THE OUTLOOK

The Myositis Association

SUMMER 2016
Quarterly Newsletter

Corner Office

Bob Goldberg, Executive Director

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***

COVER PHOTO BY
JUSTIN "JMILLZ" MILHOUSE

Dear reader,

This issue of *The OutLook* marks an increased focus on research that we hope you will appreciate. As progress has been made toward better treatments through medication and various therapies, we recognize a need to communicate with you more frequently and specifically about myositis drug trials and developments that may help you manage your disease. In this issue, you will find more articles on recent research related to myositis and practical steps you can take through exercise, infection control, and medications to manage your disease.

TMA has also added a new staff position: Research Director. This new staff member will enable TMA to bring more treatment and research developments to your attention while also allowing TMA to work more closely with the physician and research communities to draw their attention to myositis and promote myositis-related research. On the next page of this issue of *OutLook*, you will find a profile of Linda Kobert, TMA's Research Director.

Many of you may recall that last year TMA provided $747,000 in new funding for myositis research—more than it has in any year of the past decade. This year, TMA is offering additional new funding of $500,000 for innovative myositis research. In response to TMA's call for proposals, we have received 20 funding requests, with about one-third of those coming from outside the US. TMA's Medical Advisory Board and Board of Directors will decide in September which proposals merit funding.

You should expect to hear more from TMA about research that we have funded and developments that may be important to you and your treating physician. If you ever have specific questions, you may send them to **tma@myositis.org**, or you can ask them in real-time during our periodic online chats with TMA Medical Advisory Board members. You can also send questions directly to our Medical Advisory Board members—their contact information is listed on TMA's website **www.myositis.org**.

I hope you are pleased with the news above, and let us know if there is ever anything we can do to help you manage your disease.

Bob Goldberg
Executive Director

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Announcing TMA's Research Director

TMA is pleased to announce the addition of a Research Director to its staff.

Linda Kobert worked as a registered nurse for more than twenty years. She graduated from the nursing program at St. Margaret Memorial Hospital in Pittsburgh, a community hospital that served as a regional referral center for rheumatology and rehabilitation. She earned her BSN degree from La Roche College and an MSN in nursing education from the University of Pittsburgh. She has taught clinical nursing at Western Pennsylvania Hospital and at the University of Pittsburgh.

Linda has also worked as a freelance writer and creative writing teacher for many years. Most recently, she has been a writer and editor for several publications of the University of Virginia. As a journalist, she uses her knowledge of science and medicine to help lay readers understand complex treatment concepts and scientific innovations.

In addition to being a liaison with the myositis research community and working with TMA's 20-member Medical Advisory Board, Linda will assist with the writing of research articles and other communications for TMA publications related to educating patients about myositis treatments.

Linda Kobert

Linda lives in Charlottesville, Virginia, where she will work remotely for The Myositis Association.

> *"Linda has a wonderful blend of clinical knowledge and writing skills," said TMA executive director Bob Goldberg.*
>
> *"One of her responsibilities will be communicating research news and developments to TMA's patient members. She will also be assisting me with work related to our research program, liaison with the myositis medical community, the Myositis Symposium, program and speakers for TMA's Annual Patient Conference, etc. This position will evolve over time and I believe it will strengthen the capacity of TMA in this most important area."*

Goal digger: Kori Winters finds purpose in DM

By Charlia Sanchez

In early 2012, Kori Winters was at the peak of her undergraduate career. She was a resident advisor at Howard University in Washington, DC. She was spearheading a major conference for her sorority, Delta Sigma Theta. Most importantly, the Detroit native was months away from graduating college. During this busy time, however, Winters caught a cold, which turned into the flu with lingering symptoms that would leave a much greater impact on her life.

Now a 26-year-old writer and digital content strategist, Winters recalls feeling relieved when that conference was over, but worried that her cold was still hanging on. One day, she received a care package in the mail from her mother—a standard-sized box containing snacks and treats that weighed no more than a few pounds. But as she walked the short distance to the administration building to retrieve the package and back to her dorm room, she felt as if she'd run a marathon. She went to see a doctor, but all of the test results came back normal.

"Most people would feel relieved to hear their tests were all normal," Winters said. "But I was more upset. I knew that something was wrong with me, and I needed to figure it out."

In March, while her friends went off to Miami for spring break, Winters went home to Michigan to seek additional medical attention. She was referred to a holistic doctor who took samples of her urine and saliva to test her body chemistry. The results indicated a lack of several important nutrients but could not provide a diagnosis. She was tired, depressed, and desperate for some answers.

"I needed to make it through the last two months of school to graduation, but some days I couldn't even get out of the bed," Winters said.

Kori calls herself a "goal digger," striving to reach new goals everyday.

Kori Winters
PHOTO BY IEISHA SELF

Journey to diagnosis

The holistic doctor suggested a strict diet and prescribed supplements and vitamins, a plan that Winters followed closely. Within a week, she felt better, had more energy, and felt more clear-headed. She went back to school feeling relieved that her health was finally beginning to improve.

