

THE OUTLOOK



THE MYOSITIS ASSOCIATION



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THE OUTLOOK

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Photo by Camille Lesoine

Photo of the Prathers on page 10
by Wende Mayes-Hernandez

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**The mission of
The Myositis Association
is to:**

- *Increase program support to the myositis patient and caregiver community*
- *Increase awareness of myositis throughout the general population with emphasis on the medical community*
- *Increase funding for TMA operations and myositis-related research*

Dear TMA member,

If you were not at TMA's Annual Patient Conference in New Orleans in September, we hope you can be with us next year in San Diego (September 7-10). More than 450 persons who have myositis attended this year along with spouses, partners, and caregivers. Next year's Conference promises to be even bigger and will be held at the Sheraton San Diego Hotel and Marina located directly across the bay from the San Diego airport. The Sheraton will not be taking room reservations for the group discounted rate of \$125/night until February 15. A Conference registration mailer and additional information about the Conference will be sent to you in January.

Among the topics covered at this year's Conference were updates on the latest research and upcoming clinical trials focused on myositis. Bristol-Myers Squibb announced their trial that will test the effectiveness of the medication Orencia for those who have dermatomyositis and polymyositis. Idera Pharmaceuticals shared information about their upcoming trial to test IMO-8400 for those who have dermatomyositis and polymyositis. Octapharma Pharmaceuticals announced that they will be testing the effectiveness of IVIg for dermatomyositis. There will be an upcoming trial to measure the effectiveness of arimoclomol for those who have inclusion body myositis. There was also news that, although BYM338 had failed to meet the hoped-for results in Novartis's trial for inclusion body myositis, there was clearly increased muscle mass resulting from treatment with BYM338, and this may lead to other efforts to use this treatment for those who have suffered muscle loss.

These announcements were exciting news since they are all attempts to find treatments to help those who suffer from myositis.

Concurrent with the Conference, TMA's Medical Advisory Board met to evaluate the research grant and fellowship applications TMA received this year. These applications were largely focused on basic science to gain a better understanding of myositis and what treatment approaches might be worth pursuing. The Medical Advisory Board concluded that three of the applications merited funding, given the amount of research funds TMA has available at this time. The three research proposals were subsequently approved for funding by TMA's Board of Directors. The research to be conducted in all three proposals is considered to be applicable to all forms of adult myositis. Further information about these trials will be forthcoming. To read about previous research funded by TMA, please turn to page 15 of this *OutLook*.

Bob Goldberg
Executive Director

Communication: Key for patients in clinical trials

by Linda Kobert

The sudden termination of a clinical trial for what was touted as a potential breakthrough drug to treat sporadic inclusion body myositis this spring was a profound disappointment to many TMA members. A number of those involved in the study reported improved muscle strength and physical functioning, despite researchers' claims that outcome measures did not meet the study's stated goals. They looked forward to the day when this treatment would be approved by the FDA and available for use. But these hopes were dashed when the pharmaceutical company discontinued all use of the drug. The thing that upset these patients the most, however, was how poorly this company communicated with them during the trial.

"We had friends who participated in this trial, and they felt like no one listened to them," says Marianne Moyer, a TMA KIT group leader from southwest Florida. "It was as though the company said, 'Come for your monthly shot or weekly pill and then just be quiet and go away. You're just a patient and we are the medical experts.'"

Patient input is important

Not all pharmaceutical companies feel this way about those who might benefit from their efforts to develop new treatments for myositis diseases, however. In fact, two companies are currently or will

soon start recruiting participants with myositis for clinical trials, and both want to hear from patients.

"Having patients at the center is key to our mission as a company," says Helen Kellar-Wood, associate director for patient engagement at Bristol-Myers Squibb.

Later this year, BMS plans to roll out a study testing the safety and efficacy of a new medication for myositis. This phase III trial will be open for patients with active dermatomyositis (DM) and polymyositis (PM). But myositis is a new disease for the folks at BMS, and they wanted to learn more about how the disease affects patients and what is important to them in their daily lives.

"When we were at the point of writing the clinical protocol, we really felt we needed to gather insights from patients and caregivers as we developed the clinical design," Kellar-Wood says.

So the BMS team created what they call a Patient Engagement Network (PEN). It's a way for the company to hear the perspectives of patients, caregivers, and study sites over the course of a clinical study.

Through the Myositis PEN, Kellar-Wood and members of the clinical team met with patients, caregivers, and TMA staff at TMA's Annual Patient Conference to discuss the disease and problem solve about how BMS can address patient concerns during the drug development process. As a result of these meetings, the company made changes to the clinical protocol.

"Members of the Myositis PEN provided insights and the patient's perspective on things that would never have occurred to us as a pharmaceutical



company,” says Kellar-Wood. “This has allowed us to design better clinical trials. That’s win-win for us and the patients.”

Looking for results that are meaningful

Researchers at Idera Pharmaceuticals were also unfamiliar with myositis diseases when they started developing a drug known as IMO-8400. So in preparation for a phase II clinical trial to study the safety and efficacy of the medication in adults with DM, they got in touch with TMA to talk with its members.

“We were looking for patient feedback on what mattered the most to them,” says Kate Tighe, associate director for patient recruitment at Idera. “We wanted to know what’s the hardest thing for patients living with this disease. We wanted to design the study so the endpoint is not only scientifically meaningful but is also meaningful to the patient.”

