are often required to stop their current treatment. This is called a **washout** period. This means, however, that their condition may get worse before the trial begins. Even once they make it into the trial, they may worsen if they are assigned to the **placebo** group. Some trials, however, allow participants to continue receiving their prior therapy.

This risk turned into reality for Veronica Fatura, coleader of TMA's Adelante! Spanish Affinity Group, who told her story for the EL-PFDD meeting. "I understand the reason for placebo in clinical trials, but I would love to find a way to avoid this option," she wrote. "As a clinical trial participant, it was terrible to get the placebo. Not only did I experience a flare, but my symptoms became worse than when I first was diagnosed."

To help her patients feel more comfortable with the risks, Christopher-Stine reassures them that they are still able to get "rescue treatments" (traditional medications like prednisone, for example) if they worsen while participating in a clinical trial. To help with both washout and placebo concerns, she also suggests that drug developers consider whether participants really need to stop taking their medications.

Drug developers don't want to confound the results with a second treatment; they want to be sure it's their drug that's causing improvement. Many times, however, the study drug is an add-on therapy. If this is how it's going to be used, Christopher-Stine reasons, maybe it's possible that patients don't need to stop their current treatment.

Burden of participation

While concerns about the washout period and receiving a placebo were major among patients who participated in the EL-PFDD, the most common reason for not entering a clinical trial was needing to travel to one of the widely scattered study sites. The myositis experts at specialty centers like Johns Hopkins care for patients from across the country.

"Imagine leaving your children, your job," Christopher-Stine says. "Many of these patients are in the prime of their lives when they get this disease. They can't be a mom or a dad and a trial participant. And they can't not work."

One important way to help patients would be to bring as many of the trial visits as possible closer to home. This could be done through technology like telehealth, or by having certain tests done by providers who come to the patient. This concept, called decentralization, is still new and there are limitations, but Christopher-Stine says it can be done.

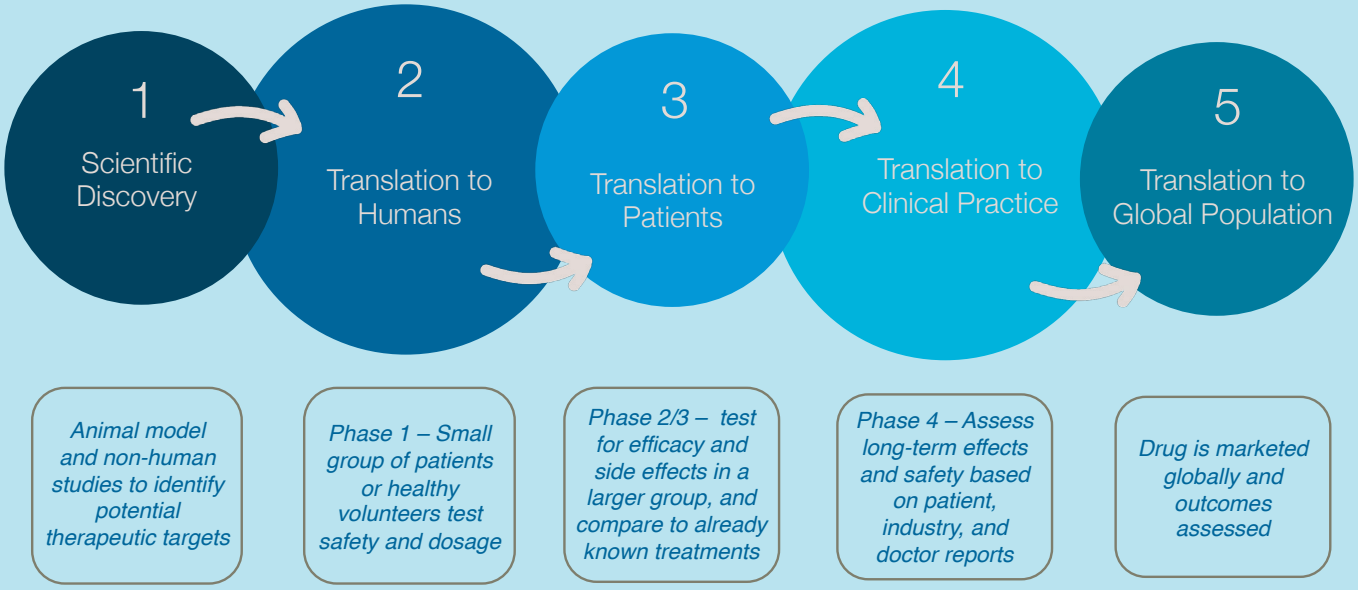
Making it easier for patients to participate in clinical trials would not only increase participation, making the completion of trials and approval of new treatments more likely, it could also increase the diversity of participation. More diversity would improve **generalizability**, making the results more meaningful to a wider variety of patients.

