THE OUTLOOK

The Myositis Association®

SUMMER 2024
Quarterly Magazine

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TMA's vision is a world without myositis.

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TMA grant recipient awarded prestigious NIH grant

Interstitial lung disease (ILD) is a devastating complication of myositis, causing irreversible lung inflammation and scarring. Doctors don't understand the condition very well, so they have limited treatments to offer patients.

TMA medical advisor Dr. Cheilonda Johnson, an assistant professor of pulmonary and critical care medicine at the University of Pennsylvania, wanted to take steps to change this with a TMA research grant. Dr. Johnson's Pilot Project grant, awarded in 2019, dug into the genetics of myositis-associated ILD.

When TMA created the Pilot Project program, the intent was to provide seed money for new and innovative research projects so they could develop data that could be used to support applications for larger grants from other sources such as the National Institutes of Health (NIH).

We are happy to report that Dr. Johnson's project was a success. Thanks to this TMA-funded Pilot Project, we now have a better understanding of the molecular and genetic basis of myositis-ILD. This knowledge can support the development of new treatments for those who live with this difficult disease. Her work also began building a clinical and genomic database of patients with myositis-ILD that can be used for future research.

> *"TMA grant funding is so important in supporting the careers of early-stage investigators."*
>
> — *Cheilonda Johnson*

Best of all, the data developed by this TMA-funded project enabled Dr. Johnson to receive a prestigious RO1 grant from the NIH. This is major funding that will allow her to confirm the results of this project and extend the myositis-ILD database with a much larger cohort of patients with the intention of eventually developing new treatments for those affected by this disease.

TMA unites global patient advocates

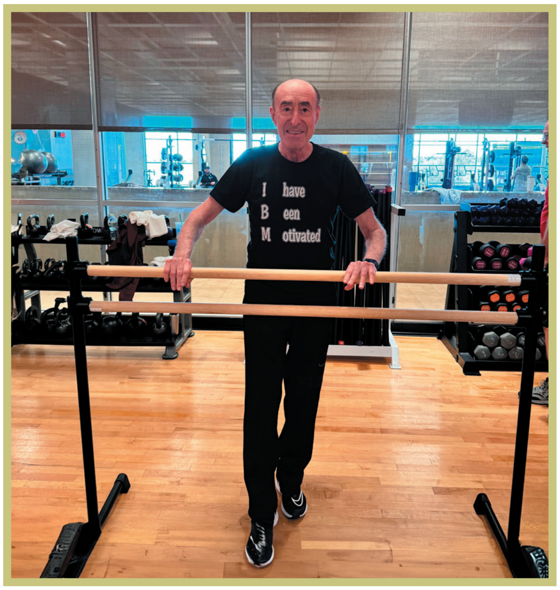
The Global Conference on Myositis (GCOM) is a biannual gathering of the world's leading clinicians and researchers in myositis. This year's gathering took place in Pittsburgh in March, and TMA was among a dozen patient organizations from around the world who were present to ensure the voice of the real experts—those who live with these diseases—was heard by the scientists and clinicians who work to understand and treat myositis diseases.

TMA was instrumental in bringing together members of international myositis patient organizations to make presentations to the scientific community, including video case studies in partnership with Drs. Rob Holloway and Kendrea Garand. In addition, the collaborative organized a Meet the Patient Experts panel, featuring six patient experts sharing about their disease, and a session on Patient-Centered Research Collaborations with case studies from Cure JM Foundation, InspireNMD from the Netherlands, Myositis UK, and Myositis Support and Understanding.

TMA also facilitated a parallel program for PAOs that featured nine PAO posters and representation from ten countries, both in person and virtually. Our parallel program was a highlight of the week for all of us, as we brainstormed about national research agendas, with inspiration from the Netherlands; consumer panels in research, with inspiration from the Myositis Association of Australia; and World Myositis Day, with inspiration from the German Myositis Group.

WHAT BRINGS YOU JOY?