Feedback from TMA members and Medical Advisory Board members was considered when designing the study protocol. Inclusion criteria, for example, were altered to allow patients to maintain some standard-of-care therapies while in the study, including common medications such as corticosteroids and IVIg. The number of tests and evaluations that patients would undergo was reduced too.

Patients have stories

For their part, patients are eager to help pharma figure out what improvement really means for them, and are hopeful that their experience with myositis will inspire other drug companies to not only come up with new and better treatments but also to create better, more effective ways of testing those treatments.

“The challenge that we have all inherited is not an easy one,” says Bill Simeral, who leads TMA’s Illinois KIT group. “Most people from the outside looking in do not understand our world. Not even one bit.”

“People are just so anxious to share what this disease is like,” says Susan McLoughlin, a DM patient who participated in a luncheon discussion

with the Idera team at the TMA Annual Patient Conference this year. “In the beginning, you think there’s something wrong with you. You’re embarrassed about things like tripping. But then you want to share it so others don’t have to struggle like you did.”

Communication is the key

During the trials, neither of these companies will know who exactly is participating in their projects; the double blind nature of the studies means neither the investigator nor the patient knows who is getting the drug and who is getting placebo. Still, the research teams at Bristol-Myers Squibb and Idera vow to keep patients in the loop.

“We can’t have any direct communication with the patients,” says Deb Pocetti, BMS’s protocol manager. “We do not know who they are, so unfortunately they are a number to us. But we realize there’s a human being on the other side and we want to make sure that we’re not disappointing them.”

In order to facilitate this communication with patients, BMS has created an online community platform where they will share clinical milestones and other information with patients, and where patients can keep in touch with the company. Idera, too, is staying in touch by providing a clinical trials website [<https://ideraclinicaltrials.com/rare-disease/dermatomyositis/>] explaining the study process in patient-friendly language. Patients can also email Tighe directly with questions. And both companies plan to communicate with constituencies through TMA and other patient advocacy organizations.

“We’re just thankful that they’re looking into myositis, wanting to help us,” says Barbara Kluding, who hopes she meets the inclusion criteria for Idera’s trial. “The more word gets out, the better people will be treated.”

*For more information about Bristol-Myers Squibb’s upcoming clinical trial, which is expected to start recruiting participants in December 2016, contact **BMSStudyConnect.com**.*

*For more information about Idera’s PIONEER study, contact Kate Tighe at **ktighe@iderapharma.com**.*

Nutrition, autoimmunity, and inflammation

by Theresa Reynolds Curry

There are dozens of theories about the connection between diet and autoimmunity. Most of them make sense, but nearly all of them remain unproven by valid studies. Proponents have described improvement in autoimmune conditions from whole-food, plant-based diets; from ketogenic (high-fat, low-carbohydrate) diets; from fasting and severely restricting calories; and from diets duplicating the foods foraged by Paleolithic populations.

TMA has funded research into the role of lipoproteins (the proteins that transport fats in the bloodstream) in myositis, and the response of

mice with inclusion body myositis to a ketogenic diet. Both studies returned interesting observations, but neither offered concrete recommendations.

Experts disagree on the best diet for overall health. They do agree, however, that many aspects of the modern lifestyle, including diet but also stress, pollution, and lack of sun exposure, have led to the emergence of many modern chronic diseases, including autoimmunity. Scientists know this by studying those people who still live in isolated, undeveloped areas and by observing that these people don't experience age-related rises in blood pressure, cancer, insulin resistance, autoimmune disease, and even the vision problems that plague the developed world.



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TMA is fortunate to have two members—both with dermatomyositis—who have sifted through much of the hype to find information about food and its relationship to inflammation and autoimmunity. Rose Mary Istre, a psychologist and leader of the Houston myositis support group, did a study examining the effects of an anti-inflammatory diet on a group of myositis patients. Istre has developed some practices, including diet, that have profoundly influenced her life as a patient with chronic disease. She shared some of her beliefs and practices at the 2016 Annual Patient Conference.

Renee Lantner, a Chicago allergist and member of TMA's Board of Directors, also presents each year at the Conference, with the latest update on foods and supplements that have shown promise in reducing inflammation in patients with chronic disease. Materials from both Lantner's and Istre's Conference presentations can be found at myositis.org, linked through the "2016 Annual Patient Conference Reports" from the home page.

[Experts] agree that many aspects of the modern lifestyle...have led to the emergence of many chronic diseases, including autoimmunity.