"We need to creatively recognize that there's a segment of the population that is either never tapped or is very suspicious of joining trials," Christopher-Stine says. "It won't be overnight, but I believe as technology gets more sophisticated, we can utilize that to reach people who are traditionally marginalized, for instance geographically or racially. We need to be thoughtful about making sure we have adequate participation that is more representative of the disease itself."

Adult Dermatomyositis EL-PFDD
Engage, Educate, Empower

Read the final report from the DM PFDD online: dermatomyositis-el-pfdd.org

Clinical trials research process



Many Trials, Few Patients

It may be surprising to know that there are only two FDA-approved medications for the treatment of myositis. In 1952, Acthar Gel was generally approved for severe inflammatory conditions. It wasn't until 2021 when a **Phase** 3 clinical trial called ProDERM showed the IVIG product Octagam 10% was safe and effective for treating dermatomyositis, that any treatment was specifically approved for a myositis indication by the FDA. (The report of the trial can be found here: **https://www.nejm.org/doi/full/10.1056/NEJMoa2117912**)

IVIG had been used for decades prior to this to treat all forms of myositis, so doctors knew it worked with all of these conditions except IBM. But it was used off-label, meaning it wasn't FDA approved for use in myositis.

"This was fantastic news for the myositis community," says Dr. Rohit Aggarwal. "Octagam is the first proven FDA approved treatment for a myositis indication. We've had drugs in the past, but this is the first one that has the kind of scientific evidence that is required for FDA approval."

Since that time, the number of clinical trials testing medications to treat myositis has grown significantly.

"The ProDERM trial opened doors for other drug manufacturers with new therapy options to experiment, because now they know there's a regulatory pathway for approval," Aggarwal says.

While this is good news for those who live with myositis, there are also challenges. Myositis is a rare disease, meaning it affects fewer than 200,000 individuals in the United States. With so many clinical trials running at the same time, the relatively small number of patients makes it difficult to recruit participants. If enough participants are not recruited to each trial, they can't be successful.

Myositis Clinical Trial Consortium

Striving for a solution

Aggarwal is personally working to address this challenge through the **Myositis Clinical Trials Consortium** (MCTC) whose goal is to increase the number of clinical trial centers and investigators throughout the world. This would make it easier for all patients, regardless of where they live, to be part of this important effort.

"We shouldn't shy away from doing more clinical trials if there is likelihood the drug will work," Aggarwal says. "If there is a difficulty with recruitment, we need to increase the pie. Myositis patients are not all going to come to Pittsburgh and Hopkins and other big centers. We need to expand the number of centers around the world that can do clinical trials."

Some drug developers have done better with recruitment than others, and some have even completed trials ahead of schedule. Their success depends partly on the patients that get to participate. More restrictive eligibility criteria make it more difficult to recruit patients.

Recruitment success may also depend on the length of the trial. An average clinical trial may last 24 weeks. In myositis studies, many are longer, running for 52 weeks or more. This is a problem. Patients find it very difficult to enroll in a 52-week study, especially if they are assigned to the placebo group.

"No drug company wants a 52-week study; no clinician wants that," Aggarwal says. "This is being enforced by the FDA for safety, among other reasons. But it's incredibly difficult, so we're trying to work on rational limitations with the FDA: 24 weeks versus 52 weeks."

Despite the long road getting to the recent FDA-approved treatment and the ongoing challenges, however, Aggarwal remains hopeful. "There are three major studies with results that will be announced this year, so we have a lot of excitement coming up. I'm hoping some of them will be positive."

Endpoints Used in Myositis Clinical Trials

Every clinical trial has a research question it is trying to answer. To answer these questions, clinical trials use measurements called endpoints. These measurements tell researchers if the treatment is safe and what effect, if any, the study drug is having on research subjects.