Stepping up to the barre

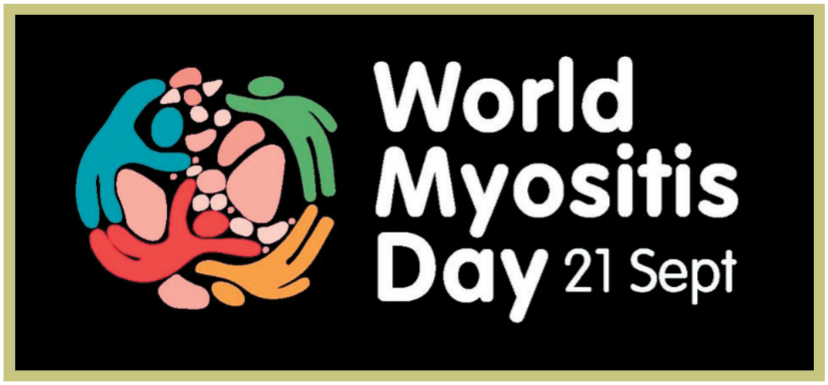


Howard Gerrin has IBM, so he knows how important exercise is for keeping his muscles as strong as possible for as long as possible. But it was difficult for him to keep up with the exercise sessions at his fitness center. Fortunately, the trainers had a solution to help him stay stable: a fitness barre.

"My fitness club figured out a way I could fully participate in aerobics with this barre. It was exciting to be able to work out at a good level with other members of the class."

—Howard Gerrin, TMA Board member

Tell us what brings you joy! Do you have a hint or hack to share? Email us at **TMA@myositis.org**.



Another chance to raise awareness

In 2001, TMA member Janice Goodell organized the first Myositis Awareness Day in Massachusetts to be recognized annually on September 21. Because one day was just not enough, in 2015 TMA expanded its awareness efforts to include the entire month of May. Nevertheless, myositis advocates worldwide have continued to recognize September 21 as a day to spread awareness of this rare disease.

Capitalizing on our success at the Global Conference on Myositis, TMA continues to lead a coalition of patient advocacy organizations (PAOs) in an ongoing effort to network, share best practices, and brainstorm solutions to common issues. This group has unified around September 21 as World Myositis Day as a way to bring the patient's voice to bear on research, legislation, and community understanding. TMA encourages myositis warriors everywhere to participate in this special international recognition by raising awareness in your own community.

Watch for programming details in our monthly newsletter and on our calendar **Learn more**.

Julie Tierney upper right corner

FDA official speaks to TMA members

During Myositis Awareness Month, TMA greatly expanded our free educational and awareness programming. Among the more than two dozen sessions included in this lineup was a presentation by Julia (Julie) C. Tierney, JD, Deputy Center Director for Strategy, Policy, and Legislation at the Center for Biologics Evaluation and Research (CBER), a division of the FDA.

Ms. Tierney spoke on “Amplifying the voice of myositis patients at FDA: Applying patient advocacy to advance rare disease.” In her remarks she discussed ways in which the FDA seeks input from patients and patient organizations like TMA in the drug approval process. Afterward she spent a lot of time responding to questions from those in attendance.

Thanks to Nancy Marx Erickson and the TMA Women with IBM Affinity Group for making this presentation possible. TMA was honored to host Ms. Tierney.

The video recording for this and all Myositis Awareness Month webinars are available on TMA’s **YouTube channel**.

ILD Day
Interstitial Lung Disease
Awareness and Education

Each year in September, the Pulmonary Fibrosis Foundation and eight partners, including TMA, host **ILD Day**. Like Myositis Awareness Month, it’s a time to focus the country’s attention on this devastating condition that affects those with a variety of autoimmune conditions, including some with myositis ILD Day 2024 is September 18. Watch for programming details in our monthly newsletter and on our **calendar**.