But as she started to feel like herself again, Winters went back to enjoying some of the foods she had cut from her diet, including alcohol, as she celebrated with her friends their approaching graduation. Her original symptoms returned, and, worse, people started noticing that her face looked swollen.

"I remember vividly the night before graduation," Winters said. "I was trying on clothes with my mom, and I couldn't lift my arms up to dress myself."

Winters fought through this flare and still celebrated with her friends, but she felt worn out and swollen. By the end of her graduation party, she was so fatigued, she lay on the floor and could not get up.

Back home in Michigan, Winters developed a series of rashes, one of which spread like a butterfly over her face. A dermatologist took a biopsy and suggested that she may have lupus, because this butterfly rash

was characteristic of the disease. She was referred to a rheumatologist, but this new doctor could not see Winters for several months.

Meanwhile, her symptoms worsened. She started falling a lot, could not get out of bed, could not get out of the car without help, could not dress herself, and finally could not even speak. Tired of seeing her daughter suffer, Winters's mother drove her to Henry Ford Hospital in Detroit where the rheumatologist she was scheduled to see months later worked. After another battery of blood tests and a muscle biopsy, the rheumatologist gave Winters the results: she had dermatomyositis.

Winters was grateful to finally have a diagnosis, but now she had even more questions. She had never heard of this disease, had no family history of it, and learned that she had not caught it from someone else. Not for the first time, she wondered, "Why me?"

The road to recovery

On June 2, 2012, Winters started taking a high dose of prednisone daily. Her rheumatologist let her know about the adverse side effects of this corticosteroid drug, started her on Imuran, another immunosuppressant, and informed Winters that she may need to be on medication for a long time, possibly for the rest of her life.

"That wasn't an option for me," Winters said. "I don't believe people should be medicated forever."

So in addition to following her holistic doctor's advice about avoiding stress and negative feelings, Winters started on an anti-inflammatory and gluten-free diet, paying attention to what she ate and how it made her feel. She added foods such as ginger root, carrots, celery, and apples. She made fruit smoothies and fresh salads and drank homemade juices every day.

> ***Winters challenged herself to find ways to recover and maintain her strength and mobility, even if they were small.***

Kori Winters
PHOTO BY IEISHA SELF

And she still took her meds.

In July, Winters took a big step toward recovery. She attended the wedding of a friend with whom she'd previously fallen out, and afterward she was surprised to notice that her DM improved. It reminded her of what the holistic doctor had advised; making amends had allowed her to release that negative energy. After that, Winters challenged herself to find ways to recover and maintain her strength and mobility, even if they were small. She started to believe in the holistic doctor's words and kept her mind, body, and spirit in tune with positive energy and encouraging affirmations.

Back on track

Over the summer, Winters's creatine kinase (CK) levels dropped down to the normal range, and on Labor Day, she felt strong enough to attend a barbecue—her first outing, other than the wedding,

since her diagnosis. In November 2012, she got her first "real" job and started working full time as an online content coordinator. By the end of the year, she was able to stop the prednisone and started tapering off Imuran. By early in the new year, she was medication free. She travelled some and started eating gluten and drinking socially again. But when she felt a flare coming on, she immediately went to her rheumatologist who prescribed another round of prednisone. Now Winters is off the meds again, and she's careful to stick to her diet to avoid a relapse.

During the hardest times, Winters still depends on prayers and affirmations. The experience of dealing with dermatomyositis has taken a toll on her confidence, and she often looks in the mirror and reminds herself that she is beautiful, amazing, and has a purpose.

Part of that purpose is Winters's Free EGO, a clothing line she started during the summer of 2012 while she sat at home trying to recover. EGO stands for Empowerment, Gratitude, and Optimism, an affirmation that gives her inner strength and a vision for a future free of disease. She calls herself a "goal digger" and strives to reach new goals every day, whether it's walking a longer distance or coming up with a new design for Free EGO.

Beyond dermatomyositis

Now that she's more settled with myositis and conscious of how best to care for herself, Winters feels that the ordeal she went through with DM also has a purpose. She writes about her experiences on her blog, **www.accordingtoWinters.com**. She also contributes to **www.themighty.com**, an online community that features stories of people facing a variety of disabilities, illnesses, and other life challenges. Sharing her story, she feels, is a way she can help others in similar situations.

This year, she will also relaunch Free EGO and hopes to release new designs soon.

"During the hardest times, Winters still depends on prayers and affirmations."

The Winters Family: (left to right) Kori, her mother Andrea, father Mark, and sister Erin (22)
PHOTO BY BREE GANT

Read about Winters's experience on her blog: **accordingtoWinters.com**

Read Winters's contributions on **themighty.com**, an online community featuring stories about a variety of other life challenges, disabilities, and illness.

Get moving: Exercise advice from Dr. Helene Alexanderson

By Linda Kobert

Exercise is important for individuals with myositis. This was the message from Dr. Helene Alexanderson during an online discussion on March 17, 2016. A member of The Myositis Association's Medical Advisory Board, a physical therapist, and an associate professor of physiotherapy at the Karolinska Institute in Stockholm, Sweden, Alexanderson is an international authority on exercise for myositis patients.