Here are the major tools currently used to measure effectiveness of a study drug in myositis clinical trials.

Endpoint	Description
Total Improvement Score (TIS)	Measures overall disease improvement using a score of 0-100 calculated from 6 core set measures • Extra-muscular global disease activity: Measures the disease activity of non-muscle organ systems and muscle • Physician-reported global disease activity: A global evaluation of the patient's overall disease activity by the treating physician • Patient-reported global disease activity: A global evaluation of the patient's overall disease activity by the patient • Manual muscle test (MMT): Assesses muscle strength using manual muscle testing • Health Assessment Questionnaire (HAQ): Assesses physical function • Muscle enzymes: Measures blood levels of muscle-associated enzymes Minimal improvement = 20 or higher; moderate improvement = 40 or higher; major improvement = 60 or higher
Cutaneous Dermatomyositis Disease Area and Severity Index (CDASI)	Measures disease activity and damage in the skin • Activity measures: erythema, scale, and erosion/ulceration • Damage measures: poikiloderma and calcinosis • Evaluates Gottron's papules on the hands for activity and damage • Assesses activity of periungual changes and alopecia
Inclusion Body Myositis Functional Rating Scale (FRS)	Assesses patient's ability in completing 10 functional activities such as swallowing, handwriting, dressing, hygiene, walking, and climbing stairs
Functional Index	Assessment of muscle endurance and function in frequently affected muscle groups
6-minute walk test (6MWT)	Measures heart and lung function and can also be used to assess exercise tolerance and ability to function
30 Second Sit-to-Stand	Measures leg strength and endurance
Patient Reported Outcomes Measurement Information System (PROMIS)—selected items	Assessment of patient's experience of myositis symptoms and disease burden • Pain Interference – assesses pain • Fatigue – assesses fatigue • Physical Function – assesses physical function

Clinical Trials Glossary of Terms

- **Adverse reaction** - A health problem that happens during the study and is reported as possibly caused by the study treatment.
- **Clinical trial** - A research study that tests drugs, devices, and treatments to see if they are safe and work in people.
- **Control group** - The people in a study who do not receive the study treatment or do not have the condition being studied.
- **Double-blind study** - A study that is set up so that the study treatment that each participant receives is not known by the participants or the researchers.
- **Efficacy** - How well a study treatment works in the study.
- **Endpoint** - A measure of the expected effect of the study treatment.
- **Exclusion criteria** - A list of reasons a person cannot be included in a study.
- **Generalizability** - How research results can apply to people who were not part of the study.
- **Inclusion criteria** - A list of requirements a person must meet to take part in a study.
- **Informed consent** - The process of learning and discussing the details of a research study before deciding whether to take part.
- **Open-label** - A type of study where participants and research staff know which treatment participants are being given.
- **Outcome (of study)** - The overall results of the study.
- **Outcome measure** - The way a study endpoint is measured.
- **Patient Reported Outcomes (PROs)** - The information patients share about their own health or well-being.
- **Phase** - A step in the overall clinical research process to test a new drug, device, or treatment. Phases of research studies build on each other, and each phase has a separate goal.
- **Placebo** - Something that looks like the treatment being studied but doesn't contain any medicine.
- **Placebo-controlled study** - A study with two or more groups where one group is given a placebo.
- **Preclinical study** - A study to test a treatment in the lab or in animals before testing it in people.
- **Primary endpoint** - A study measure that is used to answer the main research question.
- **Protocol** - A complete description of the research plan and procedures.
- **Randomized controlled trial** - Research that uses chance to assign participants into study groups.
- **Sample size** - The number of participants in a study or study group.
- **Secondary endpoint** - A measure used to answer other important questions in the study that are not the main research question.
- **Sponsor** - The group that is in charge of, or pays for, a research study.
- **Statistically significant** - Results that are unlikely to have occurred by chance.
- **Study arm** - A group of study participants who all receive the same treatment.
- **Study design** - The way a study is set up to answer the study question.
- **Washout** - A time before starting a study treatment when a person stops taking other medicines.