For all their differences, researchers have agreed on a few key points:

- ⚡ There is an association between vitamin D and autoimmune disease. A 2001 study in *The Lancet* followed people for 30 years and found vitamin D influenced disease course in children; the Iowa Women's health study found it to be true in women; and multiple other studies found that either sunshine or vitamin D supplementation were protective against lupus, rheumatoid arthritis, and multiple sclerosis, by limiting T cells and decreasing proinflammatory cytokines.
- ⚡ You can't get through the day anymore without hearing something about the impact of balancing the microorganisms that live in our gut. Prebiotics and probiotics are the new players identified by researchers as important for remaining healthy. Animal and human studies show that probiotic cultures can promote immunoreactive cells, regulate immune factors, and promote gut barrier function and anti-inflammatory responses. It's important to note that fiber-rich vegetables, whole grains, and fruits are prebiotics, acting as food for probiotic organisms.
- ⚡ There may be benefits from omega-3 fatty acids in autoimmune disease. These fatty acids, particularly those from fish oil—EPA and DHA—possess powerful immune modulating activities, says a 2002 review in the *Journal of the American College of Nutrition*. The anti-inflammatory properties might make them useful in the management of autoimmune diseases, reducing the need for steroids. Other studies have found improvements in energy and grip strength related to fish oil consumption. One vegetable source of omega-3 is flax.
- ⚡ Antioxidants play a role in autoimmune disease, and they come in packages that also supply vitamins and minerals, such as fruits, vegetables, whole grains, legumes, nuts and seeds, extra-virgin olive oil, avocado, nuts, fish, tea, spices and herbs, and, in moderation, red wine and dark chocolate.

Finding strength in the mind-body connection

by Theresa Reynolds Curry



Chronic illness is one of the greatest challenges anyone can face, says Leslie Saketkoo, a rheumatologist and associate professor at Tulane University School of Medicine. Dr. Saketkoo spoke on “Mind and Body Strength” at TMA’s Annual Patient Conference in New Orleans in September.

Dr. Saketkoo often speaks to patient groups on this topic, a special interest of hers. Part of her interest is professional, part personal. In an article published in “Scleroderma Voice,” she writes about her family’s experience with her father, who hid his lupus from the family until the disease finally became impossible to hide. His wish was to spare the family from worry and anxiety.

Once he let his family share his difficult journey, however, everything shifted. Here’s the truth, Saketkoo writes: “It was indeed painful, but ultimately it was a deepening and tender experience for an already very close family.”

Knowing your family is worried about you is just one of the difficulties a person faces after being diagnosed with a chronic disease. Everything—reduced energy and ability, symptoms such as pain, difficulty moving and breathing, and medication side effects—can seem terribly unfair. That’s

because these symptoms make it difficult for us to live as we expect to live, Saketkoo says, “as we thought we were entitled to live.”

Mindfulness practices can be one way for patients to find some peace. Most of us, in fact, already understand and use many of these strategies, but often we do it haphazardly. Intentionally adding activities, such as relaxation, meditation, yoga, and gratitude, to the tools you use to manage your illness can have significant positive effects.

Saketkoo has found this to be true in her work with her father, with her family, and with her patients. She teaches the practices she’s found most helpful to patients with an assortment of chronic diseases, and also shares these strategies with her colleagues and medical students.

Like those physicians who recommend mindfulness practice along with treatments for major chronic diseases such as cancer and heart disease, professionals treating chronic autoimmune diseases report improvement in related conditions such as nausea, insomnia, anxiety, depression, pain, and addiction.

Saketkoo teaches an adapted version of mindfulness, one that doesn’t require a spa, a great deal of time, or the ability to learn complex techniques. It works well for beginners and can be done on the spot as short periods of time become available during the day. Those patients (and caregivers) who attended her sessions at the Annual Patient Conference reported being able to feel more relaxed just by doing some introductory exercises.

Here are a few of the exercises she passed on:

1. Sit in a chair with feet resting on the floor and hands in lap. Close your eyes or soften to an unfocused gaze 4-5 feet ahead of you. Sit for seven minutes and observe what feels either tough or pleasant.
2. Sit as above, but rest your hands on your belly while your attention focuses on the sensations in your nose as you breathe gently and naturally. Your attention will wander, and when you notice this, simply direct it back to your breathing.

3. Sit as above, but this time focus on another physical sensation, perhaps how your back feels against the chair, or the slight stretch in the shoulder blades as you breathe.

Saketkoo says you can use almost anything as an anchor in these exercises: if not the breath, then the sensation of air on your skin or the sounds around you. The trick is to return your mind to its healthy focus, the focus that is displaced when you react to anxiety, anger, disappointment, or shame. Recognizing the “runaway brain” in this way breaks the cycle and allows the mind to notice the physical sensation of the gentle breath.

Once you’ve mastered the beginning exercises, you’re ready to incorporate mindfulness into your routine, using Saketkoo’s daily guide:

1. Sit (as outlined in the exercises) for 3-5 minutes.
2. Notice the pattern of your breath for 20-30 seconds each hour.
3. In anxiety-producing situations, take three purposeful breaths, using each breath to soften the mind, spine, and the rest of your body.
4. Make it a point to notice the small, beautiful things about your life as you dress, work, and walk.
5. While lying in bed, rest hands on your belly and allow the eyes to soften and close, allowing the attention to rest on the rise and fall of the breath under your hands. Saketkoo encourages patients to envision a balmy night, floating on a raft in a tropical lagoon, with gentle waves rising and falling.

But can you prove it works?

Patients already suffering from side effects from multiple drugs beg for nonmedical approaches for symptom and stress relief. Lots of research has been done demonstrating positive effects for patients with chronic disease through not only mindfulness, but also diet, massage, and yoga.

In a recent controlled clinical trial, for example, integrative medicine guru Deepak Chopra, MD



showed measurable decreases in a set of blood-based metabolites associated with inflammation, cardiovascular disease risk, and cholesterol regulation among participants in a six-day well-being program that featured a vegetarian diet, meditation, yoga, and massages. The findings, published in the journal *Scientific Reports*, represent a rare attempt to use metabolic biomarkers to assess the reported health benefits of integrative medicine and holistic practices.