During the live discussion, Alexanderson fielded questions about the effects of exercise on the range of myositis disorders, including polymyositis (PM), dermatomyositis (DM), and inclusion-body myositis (IBM), as well as those complicated by interstitial lung disease related to antisynthetase syndrome. She affirms that, regardless of the disorder, exercise has proven beneficial effects.

"There are many studies that confirm the safety and efficacy of exercise in patients with adult and juvenile idiopathic inflammatory myopathies," Alexanderson said. "In fact, intensive resistance exercise and/or aerobic exercise can even have an anti-inflammatory effect. Studies have demonstrated reduced impairment and activity limitation as well as improved quality of life."

Exercise and myositis

Generally, exercise guidelines for those with any form of myositis are similar to those for a healthy individual. The only caution is that myositis patients should always adapt an exercise program to accommodate their own disease activity, muscle strength, fatigue, and pain levels.

Muscle function and aerobic capacity can be improved, but Alexanderson advises patients with myositis to consult a physical therapist, at least when starting out in an exercise program or after a flare. She recommends beginning with low-intensity exercise and increasing duration and intensity slowly.

Building up in this way can actually build new muscle. While easy-to-moderate exercise can increase the number of endurance muscle fibers, more intensive exercise is needed to build the size of muscle fibers.

Building muscle can be especially important in some situations. Weakness in the neck muscles, for example, is often a problem. Alexanderson recommends that PM patients especially include head-lifting (lie on your back, lift your head as high as you can, hold for 5-10 seconds, repeat five times) in their daily routine.

Thigh muscles are another area of special concern as weakness may make it difficult to climb stairs or stand up from a seated position. Weak thigh muscles can also cause strain on the knee joints. Biking and working out with a leg press are ways to focus on these quadriceps muscles.

Functional exercises, such as standing from a seated position, climbing stairs, or walking uphill, can also be beneficial. High-impact activities, however, such as jumping or running, should be approached with caution.

Alexanderson cautions those on long-term treatment with corticosteroids, such as prednisone.

These drugs can make muscles and bones fragile, so individuals using these treatments should start off slowly. She recommends low-impact exercises such as walking or biking in a routine that raises the heart rate for at least 20 minutes three times a week. She also suggests performing resistance training, starting with a load that is low enough that you can complete 20 repetitions.

Interestingly, while this three-times-a-week recommendation applies to most people, patients with IBM are recommended to exercise twice a day. Alexanderson says it's unclear why this is the case, but she has seen improved function for IBM patients who exercise more frequently.

Special considerations for antisynthetase syndrome

While the lung involvement associated with antisynthetase syndrome creates special considerations, shortness of breath with exertion should not keep people from exercising. In fact, Alexanderson asserts that it is possible for patients with antisynthetase syndrome to do both aerobic and resistance training; one need only adapt the exercise to the current lung capacity and build from there. She recommends having a physical therapist assess aerobic capacity before starting an exercise program.

Any type of physical activity can be helpful—and enjoyable. An exercise routine should be incorporated into your daily activities. This can be as simple as getting out for a walk every day, starting with short distances in the beginning and increasing as capacity builds. Alexanderson also suggests varying the walking speed, starting slow, then speeding up for a minute or two, then slowing down again. Over time, try to increase the duration of the more intense, faster-paced walking. This approach can be used for any type of physical activity.

Additional resources

Myositis patients can find additional resources on the TMA website **www.myositis.org**. Those with IBM especially can find a home exercise program with specific recommendations. Patients are invited to share these routines with their physical therapist as well the **Functional Index 2 Myositis** evaluation tool found on YouTube. Alexanderson also invites therapists to email her (**helene.alexanderson@karolinska.se**) for additional information or specific exercise regimens.

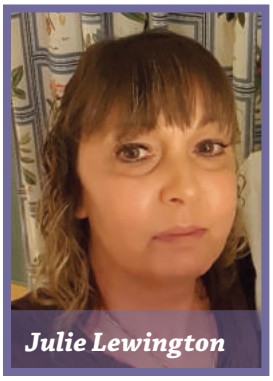
Regardless of the type of myositis, Alexanderson asserts one cannot get too much exercise: "As long as you feel well during and after exercise and improve in physical capacity, you should continue and increase your exercise over time. If you experience a flare, you will need to adapt exercise intensity, but you should still try to exercise and be physically active with walking."

Helene Alexanderson will offer several sessions discussing exercise for patients with myositis at TMA's Annual Patient Conference in New Orleans September 1-4. To register, go to the TMA website: www.myositis.org.

> *Any type of physical activity can be helpful—and enjoyable.*

Antisynthetase syndrome: Some stories

By Theresa Reynolds Curry


Julie Lewington

Julie Lewington thought she had the flu. Mike Peck thought he had asthma. Wayne Parnell thought he had a lung infection. All three got worse very quickly. Mike's fingers were turning purple, Julie got stuck in a bathtub, and Wayne could barely leave his bed. All three spent some time with prescribed antibiotics, pain killers, or asthma medications, trying to tough it out until the sheer gravity of their illnesses finally landed them in a hospital, where they were correctly diagnosed with antisynthetase syndrome, a collection of severe symptoms that includes dermatomyositis or polymyositis.