*Adapted from the **Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard**.*

Rare Disease Research Depends on You

Janine Lewis, Director of Research Operations NORD

If 300 million people had the same disease, scientists in every country would be rushing to find a cure, and it would be easy to enroll patients interested in testing promising drugs. But **study design**, promotion, and recruitment efforts targeting 300 million patients with 10,000 different rare diseases is not so easy. Patients are spread out and hampered by financial constraints and isolation. Each disease or group of diseases needs well-designed studies with full participation to make progress in finding treatments for myositis and the other 95% of rare diseases that so far lack a cure.

Sluggish recruitment means some trials must be abandoned. In other cases, delays can dramatically increase costs, according to Janine Lewis. She's the director of research operations for the National Organization of Rare Diseases (NORD), the umbrella organization for the 10,000 or so rare diseases identified so far.

"Suppose you're recruiting for a trial set to start in 2025 and it's 2027 when you finally have enough patients to get started," she says. "That delay can mean millions of dollars, dollars that weren't included in the original research grant."

Yet miracles in rare disease treatment can happen. Lewis mentioned the recent discovery of a treatment for a type of spinal muscular atrophy identified in newborns. A few years ago, a child born with this rare disease would be expected to die before their second birthday. Thanks to several recent breakthroughs in research, however, that child has a chance to develop and grow up normally.

Many myositis patients would like to be in drug trials but rule themselves out when they read the exclusion criteria. Lewis says some conditions for joining a trial can be pretty nuanced and suggests that patients who have questions about their eligibility should call the trial nurse (the number listed on the recruitment statement) directly rather than automatically disqualifying themselves.

While it's exciting to think of participating in a trial that might result in a pharmaceutical breakthrough, Lewis says it's also important to contribute information about your particular case to the studies that help scientists understand how diseases progress over time. Scientists use this information to establish damage markers for each disease and to reach consensus on what constitutes successful treatment. That's why natural history studies and patient registries matter, she says.

Also important: Helping clinicians, pharmaceutical companies, researchers, lawmakers, and others understand what it really means to live with a rare disease, especially one that's not normally in the public eye. You can do this informally by educating community and national leaders about your lived experience, or by participating in more formal sessions, like the Externally Led Patient Focused Drug Development (EL-PFDD) meeting with the FDA held last June for patients living with dermatomyositis and their care partners. Lewis also invites all those affected by myositis to share their experience of living with a rare disease on NORD's **Living Rare study**.

NORD
2025 MEMBER
PLATINUM

NORD was instrumental in helping TMA get started as a rare disease organization, providing contact information for 16 founding members. TMA has been a member of NORD since our beginning in 1993.

Currently Active Myositis Clinical Trials

Trial	Sponsor	Conditions	Contact (ClinicalTrials.gov code)
Anifrolumab	AstraZeneca	Adults with DM, PM	**More details** **AstraZeneca** 877-240-9479 NCT06455449
Brepocitinib (VALOR)	Priovant	Adults with DM (currently not recruiting)	**More details** 646-402-6892 NCT05437263
CABA-201 (CAR T cell therapy)	Cabaletta Bio	Ages 6-75 with DM, ASyS, NM	**More details** **Cabaletta Bio** NCT06154252
CD-19-Targeted (CAR T cell therapy)	Bristol Myers Squibb	Adults with refractory DM, PM, NM	**More details** **Bristol Myers Squibb** 855-907-3286 NCT05869955
Daxdilimab	Amgen	Adults with DM or ASyS (currently not recruiting)	**More details** 866-479-6742 NCT05669014
Dazukibart	Pfizer	Adults with active DM or PM	**More details** **Pfizer** 800-718-1021 NCT05895786
Efgartigimod (ALKIVIA)	argenx	Adults with IIM	**More Details** 857-350-4834 NCT05523167
Empasiprubart (Empacific)	argenx	Adults with DM	**More details** 857-350-4834 NCT06284954
Enpatoran (NEPTUNIA)	EMD Serono	Adults with DM, PM, ASyS	**More details** 888-275-7376 NCT05650567
GLPG3667	Galapagos NV	Adults with DM (currently not recruiting)	**More details** 321-534-2900 NCT05695950
Nipocalimab (SPIREA)	Johnson & Johnson	Adults with IIM (currently not recruiting)	**More details** 844-434-4210 NCT05379634
NKX019 (allogeneic CAR NK cells targeting CD19)	Nkarta, Inc.	Adults with DM, NM, ASyS	**More details** **Nkarta, Inc.** NCT06733935
Rapcabtagene Autoleucel (CAR T cell therapy)	Novartis	Adults with DM, NM, ASyS	**More details** 1-888-669-6682 NCT06665256