Open House at TMA headquarters highlight of Myositis Awareness Month

Local members enjoyed getting together with TMA staff

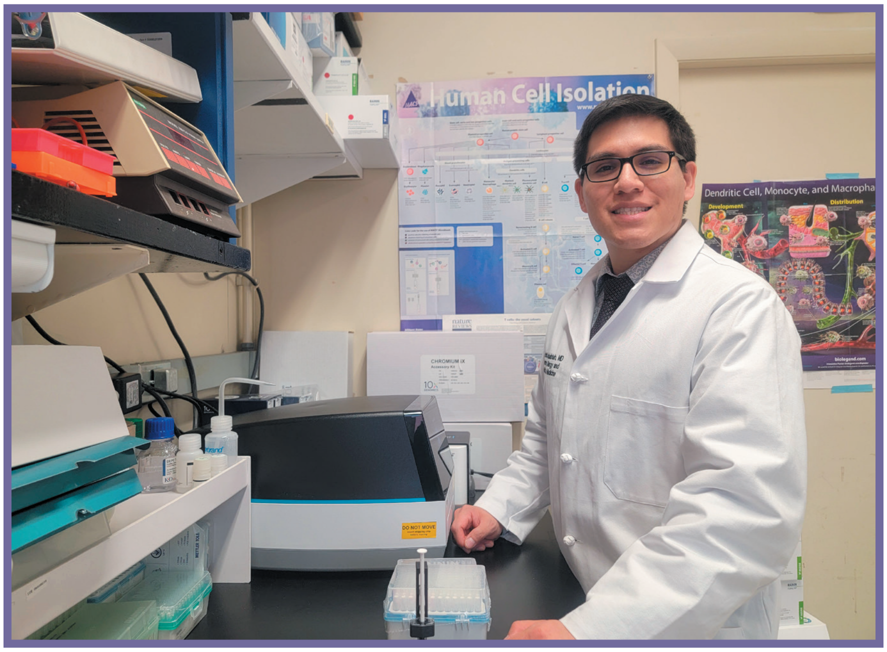
Thanks to our sponsor Abcuro

Handmade quilt from 2003 portraying TMA history!

Dr. Jemima Albayda responds to member questions during a Facebook Live "Ask the Doc" session—the first time ever for TMA!

Local guests visited TMA headquarters during Myositis Awareness Month

TMA research fellow revives lost technology to identify ILD biomarker



The path of discovery is not often a straight line. Most times it winds around from one question to another. Sometimes the trail just stops until someone else with a vision comes along and imagines where that dead end can go.

When Dr. Julio Huapaya arrived at the NIH for a fellowship in pulmonary and critical care medicine, he was eager to work on interstitial lung disease in those with autoimmune conditions like myositis. He was surprised, however, to discover that, despite the vast resources of the NIH and the prevalence of pulmonary fibrosis, studies on interstitial lung disease (ILD) were conspicuously lacking. This revelation inspired the pulmonologist to focus his research on understanding this chronic condition that causes scarring of the lungs making it difficult to breathe.

"We don't have really good treatments for pulmonary fibrosis," says Dr. Huapaya. "We really need to change this. So I thought, why don't we look at biomarkers in autoimmune interstitial lung disease? It would add one more piece of data to our understanding."

With the help of one of his mentors, Dr. Sonye Danoff from Johns Hopkins Myositis Center, Dr. Huapaya began to explore ILD biomarkers, signs that can be measured in blood, urine, and tissue to help identify whether a condition is getting better or worse. Autoantibodies are one such biomarker in those with autoimmune conditions. Dr. Huapaya also believed certain ILD autoantibodies could also cause disease.

But measuring autoantibodies is a challenge. Currently available testing measures—ELISA and the Western blot—are not good at quantifying the proteins they are testing for. This makes them insufficient as a measure of disease activity.

One of his mentors, however, Dr. Anthony Suffredini, was aware of work that had been done a decade or more ago to develop a blood test that could measure autoantibodies more accurately than the usual tests.

Developed by Dr. Peter Burbelo, this novel test was called luciferase immunoprecipitation system (LIPS). It had been used to identify autoantibodies in patients with acute respiratory failure. One of these antibodies turned out to be one for antisynthetase, the condition that most often leads to myositis ILD. Dr. Huapaya was excited to explore this technology for measuring autoantibodies associated with myositis ILD.