In another study from the National Institutes of Health Center for Alternative and Complementary Medicine, researchers compared certain biological factors related to stress and inflammation in two groups of volunteers. The first group performed eight hours of intensive mindfulness practice during one day. The other group spent eight hours performing quiet leisure activities in the same setting as the meditators.

Before this intervention, investigators found no significant differences between the two groups in measurements of these biological factors. Afterward, however, the meditators showed changes not seen in the control group, including reduced levels of factors related to both stress and inflammation. The researchers suggest that their findings may offer a possible mechanism for explaining beneficial effects from meditation on inflammatory disorders, and an avenue for future research in chronic inflammatory conditions.

Volunteer spotlight: Supporting TMA at the Annual Conference and beyond

by Charlia Sanchez



Sheila & Stanley Prather

Stanley Prather feels a leader does not wait to be followed. As leader of the New Mexico, Texas Northwest KIT group, Stanley, who lives in Odessa, Texas, takes his support group wherever members need it.

Stanley was diagnosed with dermatomyositis in August 2008 after 10-12 years of symptoms that first affected his skin and then involved his muscles. Researching myositis online, he found TMA. In 2010, he and his wife Sheila attended the Annual Patient Conference in St. Louis, Missouri. They were grateful for that experience and have been attending ever since.

After being moved by that first conference, Stanley looked up the support group in his area and realized there was no leader to cover the large area of New Mexico and Northwest Texas. Sheila encouraged her husband to lead the group himself, and he began leading the group in 2011.

“My biggest difficulty was that it was so much area,” recalls Stanley. “Most people live in the El Paso/Las Cruces areas or in Albuquerque.” These areas are more than five hours drive from Stanley’s home in Odessa, so if he wanted to see his KIT group flourish, he had to figure out a way to make it work, despite the distance.

Stanley uses TMA conferences to recruit and reunite with members who live in the broad area covered by his KIT. And he is intrepid in his efforts to accommodate the needs of his members. Three

or four times a year, he travels to various areas across New Mexico and Northwest Texas, arranging meeting spaces, driving for hours, and staying in a hotel, to bring his support community together.

Stanley is committed to community service, even beyond his traveling KIT group. He’s an active, 30-year member of the Lions Club where he has served as chapter president three times. In 2015, he was named the Lion of the Year for his district.

Now retired from his job at a chemical plant and hoping to sell his auto estimates business soon, Stanley looks forward to having more time to devote to his family: two adult children and three grandchildren.

Still, he hopes one day to organize his KIT into mini groups that can be led by local co-leaders. He has a few people willing to help, and he will provide direction. He even wants to stretch the KIT to reach Amarillo, where about ten TMA members live.

“The best part of volunteering is the people; it has helped me more by being able to do this than I ever would have thought,” says Stanley. “I was hesitant to volunteer in the beginning, but this has been one of the most rewarding things I’ve ever done. And it’s because of the people. I often say you have to be an awful nice person to get this disease, because I’ve never met anyone [with myositis] who wasn’t top notch.”



LaShan Davis Lanier & Regina Davis

Lanier, who was diagnosed with polymyositis and interstitial lung disease.

Regina Davis doesn’t have myositis, but she has attended ten TMA Patient Conferences over the last 14 years. She first attended the conference in Houston, Texas in 2003 to support her sister, LaShan Davis-

“Regina is actually the one who researched my disease and found information online about TMA and the patient conference,” says LaShan.

When they first came to conferences, Regina and LaShan would split up and go to different sessions to soak up the most information. As the conference schedule expanded and repeat sessions were added, Regina saw an opportunity to volunteer her time during the event.

Regina brings a caregiver's perspective to the staff, effortlessly connecting with patients and attendees, as she has been on both sides of the registration desk.

Her dedication to serving TMA and the myositis community is second to supporting her sister, though. Now her care also extends to her father, James Davis, who was diagnosed with inclusion body myositis in the last few years.

Regina currently resides in Clinton, Maryland and works as an IT help desk analyst. She is originally from New York City and has 20 years of experience, working in nursing home facilities as a certified nursing assistant.

Regina has always enjoyed helping people and each year looks forward to seeing familiar faces as well as meeting new members and conference attendees.



Leslie DeAugustinis

It's Leslie DeAugustinis's faith that provides her with a unique ability to recognize support opportunities and connect with people in need. Leslie's husband Augie was diagnosed with inclusion body myositis in December 2008, and their faith also helped them manage this challenge.

Leslie has always lived a busy life, first as a loan officer then as a stay-at-home-mom for the blended family she and Augie brought together. She also volunteered with their church, participating in the women's ministry and planning women's retreats. "We were never not busy," she recalls.

When Augie got sick, Leslie gave up a lot of her own activities to determine and better understand what Augie needed. But, she says, "I like to take care of people. I'm a caregiver."

Her supportive nature and hostess heart carried over to TMA well. The couple attended their first TMA Patient Conference in 2009 in Charlotte, North Carolina and has been involved with TMA ever since.