RAY121 (RAINBOW Trial)	Chugai	Adults with DM, NM	**More details** **Chugai** NCT06371417
Sirolimus	Investigator led	Adults with IBM (currently not recruiting)	**More details** 913-945-9926 NCT04789070
Ulviprubart (ABC008)	Abcuro	Adults with IBM (currently not recruiting)	**More details** 617-865-5079 NCT05721573

Participating in a clinical trial can offer access to new treatments and contribute to medical research, but it also comes with important considerations. You should:

- **Understand the purpose:** Know what the study is testing and why it's being conducted.
- **Review the risks and benefits:** Experimental treatments may not be effective and could cause side effects or complications.
- **Know your rights:** You can withdraw from the trial at any time without penalty or loss of medical care.
- **Ask questions:** Clarify anything you don't understand with the research team, including procedures, duration, and follow-up care.
- **Review the informed consent document:** This outlines all known risks, benefits, and your responsibilities.
- **Consider alternatives:** Ask about other treatment options outside the trial.
- **Always consult with your healthcare provider** before making a decision to participate in a clinical trial.

You can also find currently recruiting myositis **clinical trials** and **natural history studies** on TMA's website.

Living Rare Study

Clinical Drug Trials

Natural History Studies

Diversity in Clinical Trials

A statement from the Association of Women in Rheumatology

Diverse populations in clinical research is crucial to ensuring therapies are safe, effective, and accessible to all patients. They allow us to better understand the varying presentations of rheumatic diseases, reduce health disparities, and improve patient outcomes.

Poor representation in rheumatology trials leads to:

1. Limited treatment options: Lack of diversity hinders development of effective treatments.
2. Delayed disease understanding: Missing diverse populations delays identification of disease subtypes and therapy options.
3. Treatment confusion: Incomplete data misguides treatment decisions, jeopardizing patient care.
4. Perpetuation of disparities: Non-representative trials worsen health disparities and erode trust in the medical system.

Given the disproportionate impact of rheumatic diseases on minority groups, diverse representation in trials isn't just important—it's essential for advancing equitable, evidence-based care.

Special thanks to the sponsors of this special themed issue on clinical trials:

AstraZeneca

Bristol Myers Squibb

CHUGAI

nkarta

World Myositis Day 21 Sept

Save the date!

September 21 is now observed around the world, spreading awareness of these rare conditions internationally.

TMA Champions Myositis Awareness Month Resolution in US House of Representatives


H. RES. 277

IN THE HOUSE OF REPRESENTATIVES

RESOLUTION

May is Myositis Awareness Month, a time dedicated to raising awareness about a rare autoimmune disease that affects muscles and skin. The Myositis Association (TMA) leads this initiative, aiming to educate the public, support those living with myositis, and advocate for better treatments and a cure.

TMA is proud to announce a significant milestone in this ongoing effort to raise awareness. On March 31, 2025, Representative Rich McCormick, who serves Georgia's 7th Congressional District, introduced a resolution in the US House of Representatives supporting the designation of May as National Myositis Awareness Month. This resolution, H.Res. 277, underscores the importance of public awareness and education campaigns for those who live with rare diseases like myositis.

The resolution introduced by Representative McCormick, himself a physician, highlights the need for increased awareness and support for individuals and families affected by myositis. It encourages all Americans to become more informed about myositis and to support those living with this condition.

"As an emergency room physician, I have seen firsthand the impact a rare disease can have on a patient and their family. From the difficulty of diagnosis to little-known treatments, individuals may spend many years learning how to live with diseases like myositis. Increasing awareness of myositis, within the public as well as within the medical and research community, is crucial to speeding the time to diagnosis and to improving health outcomes," Congressman McCormick says. "Although each rare disease affects a small percentage of the population, rare diseases collectively affect 25-30 million people, or one in ten individuals. It is my intent, with this resolution, to advance awareness of the idiopathic inflammatory myopathies, collectively referred to as myositis."