Testing his idea on a handful of blood samples, he was able to show that this new test could accurately measure myositis autoantibodies levels. More testing would need to be done on patients, however, to be sure LIPS could be used as a new way to understand the many layers of myositis diseases.

Using the data from this small study as support, in 2023 Dr. Huapaya applied for a two-year, $100,000 TMA Research Fellowship Grant to continue his quest for a biomarker of ILD. Now he is working to expand his initial research, using LIPS to solidify the role of autoantibody levels in ILD diagnosis and management. He is hoping this technology will be sensitive enough to detect myositis autoantibodies even before the onset of symptoms.

Still, the fact that the LIPS assay has been seemingly forgotten for more than a decade puzzled Dr. Huapaya.

“You know, Dr. Burbelo created this assay ten plus years ago, and I couldn’t understand why we are not using it,” Dr. Huapaya says. “Part of the reason is nobody thought that antibodies were what caused disease, so they never explored that route.”

The other reason, according to Dr. Huapaya’s mentor was that he never had the chance to collaborate with someone who had the kind of interest in autoantibodies as biomarkers…until Dr. Huapaya came along.

Now there are studies suggesting that these antibodies may actually cause disease, which may give us a new target for drug therapy for ILD and other myositis conditions. And now, thanks to a TMA Research Fellowship award, Dr. Huapaya is eager and able to do the necessary research to explore these questions.

TMA is a nonprofit organization that relies on the generosity of members of the community. It is part of our mission to fund innovative research to help understand the causes of myositis and pathways to treatment. If you value this work, please consider making a contribution in support of TMA’s Research Grant Program. We would all be grateful. ***https://www.myositis.org/donate/***

Please help others attend TMA's Conference!

Patients and families who attend TMA's International Annual Patient Conference (IAPC) say the people they meet there, and see every year, have changed their lives. Would you consider donating to a scholarship fund to help more people who live with myositis and their families attend the IAPC?

This year, your donation can go even further. Two generous TMA leaders, Augie and Rich DeAugustinis, have offered to match every gift to the scholarship fund, dollar for dollar, up to $10,000. Your generosity will help people who may feel very alone connect with others with the same diagnosis.

Augie DeAugustinis, former board chair, says, "TMA's IAPC is a life-changing experience for myositis patients. Everyone who attends this conference for the first time is amazed to meet so many people dealing with the same health challenges they are. I felt relieved to know I was not alone. My son Rich and I want to help people attend the conference even if they don't have the money to do so. Thank you for helping us make a difference!"

Rich DeAugustinis, current treasurer and board member, says, "Building face-to-face community is so essential, and TMA's IAPC is the 'Grand Central Station' of in-person networking for myositis patients. Attendees learn so much from international experts, and they also learn coping skills from other patients and care partners. My dad Augie and I want as many people as possible to experience this incredible event. Please join us in helping those who live with myositis attend the 2024 conference!"

You can donate at www.myositis.org/scholarships or by mailing a check to TMA at 6950 Columbia Gateway Drive, Suite 370, Columbia, MD 21046. Please include "scholarships" on the memo line of your check.

Thank you so much for your generosity!

In the early days of my diagnosis, I felt very alone. I was not able to get much information about my disease until I found The Myositis Association. They provided me with a wealth of information along with go-to resources. This also led me to my first patient conference. They were invaluable to me during this time.

— Dale Scott, former TMA board member and support group leader

Core Values

- **Diversity and Inclusion:** We recognize that different ideas and experiences enable growth, innovation, and continued progress for the community. We strive to create a community that creates a positive culture that welcomes, respects, supports and values all members and their varied experiences.
- **Compassion for the Community:** We seek to respect, honor, and support the journeys of our members, their families, and caregivers through our programming.
- **Collaboration:** We work together within TMA and with others serving the myositis community to achieve our best outcomes.
- **Responsible Governance:** We ensure the highest standards in service, quality and member satisfaction and commit to the pursuit of unwavering ethical conduct.
- **Stewardship:** We commit to maximizing the resources entrusted to us.