As she became a regular conference attendee, Leslie noticed little ways she could help out and improve new patients' experiences. She suggested TMA have greeters at the conference, for example, to welcome first-time attendees while they checked in at registration. Leslie has been known to pull people out of line and tell a joke or share kind words with attendees who appear to be lost or nervous about the conference.

"I sensed the discomfort of newcomers and tried to ease their tension," she says.

While Augie served on the TMA Board of Directors (as chair from 2013-2015), Leslie found her own place to fit in and has served as a community builder at the conferences. She connected with other IBM caregivers and is part of that growing community within TMA. And after meeting people who were troubled by their diagnoses, she suggested TMA offer a session on finding strength in faith; Augie has been presenting on the topic for several years.

Leslie wants people to leave the conference feeling cared for, understood, and with a sense of camaraderie and fellowship. She has made some lifelong friends through TMA and says, "As Augie's disease progresses, we're finding a new normal."

Leslie is still active in Lakewood Baptist Church of Gainesville, Georgia, the church she and Augie attend. She continues to serve in the women's ministry as well as a program that offers support and fellowship in prisons and halfway houses. Through this program, the church helps women who are transitioning out of the correctional system find apartments, jobs, and even cars.

She is not as involved in these activities as she once was, however, because Augie is her first priority. They have three children and eight granddaughters between the ages of 5 and 23.



You can do it!

by Nancy Harber

Nancy Harber's husband Charlie had inclusion body myositis (IBM). A registered nurse and an informal consultant, Nancy shares the wisdom she gained as Charlie's long-time caregiver with patients and caregivers struggling with the challenges of myositis. While her experience was specifically with IBM, many of her solutions apply to other forms of myositis as well.

How do you keep from getting discouraged and worn out on this journey that you never wanted to take? And how can you keep the person you are caring for from seeing your discouragement and exhaustion? The simple answer is you can't, so you need to be honest with yourself. These are difficult diseases we're dealing with. We can't change how the disease deals with us, but we *can* have an effect on how we deal with the disease.

Make no mistake: you *will* have discouraging moments (or hours or days!), and there will be times when you wonder if you can take another step. Some of my most exhausting times were in the last year of Charlie's life, when I wondered if I would ever again be able to sleep more than two hours at a time. But just as a new mother learns to rest when her newborn rests, I learned to nap in my recliner in the living room during the day while he dozed in his wheelchair. And if he wanted to go to bed earlier than usual, I went down too.

I also learned to set priorities. Charlie's needs were at the top of my list, and I don't mind admitting that dusting and vacuuming were at the bottom. In between, things changed from week to week. You can't do everything that you used to do, particularly if you are trying to do it alone. It's a good idea to ask for and accept help!

Charlie was a very bright, highly educated man whom I met when we were in first grade. Though we weren't married a long time (26 years), he knew me well and could tell by looking at me that I was having difficulty coping. We tried to be honest with each other and laugh when we could. I told him I needed a wife to help me out, for example. He told me he needed a new nurse, the old one was wearing out.

Rethinking your attitude is especially important at this time of year. Regardless of which holidays you celebrate, it's important to adapt your family traditions to fit your current capacities. Going to our mountain cabin for Thanksgiving, for example, was great fun for our extended family. But snow was always a possibility at 6,000 feet (even in California) so the last few years, we moved the family gathering to good wheelchair weather and celebrated Thanksgiving in July.

Christmas was a great time at our house. Charlie hung lights outside, and I cooked and baked for weeks. The outside lights disappeared in the last eight years of Charlie's life, but I hung wreaths and ribbons on the front porch. The live pine Christmas tree shrunk in size, and though I still cooked a traditional dinner, my cookies became the quick refrigerator dough kind.

The point is, you don't have to give up your holiday celebrations. Trim them down to a manageable size, but continue to enjoy the music, lights, and fellowship that you've always enjoyed.

I know you face, as I did, many days when you want to scream, "Stop the world, I want to get off." Since that's not possible, all you can really do is hold on tight to each other, say a prayer, and begin each day with renewed hope. You can do it!

Pacman: The newest member of “Team Ray”

by Ray Lesoine with Camille Lesoine

Ray Lesoine was diagnosed with PM in 2005 and IBM in 2012. He and his wife Camille are dedicated TMA members. They live with Pacman in Parrish, Florida.

I am a dog nut, but I was initially reluctant to apply for a service dog, because I thought it would create too much work for my already busy wife/caregiver. Camille encouraged me to apply for one of these specially trained service dogs for people with disabilities, however, and in the fall of 2015, Camille and I got Pacman, a two-year-old yellow lab that I fell in love with immediately.

To find Pacman, we worked with Canine Companions for Independence (CCI) in Orlando, Florida. CCI is a nationwide organization based in California with campuses throughout the country. Before we chose CCI, we saw there were many organizations that supplied service dogs. We were advised that some of these are scams, and we should choose an organization that is certified by Assistance Dogs International (ADI). CCI came highly recommended. They are funded entirely by private donations, and their dogs are given to the recipients at no charge.

When we applied with CCI, we had a telephone interview in which we were asked what we felt was important in our service dog. We told the interviewer that it would greatly improve my freedom and allow me to go out of the house on my own. It would also ease Camille's workload, freeing her from taking care of some of my daily needs. I also wanted a dog that could travel with us and march with me in veterans' parades.