All were very aware their lives were threatened. Wayne heard the doctors saying that if the massive dose of prednisone didn't kill him, the disease surely would. Julie was asked to make a decision about intubation, and Mike was asked about his next of kin.

"This disease should be called 'total devastation,' instead of antisynthetase syndrome." Norma Mondragon wrote in an email. Norma emailed TMA in mid-May, after losing her husband. He was hospitalized for five weeks, without a diagnosis, before he died. The timing of Norma's email made TMA members' efforts to educate the public and the medical profession during Myositis Awareness Month seem even more urgent. Any sign of this syndrome should be taken very seriously.

What is antisynthetase syndrome?

Former TMA medical advisors Drs. Fred Miller of the NIH and Lisa Christopher-Stine of Johns Hopkins University recently fielded questions from patients in a podcast produced by the Rare Genomics Institute.

"It's called a syndrome because it generally groups a bunch of clinical features together," said Dr. Christopher-Stine. "So, when you ask how many different kinds of antisynthetase patients or different kinds of syndromes, we can answer it a few ways. You could look at the antibodies or the immune system proteins that are targeted in this syndrome. You may have heard of anti-Jo-1, for example. Others are PL-7, PL-12, EJ, and OJ. Those five are clinically testable in specialty laboratories.

"Other antibodies are more rare and generally not as often tested in a clinical laboratory. These would be anti-KS, anti-ZO, and anti-HA. Fewer people seem to express those.

"In general, the syndrome itself can be comprised of many things, which can include interstitial lung disease and myositis and then various other clinical features," she said. "Some patients have all of the features."

Mike, Wayne, and Julie all experienced the profound muscle weakness of PM or DM, but also experienced attacks on other major organs. Some common targets include:

- **Joint as well as muscle inflammation.** Arthritis is a common part of the syndrome.
- **Thickening of the lung lining, making it hard to breathe.** For the three patients we interviewed, this was the most dangerous symptom. Although not every patient with antisynthetase syndrome has lung disease, many will develop it.
- **Thickening and cracking of the hands,** known as "mechanic's hands." This distinctive skin sign looks just like you've skinned your knuckles badly over and over again.

Raynaud's syndrome is a very common complication that involves extremely reduced blood flow to the hands in response to cold or emotional stress. This explained Mike's purple hands. Julie and Wayne later developed Raynaud's as well.


Mike Peck

About a third of patients develop myositis before some of the other symptoms, about a third develop them all at the same time, and about a third develop the lung disease first before any other symptoms of myositis.

"It appears that the different antibodies have slightly different frequencies," Dr. Miller said. "So the Jo-1 patients, for example, which is the most common form of antisynthetase syndrome, tend to have more muscle disease, mechanic's hands, joint pains, and arthritis. The non-Jo-1 patients tend to have more fevers and the interstitial lung disease."

What causes antisynthetase syndrome?

Dr. Miller said that some hints as to what causes these diseases come from other researchers, with some evidence that cigarette smoking is a risk factor in people with certain genetic backgrounds.

One genetic factor is the HLA-8.1 haplotype gene. The human leukocyte antigen (HLA) genes are important in immune responses to different environmental agents. These genes are present in a number of different Caucasian populations.

"They are frequent and not really abnormal genes that people have," he said, "but there are different spellings of this gene and the spelling that seems to be a risk factor for the antisynthetase syndrome is the HLA-DR3, part of the 8.1 haploid type."

Another possible risk factor is having an occupation that provides exposure to dust and certain gasses or fumes.

"In fact, it's probably a combination of these genetic risk factors and certain environmental risk factors that together result in these diseases," Dr. Miller said.

Dr. Christopher-Stine and other researchers are involved in a study through the NIH that compares patients with antisynthetase syndrome with other myositis patients and with healthy controls. Patients who are interested can call the NIH recruitment office 1-800-411-1222 and ask to be referred to the MYORISK study.

How is antisynthetase syndrome treated?


Wayne Parnell

Between Mike, Wayne, and Julie, they've received almost every possible treatment. The traditional approach is to start with steroids. Current thinking calls for more aggressive steroid treatment early on. Additional medications may be prescribed, even from the very beginning of the disease: methotrexate, mycophenolate cyclosporine, tacrolimus, and intravenous Cytoxan, especially when there's lung involvement. A recent study of rituximab in myositis showed that those with the antisynthetase syndrome were especially responsive, and many people respond well to intravenous immunoglobulin (IVIG).

Drs. Miller and Christopher-Stine expressed excitement about growing interest from pharmaceutical companies in these previously neglected rare diseases and the developing knowledge of gene therapy as future sources of more effective, less risky treatment.