THE MYOSITIS ASSOCIATION

- **Integrity:** We strive to do the right thing always, to act truthfully and honorably, and to carry out our work with the greatest responsibility and accountability.
- **Respect:** We treat all people with respect and compassion.

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Cutaneous

By Kambiz Roghanchi

Livingston, New Jersey, February: A frosty gust nipped at my skin, but the real chill crept from within. Crimson tendrils snaked up my arms, an alien map drawn by my own traitorous immune system. These Gottron's papules, these fevered blossoms of war, were the first whispers of a coup. My body, once a trusted fortress, was under siege, my own defenses turned against me.

The enemy had a name, a cold clinical one—Dermatomyositis.

Doctors, trained to vanquish unseen foes, saw a textbook case. But I saw anarchy, my muscles turned traitorous battlegrounds, my skin painted with the scarlet scars of betrayal. The calendar became a tally of stolen dreams: no more hikes with my boyfriend, no more lazy mornings basking in stolen sunlight.

Before, my immune system was a silent roommate, its workings as hidden as the plumbing behind the walls. Now, it roared with the fury of a dragon, spewing cytokines as fire, my very tissues its fuel. Every morning, the mirror became a battlefield, each rash a fresh skirmish won by the enemy. The bathroom—once a sanctuary—transformed into a war room, where I assessed the damage, a medic surveying the fallen.

Yet, in this crucible, a warrior was forged. I learned the enemy's language, deciphering the code of my body. I became the cartographer of my own ravaged landscape, charting the treacherous paths of fatigue and pain. Each medication was a precarious truce, each flare-up a declaration of renewed hostilities.

Living with dermatomyositis is not a passive battle; it's a relentless dance with pain, a constant negotiation with fear. But within this struggle, a fierce resilience blooms. I cherish the good days, the moments of stolen sunlight, the laughter that echoes despite the ache in my muscles. I find solace in the shared stories of fellow warriors, their battles forging a kinship in the trenches of autoimmunity.

This is not a tale of triumph or surrender, but of an ongoing war. A war I fight not just for myself, but for the countless others facing invisible enemies within. For them, I raise my voice, a defiant banner against the tyranny of illness. For them, I fight, a warrior in my own skin, living within the walls of my enemy's fortress, but never yielding its dominion.

Kambiz Roghanchi, born and raised in New Jersey, was diagnosed with refractory dermatomyositis three years ago. Currently pursuing a degree in social work with a focus on the chronically ill, Kambiz is also an avid poet, finding solace and treatment in writing about his journey with the disease.

THE MYOSITIS ASSOCIATION®

THE INTERNATIONAL ANNUAL PATIENT CONFERENCE

SEPTEMBER 6-8, 2024

HILTON BALTIMORE INNER HARBOR

TMA's International Annual Patient Conference is the most celebrated event of the year for the myositis community! The 2024 conference takes place in Baltimore, Maryland on September 6–8 at the Hilton Baltimore Inner Harbor Hotel.

Here are just some of the highlights you can expect this year:

- Exclusive preconference virtual educational sessions
- Dozens of educational sessions from myositis specialists and expert presenters
- Your questions answered by the world's most distinguished myositis experts
- Industry and equipment exhibitors
- On site wellness fair
- Free access to all recorded sessions
- Optional attendance at TMA's annual Heroes in the Fight Awards ceremony
- Optional excursions to the Myositis Center at Johns Hopkins University and the resources at the National Institutes of Health
- Optional sightseeing tour at Baltimore's Inner Harbor

A lifechanging experience

For many attendees the chance to **meet and make friends with others who have their disease** makes this a not-to-be-missed experience. TMA's International Annual Patient Conference provides one of the only opportunities to connect with the myositis community in this way. It's one reason many attendees return to this gathering year after year.

Join us in Baltimore and Celebrate our Connection!

Register at www.myositis.org

THE MYOSITIS ASSOCIATION®

THE INTERNATIONAL ANNUAL PATIENT CONFERENCE

SEPTEMBER 6-8, 2024

HILTON BALTIMORE INNER HARBOR

Keep in touch with TMA!