A few months later we drove to Orlando for a face-to-face interview. During the interview, instructors

demonstrated what the dogs can do. When I watched one of the dogs retrieve dropped items, then place its head in the instructor's lap to give them back to her, I decided I really wanted one of these service dogs.



Ray Lesoine with Pacman
Photo by Camille Lesoine

Training is intense...for dog and master

CCI has a great group of breeders and puppy raisers. The breeders keep the puppies until they are around eight weeks old, then they go to a puppy raiser for a year and a half to learn their first 30 commands. The dog's next move is to one of CCI's facilities for six months of advanced training where they learn an additional 30 commands, including maneuvering around wheelchairs.

It was 14 long months after our initial interview before we got the call that there was a possible dog for me, and Camille and I drove more than 100 miles to the Orlando facility for two weeks of training. We learned about dog behavior and motivation, and I worked with six different dogs for the first two days. During the second day, I was matched with Pacman, and we began working as a team.

We met Ray and Carole Burke, Pacman's puppy raisers, at the Orlando graduation where they officially turned the leash over to us (a tearful moment for all). Years ago, when the Burkes lost their last pet dog (not a service dog), it was so hard putting the dog "to sleep" that they vowed never to go through that ordeal again. Instead, they became puppy raisers for CCI. Ray and Carole say it's hard to give up a dog after a year and a half, but knowing it's going to a good cause softens the pain.

More than a pet

Pacman does many things to help me. Of course he is a great companion and gives me purpose, but he also helps me do things I can't. He picks up objects that I drop and puts them in my lap, for example. He opens and closes doors and turns lights on and off, and he accompanies me outside so I am able to take long walks without assistance. He loves it when Camille lets him jump up in bed to "hold my hand" or stretch across my middle to say "Good Morning."

There are even health benefits with Pacman. My blood pressure, for example, has dropped 20 points. We take him everywhere: the ballet, restaurants, movies. His puppy raisers have even had him on airplanes. He is great to travel with; he just lies down in the back of the car and goes to sleep. When he is not working, he gets to be a real dog, but he *loves* to work.

What is required of us

We are required to keep him within five pounds of his ideal weight (66 pounds). Every day he has to be brushed, ears cleaned, paws and coat inspected, and exercised. His teeth need to be brushed and toenails trimmed weekly, and he gets a bath monthly. He can never be left off the leash while outside unless he's in a fenced-in area, and we have to be present. We assume all responsibility for food (high quality), medicine, vet bills, and shots.

But Pacman is very easy to care for, because he is so well-trained. He enjoys all the cleaning and trimming processes and looks forward to having his teeth brushed with chicken flavored toothpaste. As we said in Camille's "Share your favorite strategies and devices" sessions at the 2016 TMA Annual Patient Conference, he is the best gadget out there. We really love this guy!



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Seeking a cure: TMA's research funding program

By Linda Kobert



Malin Regardt

with researcher Lisa Christopher-Stine, MD from the Johns Hopkins Myositis Center, and she came to the conference to collect data.

During the conference, members stopped by Regardt's table in the exhibit hall, and she invited people with inclusion body myositis to participate in her research project. Participants completed a written questionnaire designed to assess their ability to perform certain tasks, and Regardt measured different aspects of hand function, such as grip strength and dexterity, that could influence their daily activities.

Regardt's project was one of seven that were selected in 2015 through TMA's research funding program. Since 2002, TMA has provided more than \$5 million dollars in funding for promising research projects such as Regardt's in the ongoing search to understand the underlying causes and natural progression of myositis, develop better treatments and more effective therapies, and ultimately to create a cure for this collection of disabling diseases.

Malin Regardt, PhD attended the recent TMA Annual Patient Conference in New Orleans, but she didn't have a lot of time to attend many of the sessions. Regardt is an occupational therapist and post doctoral fellow working

Support to promote more research

Part of TMA research funding specifically targets research fellows like Regardt, providing salary support for these trainees as a way to attract and encourage young physicians and scientists to pursue careers that include investigations into myositis.

"I am so glad that I've got this opportunity," says Regardt, whose entire focus is on myositis and includes research into polymyositis and dermatomyositis as well as IBM. "There are not many occupational therapists doing research in rheumatology and even fewer in myositis. To do good research and develop as a researcher, funding is important. The TMA grant enables me to focus on my research, not just do it in my spare time."

TMA also provides seed funding to kick start innovative projects so researchers can gather preliminary data in support of larger grant applications. Neurologist Jerry Mendell, MD, for example, received several TMA grants for gene therapy research using the tissue building protein follistatin to treat IBM. His work at Nationwide Children's Hospital in Columbus, Ohio demonstrated increased muscle size and strength in a genetically-modified mouse model and subsequently in nonhuman primates and in humans. Mendell is currently conducting clinical trials using this innovative therapy with human subjects.

TMA supports established myositis researchers for promising projects as well. Former TMA Medical Advisory Board member Fred Miller, MD, PhD, for example, has received TMA funding for his ongoing work at the National Institute of Environmental Health Sciences at the NIH. His "Myositis in Military Personnel" study compared veterans who developed myositis with matched subjects who did not develop an autoimmune disease during active duty service. The project is looking for environmental factors that might trigger the development of inflammatory myopathies.