Wayne acknowledges that, although risky, the extremely high doses of prednisone saved his life: "I was up and walking within days," he said. "It was my own personal miracle."

Julie felt better after a few hours of her first steroid infusion; she also received Cytoxan. Mike was able to remain stable after the addition of methotrexate to his regimen. But all experienced some unwelcome effects from the massive steroids that saved their lives, including facial swelling and hip necrosis.

Dr. Christopher-Stine says that antisynthetase syndrome is absolutely treatable, and she and Dr. Miller offer this advice for the newly-diagnosed:

- **Do whatever you can to get to a tertiary care center** (a large teaching center) with experience in myositis. To find one, email TMA at **tma@myositis.org**.
- **Find out about myositis on TMA's website, www.myositis.org**.
- **Join a study**—the "MYORISK" study mentioned above or find another one at **www.clinicaltrials.gov**.
- **Find support.** Julie, Mike, and Wayne have benefitted from the antisynthetase syndrome closed Facebook group. Drs. Miller and Christopher-Stine recommend attending a TMA Annual Patient Conference; Mike attends every year.

Julie, Mike, and Wayne are much better than they were at the start of their diagnosis, and in all three cases, lung symptoms have been reversed. They also mentioned coming to a sense of peace about their disease: doing what they can to be upbeat and find a balance.

Dr. Miller agrees with this approach. "Exercise, maintaining weight, controlling blood pressure, avoiding stress, trying to maintain and enhance those close personal relationships that you already have or developing some if you don't, are important because being part of community is a very important step for all humans overall."

Synovitis

Many people with the syndrome experience this, including Matt Steven, who contributed to this story. Synovitis is a swelling of the joints caused by an inflammation of the joint lining. It can be very painful—much more painful, our members tell us, than it would appear from the outside.

*Dr. Christopher-Stine will speak on antisynthetase syndrome at TMA's Annual Patient Conference, September 1-4 in New Orleans. Dr. Dana Ascherman will speak on myositis and lung disease. Other sessions will include medications, exercise, skin symptoms, finding mind-body strength, caregiving, and coping. To register, go to **www.myositis.org**.*

*To read the whole transcript of Drs. Christopher-Stine's and Miller's podcast on antisynthetase syndrome, go to **www.niehs.nih.gov/research/clinical/assets/docs/antisynthetase_trasncript_508.pdf**.*

Don't bug me: Steering clear of infections

By Linda Kobert

Many myositis patients, especially those with poly- and/or dermatomyositis, are treated with immunosuppression therapy (IMT to control inflammation, ease pain, and increase muscle strength. Suppressing the immune system with drugs such as prednisone, methotrexate, azathioprine, or cyclosporine, however, can leave you vulnerable to a variety of infections.

Infection is a serious side effect of IMT. A research study that assessed the disease process in dermatomyositis patients, authored in part by TMA Medical Advisory Board member Mazen M. Dimachkie, MD, noted that 5% of the patients whose profiles were reviewed for the study died from infections.

Early detection of an infection is important, according to TMA Board member and infection control specialist Dr. Marvin Lauwasser. Doctors monitor patients closely for this complication through blood tests and other means. But patients can also play a role by reporting signs of infection immediately. This includes symptoms of sore throat, cough, difficulty breathing, fever, chills, painful or difficult urination, diarrhea, nausea and/or vomiting, dizziness or malaise, and unusual skin rashes.

Infections are usually preventable. Doctors often try to prevent bacterial, viral, fungal, and parasitic infections by, for example, giving the patient a flu shot or pneumonia vaccine. And they always treat signs of infection aggressively in patients taking IMT.

But individuals—even those who aren't on IMT—can also take steps to prevent the complications of infections such as colds, flu, wound infections, pneumonia, gastroenteritis, and other infectious diseases. Here is a rundown of prevention strategies:

- **Wash your hands.** Nothing prevents the spread of infection better than vigorous, frequent hand washing.
- **Pay attention to skin wounds.** Wash the wound immediately with soap and water and apply a bandage with antibiotic ointment. See your doctor if the area develops redness, swelling, foul-smelling discharge, or doesn't show signs of healing.
- **Avoid contact with others who are sick.** This may include avoiding crowds and close public spaces. It also includes using safe sex practices.
- **Avoid tick and mosquito bites,** which may transmit infections such as Lyme disease and West Nile virus. Use insect repellant and cover your skin when out of doors during the summer, especially in the evening.
- **Avoid foods that may carry bacteria,** including raw eggs, unpasteurized milk, and insufficiently cooked or raw meat. And always wash raw vegetables before eating them.
- **Be aware of unsafe drinking water,** especially if you travel outside the US. Drink only bottled water while away, use it for rinsing your toothbrush, and don't eat or drink anything that has not been cooked or peeled.
- **Discuss travel-related infections with a specialist** if you plan a trip to developing countries where certain infectious diseases, such as yellow fever and malaria, are prevalent.


Nancy Harber

Exercise: Yes, no, or maybe so?

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.