Myositis can cause significant disability and reduced quality of life. For nearly two decades, TMA has led the observance of May as Myositis Awareness Month in the US in order to increase public understanding of this debilitating condition and promote research into its causes and treatments.

TMA members have achieved remarkable success in this effort, already securing 100-plus proclamations in 33 states and counting. These proclamations are a crucial part of this awareness campaign, serving as powerful tools to educate the public and bring attention to the challenges faced by those who live with myositis. They are also a testament to the tireless advocacy and commitment of TMA members, who have worked diligently to educate their communities about myositis and its impact.

"We are grateful to Representative McCormick and to Rich DeAugustinis, Vice Chair of TMA's Board of Directors, whose dedication and leadership resulted in the successful introduction of this resolution to support TMA's awareness and advocacy efforts," says TMA Executive Director Paula Eichenbrenner. "Rich's dedication and leadership has galvanized the myositis community, inspiring TMA members to advocate for proclamations in every state through our Proclamation 50 initiative."

You can help get this resolution enacted into law. TMA urges you to write to your representative and ask them to sign on as a co-sponsor of H.Res. 277. Everylife Foundation makes this easy with an automated message that you can personalize with your own myositis story and send to your representative. **Use this link** *or scan this QR code.*

Myositis Awareness Month was a Huge Success!

Thank you to all those who participated, presented, and planned a month's worth of extraordinary programming, making May's Myositis Awareness Month such a success! This dedication, insight, and passion have not only enriched TMA's outreach but have also created lasting impact in raising awareness, building community, and empowering voices that too often go unheard.

Here are some of the many ways the community came together to raise awareness for myositis diseases.

Diagnosis Days for each myositis form, including educational "Ask the Expert" webinars featuring TMA medical advisors and world-renowned myositis experts along with an informal "Café Chat" where families gathered with others affected by their form of myositis.

Myositis Awareness Month Golf Tournament

Myositis Awareness Fundraisers, including a **Golf Tournament** and a **Myositis Awareness Car Show**.

Meditation Mondays, a space for mindfulness and healing held weekly in May.

Empowerment Sessions featuring presentations by expert panelists on **Living Well with Myositis and Care** and **Planning for our Myositis Journey**.

Autoimmune Myositis Interstitial Lung Disease (ILD) Webinar in collaboration with Nori's Fight founder Julia Nickerson and Q&A with myositis experts Dr. Sonye Danoff and Dr. Julius Birnbaum.

Bridging Experience and Expertise, a panel presentation featuring healthcare providers who live with myositis.

Feel Good Friday Game Night: Jeopardy! organized by TMA's Women of Color Affinity Group.

Fairfax County, VA proclamation ceremony

Proclamation 50, an effort to secure official designations of Myositis Awareness Month in all 50 states. (See page 14 for a story about a national declaration.)

In 2025, TMA's First Pitch campaign made its global debut when Vance's friend Drew Ponce threw the first pitch at the nonprofit Gloves for Cuba baseball game

TMA's First Pitch for Myositis Awareness Campaign honoring long-time TMA volunteer Vance Robinson's initiative to raise awareness by throwing the first pitch at nearly 40 baseball games across the country.

Thank you to Myositis Awareness Month sponsors Pfizer, Inc., Abcuro, and AstraZeneca

TMA hosted an open house at our offices on May 9

WHAT BRINGS YOU JOY?

For Autumn Faulkner, it's her daughter Lily who brings her joy.

"Lily has helped me get through this challenging time in my life! I was admitted to the hospital for 12 nights, trying to get a diagnosis: dermatomyositis. This was when I finally, after one year, got diagnosed. If I hadn't landed in the hospital and had a muscle biopsy, I wouldn't have been diagnosed."

Tell us what brings you joy!

TMA's Top 10 Travel Tips

Are you thinking about attending MyoCon: TMA's Global Myositis Patient Conference? This four-day myositis community event is a "bucket list" item for many individuals and families affected by myositis. This year we'll be in Dallas, Texas on September 18-21.

A bucket list can be very motivating. Listing the experiences or achievements you want to accomplish can help you focus on the future. No matter where you are in your myositis journey, your bucket list may include travel, personal growth, and trying new activities. All of these things are possible at MyoCon!