Malin Regardt - IBM hand study

“My association with TMA has been incredibly rewarding, both personally and scientifically,” Miller says. “It has had a critical impact on our and many others’ research over the last decade.”

Funding quality science

TMA takes very seriously the responsibility of funding the highest quality science. Each year, highly respected researchers from all over the world respond to TMA’s request for proposals, and many more applications are received than can be funded. Only the most promising projects are considered for funding.

As a very recent recipient of a TMA research grant, Malin Regardt knows it’s not just her own work that benefits from this funding.

This year, TMA received 15 applications for projects ranging from creating a viable mouse model for IBM to identifying a reliable biomarker for disease activity to conducting a multinational study of clinical outcomes. Each of these applications was thoroughly vetted by at least three independent reviewers, scientists who themselves are doing research in the field of myositis. TMA’s research committee, made up of nine members of the Medical Advisory Board, then reviewed the projects and identified three that will receive TMA support. These projects—two grants and one fellowship—total \$292,000 over the next two years.

“It was especially rewarding to meet the participants during the TMA patient conference,” Regardt says. “I feel proud to be able to write that my work was supported by TMA. This also means that it is of importance to the patients with myositis, who are, ultimately, the ones we are doing the research for.”

Over the years, TMA-funded research has advanced the science of myositis in the following areas:

PATHOPHYSIOLOGY

- ✚ Examined follicular helper T cells in myositis patients to determine their function in adult and juvenile patients
- ✚ Identified the symptoms, complications, and outcomes of patients with differing auto-antibodies
- ✚ Evaluated the role of inflammatory lung disease in the development of myositis in patients who have Jo-1 antibodies
- ✚ Investigated differences in initial symptoms and natural disease progression in IBM patients who test positive for a newly identified antibody versus those who test negative
- ✚ Identified the mechanisms for how myositis affects the muscles and skin in juvenile patients
- ✚ Characterized the range of proteins secreted by muscle in myositis to better understand the mechanism that results in underlying muscle weakness
- ✚ Studied the surface molecules of muscles in hereditary forms of IBM to understand and prevent the destruction and loss of muscle fibers

- ✚ Compared people who developed myositis during active duty military service and those who did not to find factors that might have led to their disease
- ✚ Identified contributing factors, in addition to inflammation, for muscle weakness in myositis
- ✚ Investigated the role of a protein released by white blood cells in the disease process of PM
- ✚ Studied the role of the loss of small blood vessels and consequent low oxygen content and weakness in muscles of myositis patients
- ✚ Investigated the role of specific immune system molecules that are part of the response to viral infection and their relationship to triggering DM
- ✚ Showed that the pathology of IBM muscle has several important similarities to Alzheimer's disease in the brain
- ✚ Identified the important role that misfolded proteins play in causing muscle degeneration in IBM

TREATMENT

- ✚ Examined adult stem cells associated with blood vessels and their potential for repairing damaged and inflamed skeletal muscle
- ✚ Analyzed clinical responses to therapies in JDM to better predict outcomes
- ✚ Performed initial research to support a study of using creatine supplements to ease muscle weakness
- ✚ Showed that muscle strength and inflammation in myositis patients could be improved by exercise
- ✚ Tested an assessment tool and exercise program to improve hand function in patients with IBM
- ✚ Developed an assessment tool to measure physical function in myositis patients

- ✚ Worked with IBM patients to see how methods of bracing could improve movement and stability
- ✚ Showed that treatment with follistatin results in larger and stronger quadriceps muscles in non-human primates
- ✚ Examined the effects of diet and exercise as well as effects of treatment with common anti-inflammatory drugs in mouse models of IBM

EPIDEMIOLOGY

- ✚ Developed a myositis research cohort following more than 200 patients to find out why cholesterol functions are abnormal in myositis patients and how this contributes to cardiovascular risk
- ✚ Created a clinical database and tracked follow-up data from a large population of JDM patients
- ✚ Evaluated clinical and biological data to develop a predictive model of clinical disease improvement in adult and pediatric DM
- ✚ Established an international program with long-term follow-up of patients with juvenile forms of myositis and their parents to discover factors that predict eventual outcomes
- ✚ Recruited juvenile patients from 46 countries to identify the best treatment with the least toxic effects
- ✚ Analyzed the association between age of disease onset and the role of genetic markers associated with the formation of calcium deposits, a serious complication associated with DM
- ✚ Performed gene sequencing of 79 patients with sIBM in order to discover new genetic relationships associated with the disease
- ✚ Identified differences in disease expression in Native American children with JDM

PIONEER seeks DM patients

People with dermatomyositis are needed to participate in a clinical trial known as PIONEER. Researchers are testing whether a new drug, IMO-8400, is safe and effective for improving DM-related skin lesions and muscle weakness. It is administered weekly through an injection under the skin.

IMO-8400 is an investigational medication, developed by Idera Pharmaceuticals, that is designed to block the activity of specific toll-like receptors (TLRs). TLRs are proteins that play an important role in the immune system. Researchers believe that blocking the activity of TLRs could potentially interrupt the cycle of inflammation and tissue damage in DM.

The study is underway at 25 sites around the world and is seeking 36 volunteers to participate in this randomized, double-blind, placebo-controlled, Phase II clinical trial. It is intended for adult dermatomyositis patients with active skin and muscle disease that is not well controlled with current treatment. Certain other criteria will apply.