When Charlie and I first started on this journey, we had the same concerns that many of you do: What about exercise? Will it help? Will it create more problems? It wasn't that Charlie was an exercise freak, but he loved to swim (was state champion in college many years ago), he did lots of work outside, and we owned a wonderful get-away cabin in the mountains east of San Diego where he loved to hike through the meadows, down to the lakes, and up the hills.

As we began to research this disease and talk to knowledgeable people, the general consensus then was exercise in moderation was good, but don't overdo it. "No pain, no gain" was not the word of the day for my husband. Still, Charlie decided he wanted to try to use his muscles while he had them.

One of the first things he discovered was that the San Diego Community College District had a water class geared for people with physical challenges, held at the public pool near San Diego Bay. A physician's clearance was required, but was easy to get. The wonderful thing about this pool was that it featured a chair lift into the water, and the staff who led the class knew how to use it. For almost a year, Charlie went there on a weekly basis. The mild exercise seemed to help him maintain his strength, and it certainly brought back lots of memories of a sport he had loved. It's worthwhile checking local programs to see what might be available in your community.

Eventually, Charlie had trouble getting in and out of the pool even with the lift, so he stopped going to the swim class. But we went to the beach occasionally, and there he discovered the benefit of sand. Charlie had a creative mind, and on one trip he took two clean, empty 16-ounce Coke bottles he had saved. We filled them with sand from the beach and brought them home. Now he had a pair of weights to exercise his arms—for free!

Charlie had also lost strength and dexterity in his fingers. It's difficult to just sit and keep squeezing your fingers together, so I got a couple of small, soft rubber balls, and he would sit and squeeze them while watching TV. We think it helped some. When his hand strength continued to deteriorate, however, and he lost most of his dexterity for using the computer, he got a voice-activated program so he could dictate what he wanted to write and then go back and make simple corrections with one finger.

In our local support group, we've had two individuals who were able to retain the services of a personal trainer. They both are convinced that this has helped them stay as strong as they are and, if you have the resources, this might be worth exploring.

Today the pendulum has swung more clearly in favor of as much exercise as you can handle. If this helps slow the loss of muscle strength, great. Equally as important, however, is that exercise will help strengthen other complementary muscles that can pick up the loss and do double duty for the damaged ones. This also helps protect muscles that aren't yet affected.

I suspect exercise will always be a topic of discussion: how much is too much? Your body will probably be the best judge. If exercise exhausts you and causes pain, then slow it down, but don't quit. Too many people have gotten real benefits from trying. Also, once you adopt a more sedentary life style, sitting most of the day in a chair, weight gain can become a problem. The heavier you are the more potential there is for skin breakdown and the harder it is for your caregiver to help move you around. Exercise can play a part in overall body tone and brings benefits in more ways than you might expect.

Karyn Rizzo

Making physical therapy work for you

By Karyn Rizzo

Karyn Rizzo is the author of* Aging in America: Navigating our Healthcare System. *She will speak at TMA's Annual Patient Conference in New Orleans on September 1-4.

We've known for a long time that people who regularly engage in some type of physical exercise benefit both emotionally and physically. Physical therapy (PT) and occupational therapy (OT) can be especially helpful for patients who are managing myositis. Not only has PT been proven to reduce falls and strengthen muscles, it also provides socialization and emotional wellness and can help the myositis patient manage the depression that so often accompanies chronic disease.

Paying for PT and OT services

Receiving physical and occupational therapy begins with an order from a physician and can be arranged in a private home setting or at an outpatient clinic.

For patients who have Medicare or a Medicare Advantage plan, these treatments are covered under Medicare Part A. Some Medicare Advantage plans limit the number of visits allowed, however, and may require out-of-pocket copayments. Most outpatient therapy centers can coordinate payment plans to enable the patient to continue treatment.

Private health insurance plans nearly always cover these services when a physician indicates that it is medically necessary. These plans may have restrictions on the amount they will pay, however, and they often include out-of-pocket copayments and restrict the number of sessions covered.

TMA can help supply your physician with information about the benefits of physical therapy for myositis patients.

Patients with no insurance coverage can still enjoy the benefits of these therapies. It is best to do some research into privately owned PT and OT centers (not hospital-based) in your area. These clinics are usually run by therapists who are more likely to negotiate an affordable fee for sessions and/or offer cost-effective packages for multiple sessions.

The website **health.costhelper.com/physical-therapist.html** offers some typical charges for PT and OT treatments, which can range from $50-$350 per session. Many centers offer discounted packages and different payment option plans, and some will negotiate a Medicaid rate per session, if asked.

TMA has many resources for members interested in physical therapy, including live discussions on exercise and myositis, videos of conference presentations on exercise, and links to peer-reviewed research articles. Find them at **www.myositis.org/learn-about-myositis/treatment/physical-therapy**. (If this long string doesn't work for you, go to TMA's home page at **www.myositis.org**; click on "Learn about myositis," then "Treatment," then "Physical therapy.")