Getting from here to there can be exhausting and difficult, though, especially for those who struggle with mobility issues, fatigue, and pain. To make the trek easier, here are TMA's top ten tips for traveling with myositis.

1. Research your destination, accommodations, and transportation options well in advance to ensure they meet your accessibility needs.
2. Travel with documentation about your health insurance (and/or a medical certificate), prescription medications, disability parking placard, and anything else that verifies the requirements for your safety and convenience. Download PDFs or save photos on your phone, in case you need to access documentation on the fly and out of WiFi reach.
3. Give yourself enough time for airport check-in and transfers. Be prepared for unexpected changes and have a backup plan in case things don't go as expected.
4. Notify your airline in advance that you will need special consideration. Be clear with your requests and specify what kind of assistive device you use. Check in with your airline again 48 hours in advance.
5. Request an escort pass at the airport. This is not guaranteed, but when granted it will enable your care partner to accompany you to the departure gate (even if they are not flying with you). Request the pass at the airport's special services check-in counter.
6. Your hometown airport may have a specific program to facilitate or encourage accessible travel. For example, more than 300 airports in 30 countries are part of the **Sunflower Hidden Disabilities Program**.
7. Travel smart with the equipment you own. Remove any batteries, loose parts, or cushions on equipment you're taking with you. Rent equipment as needed—scooters, rollators, bed risers for example. Check our conference "Plan your trip" page for a list of rental companies in Dallas.
8. If you travel with a service animal, make sure you have all the necessary documentation and supplies for them.
9. Pack smart. For example, don't overpack by being selective about what you bring. Keep important documents, like your ID, boarding pass, and travel itinerary, in an easily accessible place. Lithium-ion batteries, including power banks, must be carried in carry-on luggage only. Don't forget your comfortable shoes. And conference rooms are often cold; bring layers.
10. Check out "**Let's Travel: Making Disability Air Travel More Accessible**," hosted by TMA's Women With IBM Affinity Group, featuring Mindy Henderson, Vice President for Disability Outreach and Empowerment for the Muscular Dystrophy Association.

TMA is committed to providing myositis education to all. For those unable to attend the conference, we provide a package of session recordings for a nominal cost. You can also access our no-cost virtual educational programming provided year-round and on our YouTube channel for past programs. Please see our calendar to register for these programs.

Keep in touch with TMA!

facebook.com/myositis

instagram.com/themyositisassociation

linkedin.com/company/2810089

youtube.com/channel/UCef36fRGT90-DDT7KuPtnwA

x.com/themyositisassc

MYOSITIS CLINICAL STUDY

With innovation comes the hope of next-generation autoimmune disease treatments.

The Ntrust-2 study is evaluating the safety and activity of an investigational cell therapy, called NKX019, that is designed to target the root of myositis and other autoimmune diseases.

You may be eligible to participate in the Ntrust-2 study if:

- You are 18 to 65 years old.
- You have been diagnosed with myositis, systemic sclerosis, or vasculitis.
- Prior autoimmune disease treatments have not worked well for you.

You will also need to meet additional requirements. The study team will review these with you.

Costs for travel, lodging, and meals will be covered when you go to the study clinic. Compensation for study participation may also be available.

To learn more, visit clinicaltrials.gov.

Ntrust-2

NKX019-103_Half-page Ad_V1.0_03Apr2025_US_ENG

jasmine

JASMINE is a clinical trial for people who have been diagnosed with polymyositis or dermatomyositis.

You might be able to take part if you:

- Are getting treatment for polymyositis or dermatomyositis but still have symptoms
- Are between **18 and 75** years old

Additional information about the JASMINE trial can be found on the clinicaltrials.gov website, using ID number NCT0645549

Intended for US patients only

AstraZeneca

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- We are committed to helping meet the diverse treatment needs of Ig patients
- Pfizer provides SCIg and IVIg treatments, support, and resources for patients and caregivers

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Pfizer offers Ig Companion—a free mobile app designed to complement the treatment experience for patients and caregivers and help prepare them for doctor visits

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Ig=immunoglobulin; IVIg=intravenous immunoglobulin; SCIg=subcutaneous immunoglobulin.

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THE MYOSITIS ASSOCIATION

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