For more information about eligibility criteria, screening, and participating study sites please contact Kate Tighe, Idera's Manager of Patient Advocacy at ktighe@iderapharma.com. Additional information is also available online at clinicaltrials.gov (search for NCT02612857) where you will find information on the trial recruitment status, eligibility, and locations.

Yale launches IBM registry

Many TMA members were among more than 900 patients with inclusion body myositis who participated in Yale University's 2012-2013 survey. Results of the study were recently reported in the journal *Muscle and Nerve* (available at myositis.org under "explore research" tab), and the data have now been organized into a registry that will help physicians, patients, and caregivers better understand the disease, its impact, and how best to treat it.

Researchers recently launched a website, *The Inclusion Body Myositis Disease Registry at Yale* [<http://ibm.yale.edu/>], designed to serve as an informational

resource for those with IBM and for professionals interested in the clinical and research aspects of IBM. The site offers a "Frequently Asked Questions" section, providing information on IBM diagnosis, symptoms, prognosis, what to expect and when. It also provides a confidential "IBM Personalized Index Calculator" [<http://ibm.yale.edu/ibmindex/>] that helps patients compare their personal experience with IBM to that of other people with the disease.

Part of the value of such a registry is the opportunity it offers to track disease progress over time. Unfortunately, the initial project only allowed for a one-time contact with the survey participants. In order to keep the registry up-to-date so it continues to serve as a valuable resource, patients are encouraged to join the registry [<http://ibm.yale.edu/getinvolved/>]. By signing on to the free and confidential registry, patients can learn about opportunities to participate in new scientific studies, find out about updates on the study website, and receive regular reminders to update the registry with the latest information about their disease progress and quality of life.

MYORISK seeks recently diagnosed patients

The National Institute of Environmental Health Sciences has launched the MYORISK study to investigate the genetic and environmental risk factors involved in the development of myositis, especially as they relate to antisynthetase syndrome. Adults and children diagnosed with myositis within the last year (whether they have symptoms of antisynthetase syndrome or not) are invited to participate in the study, which requires you to complete questionnaires and donate blood and urine samples. More information is available at <http://www.niehs.nih.gov/research/clinical/studies/myorisk/index.cfm> or <https://clinicaltrials.gov/ct2/show/NCT01276470>.

Vets with myositis join MVP

The Veterans Affairs Office of Research and Development is recruiting participants for the Million Veteran Program (MVP). Launched in 2011 as part of the White House Precision Medicine Initiative, this study seeks to create the largest

genomic database in the world. Researchers will use information gathered in this database to better understand how genes affect health and illness.

Participants are asked to donate a blood sample from which DNA will be extracted. Baseline and periodic follow-up surveys will track the veteran's military experiences, health, and lifestyle. As part of the program, participating veterans grant researchers secure access to their VA electronic health records and agree to be contacted about participating in future research. Samples and data used are coded to protect the participant's identity and privacy.

Research using MVP data is already underway, studying a range of medical issues such as mental illness and heart and kidney diseases. Because some scientists have suggested there may be a genetic or service-related link to myositis, especially inclusion body myositis, TMA encourages members who are veterans to enroll in the study. It's one more way to help accelerate our understanding of the detection, progression, prevention, and treatment for this troubling disease.

For more information about MVP, including how to participate, visit <http://www.research.va.gov/MVP/>.

SCIg study seeks DM patients

Many patients with dermatomyositis find intravenous immunoglobulin to be very helpful in controlling their disease. Dr. Marinos Dalakas and the Neuromuscular Division at Thomas Jefferson University in Philadelphia are currently recruiting patients for a clinical trial that looks at whether DM patients requiring continuous IVIg can be treated as safely and effectively with the subcutaneous form of this therapy (SCIg). Using SCIg would make this treatment less invasive and allow patients to administer it themselves.

The study aims to enroll 10 patients with DM who are not adequately responding to corticosteroids and those currently treated with and responding to IVIg. Interested candidates may contact Lauren Fedor, Clinical Research Coordinator II, at (215) 955-4663 or Lauren.Fedor@Jefferson.edu. Complete research information is available at <https://clinicaltrials.gov/ct2/show/NCT02271165?term=NCT02271165&rank=1>



Farewell to Theresa Curry

Since 1998, Theresa Reynolds Curry has served the myositis community through her work at The Myositis Association. As Communications Manager, Theresa not only has spread the word about myositis to the public and the medical community, but she often has been that warm, compassionate voice at the other end of the phone or email, doing her best to help and comfort those who have to live with myositis. Those who have been to a TMA Annual Patient Conference may not know that the success and value of this program to the patient community is mainly due to the hard work and careful thought Theresa has put into planning the program and recruiting just the right speakers.

"Theresa's contributions to TMA and its members are immeasurable, and we will miss having her kind spirit helping to guide TMA," said Bob Goldberg, Executive Director.

Linda Kobert has been working alongside Theresa for several months now and will be taking over Theresa's responsibilities, in addition to bringing her own medical background and expertise to the position. While we are confident Linda is an excellent addition to our staff, we will never truly be able to replace Theresa.

TMA wishes Theresa the best of everything as she moves forward in life and continues on her journey.



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