The American Physical Therapy Association maintains a web-based locater that will point you towards a therapist in your area. Make sure to specify that you are looking for a specialist in muscle disease: **aptaapps.apta.org/findapt/index.aspx**

Alternatives to the PT/OT clinic

Exercising doesn't always need to be done under the guidance of a physical or occupational therapist. You can carry out your own exercise plan (perhaps after an initial consultation with the therapist), or you can engage with family members or neighbors, or take classes in the community or even in your own home.

In addition, many senior centers partner with the Silver Sneakers program, which offers regular exercise classes for free or minimal cost and is an affordable way to stay active.
www.silversneakers.com

All people tend to decline physically when they quit moving, so engaging in any type of exercise for at least 30 minutes a day can increase your overall health and wellness. As an extra bonus, exercise also provides stronger emotional resilience. Making time for consistent physical activity becomes crucial in any health and wellness care plan, but it is especially important for those managing a chronic disease such as myositis.

Aging in America: Navigating our Healthcare System ***is available from Amazon, Nook, or Kindle, and at www.agingguidebook1.com.***

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KITs celebrate Myositis Awareness Month, May 2016

By Charlia Sanchez

This May, TMA recognized the first international Myositis Awareness Month to raise awareness among patients, physicians, and the general public. Several individuals and support groups participated by hosting events and fundraisers, conducting outreach to physicians, and educating their friends and families.

TMA Keep in Touch (KIT) support groups are the pulse of our organization, and volunteer group leaders serve as ambassadors for the myositis patient community. Katherine Falcone, a DM patient, is one of these KIT leaders for the New Jersey Support Group. In a Myositis Awareness Month event, she served as a guest speaker at a "Lunch and Learn" program for Idera Pharmaceuticals in Pennsylvania. Falcone shared her journey with Idera's staff—from the difficulty of diagnosis to her current IVIG treatment regimen. The Idera team wanted every detail of Falcone's experience and were genuinely interested in her opinions, as the company is launching a clinical trial of a new drug, IMO-8400, for treating dermatomyositis in adults.

"They wanted to know what it was like to live with dermatomyositis, and they wanted to relate to patients," Falcone said. "They are compassionate people and really passionate about their work and helping myositis patients."

The Southwest Florida KIT group hosted a Bring-A-Friend Awareness Event in which 60 patients and 15 "friends" participated. Instead of the standard guest speaker format, this special meeting featured a few brave members talking about their particular forms of myositis. They educated not just the "friends" who joined the meeting but also other patients with different myositis diagnoses.

At this event, Sue Prive (IBM) and Joanne Carnevale (PM) both discussed their limitations that keep them from being the kind of grandmothers they want to be.

"Myositis makes it hard for them to play with their grandchildren and pick them up and be as active as they once were and want to be," reported Marianne Moyer, the group's founder and current leader. "This hit home for many of the members. It was a sad 'aha' moment for us."

Another speaker, Jan Marriott (DM) shared a humorous story about her difficulty being diagnosed and how her doctor initially suggested she get more sun to help with her rashes.

Wayne Prive, Sue's husband, was the only caregiver willing to speak on his experience.

"We had difficulty getting our members to agree to speak to the group," said Moyer. "People thought their stories were either too sad or not sad enough and were not so willing to be seen as the example of their disease. Those members' willingness to take that risk is a testimony to the way that our group is not just a group, but a family."

The Carolinas Support Group (Charlotte, NC) wanted their Myositis Awareness Month event to be truly different. Co-founders and co-leaders of the group, Alison Alexander and Brenda Austin, invited guest speaker Dr. Lisa Criscione, a rheumatologist and founder of the myositis clinic at Duke University, to talk about her experiences treating and studying patients with dermatomyositis, polymyositis, and antisynthetase syndrome. More than 30 attendees came to the catered event. Dr. Criscione shared the history of studying myositis, 20th century advances that led to the classification of the different forms of the disease, as well as additional facts about treatment practices and studies on the effectiveness of exercise.

These are just a few ways that TMA's members and support groups joined together to recognize Myositis Awareness and make May a special month. Groups or individuals who want to keep the momentum going can find lots more ideas for spreading the word about myositis in the "Get Involved" section on the TMA website (**www.myositis.org**).

Research Review: Current research of interest

Take methotrexate with great care

For patients with myositis, oral methotrexate is often the preferred disease-modifying antirheumatic drug, and it has been a lifesaver for many.

The most typical methotrexate prescription is for a small amount taken weekly, and the dosing schedule is often adjusted for best results, with doses up to about 25 mg weekly. Most people tolerate methotrexate well in these doses, and most are also aware that their physician is carefully monitoring them for signs of toxicity.

Harmful or fatal errors with low-dose oral methotrexate are consistently reported to the Institute for Safe Medication Practice and published in the Institute's newsletters. In a recent review, here's what the Institute found about adverse reactions:

> *"Most errors involved accidental daily dosing of oral methotrexate that was intended for weekly administration. In 2004, we published a study of methotrexate errors over a four-year period that resulted in 25 deaths and 48 serious outcomes, many due to daily dosing."*

The Institute reported on three more recent cases of severe harm or death in patients taking low-dose methotrexate. One was the familiar scenario: a patient took the prescribed medication daily instead of weekly. The other two cases involved patients who were taking no more than 20 mg of methotrexate weekly, yet they died of severe methotrexate toxic effects due to other risk factors, including drug interactions that increased the concentration of methotrexate in their blood.

Pregnant and nursing mothers—and even women and their spouses who plan to get pregnant—should never take methotrexate. The American Academy of Family Physicians advises family doctors monitoring patients taking methotrexate to take careful histories, especially of past liver disease, or current or past alcoholism or drug abuse. Obesity, use of over-the-counter pain relievers, and increased age also factor into your doctor's decisions.

Make sure to let your doctor know if you are taking sulfa-based antibiotics such as Bactrim or Septra, aspirin or other NSAIDs, barbiturates, phenytoin for seizures, retinoids (usually used for skin conditions), oral sulfonylureas for type 2 diabetes, or tetracycline. If you are already on methotrexate and you're prescribed any new drug for an unrelated condition, be sure to discuss it with your physician.

Bimagrumab fails to meet primary endpoint

Drugmaker Novartis announced in April that the phase 2b/3 study examining bimagrumab (BYM338) in inclusion-body myositis did not meet its primary endpoint. The endpoint was a change, after a year of treatment, in the results of a six-minute walking distance test. The news is a significant disappointment to those with IBM and their families, who have been anxiously watching the progress of the trial and hoping for the drug to be released onto the market.

The drug, a human monoclonal antibody administered by injection, was developed to treat pathological muscle loss and weakness by tamping down myostatin, which inhibits muscle growth. BYM338 was designated by the FDA as a "breakthrough" drug because of early promising results. Several hundred IBM patients enrolled in 43 locations in

the US, Europe, Japan, and Australia, with many reporting personal stories of good results.

The company did not report on the results of its drug on the secondary endpoints: fewer falls, improvement in physical function, stronger quadriceps, and increase in lean body mass.

At the end of June, Novartis discontinued administering the drug to all study participants. Although some individual patients seemed to be experiencing benefits from the treatment, the evidence to support continuing the current trials or planning further trials was lacking. For excerpts from the Novartis bulletin explaining the trial's discontinuation, see TMA's home page at **www.myositis.org**.

Arimoclomol shows promise in mouse trials

A new drug to treat inclusion-body myositis (IBM) reverses key symptoms in mice and is safe and well-tolerated in patients, finds a new study led by the Medical Research Council (MRC) Centre for Neuromuscular Diseases at University College London and the University of Kansas Medical Center.

The study, published in *Science Translational Medicine*, found that the drug arimoclomol reversed the disease's effects at the cellular level and improved muscle strength in mice. A safety trial in 24 IBM patients conducted in London and Kansas found that the drug was safe and well-tolerated.

In this study, the research team pursued a new treatment approach based on observations that muscle tissue from IBM patients contains many misfolded proteins. The research team reported results using an integrated investigational plan of arimoclomol to clear these proteins out by either refolding or eliminating them.

The team started by creating cells in a petri dish that mimic the muscle tissue of IBM patients and successfully tested arimoclomol on these cells. They then used genetically modified mice whose muscle cells and symptoms closely resembled the human disease. An arimoclomol trial in these mice found that it was well-tolerated, reversed key features of the disease, and importantly, improved muscle strength.

After a successful patient safety trial, the research team has obtained $1.6 million from the US Food and Drug Administration to begin a full-scale, randomized, controlled clinical trial to formally assess if the drug is effective in slowing disease progression in people with IBM. Recruitment for this study is expected to begin this fall.

DM patients needed for trial

A new study will examine the safety and efficacy of IMO-8400 in adults with dermatomyositis who have confirmed skin and muscle involvement. Recruitment has started in Arizona, with other centers soon to begin recruiting. For information, go to **www.clinicaltrials.gov** and search for "IMO-8400," or call the Idera Study Monitor at **1-877-888-6550, ext. 2** or email **patientinfo@iderapharma.com**. Please let TMA know if you have difficulty obtaining information on this study.

Precision medicine

"We're all zebras," say representatives of rare disease groups, applauding the progress of the Precision Medicine Initiative, announced by the White House in 2015, and now ready to recruit citizen-volunteers.

The "zebra" refers to the popular slogan professors use to teach medical students that the most common diagnosis is most often the most likely: "If you hear hoof beats, think of a horse, not a zebra." That is, if the patient has a headache, think tension not tumor.

Precision medicine is a change from that approach, using our increasing knowledge of the way in which genes and environment interact to affect each person's health individually.

Those committed to solving the puzzle of rare diseases believe the Initiative will give them a huge body of knowledge to help them figure out the ways in which heredity, lifestyle, diet, and toxic exposures

contribute to disease, to develop better treatments and to make risk less inevitable.

Everyone—not just those with rare diseases—will benefit from knowing a little more about themselves, so the Initiative will recruit more than a million people, of all ages and degrees of health. A healthy person might find out, for example, that he or she has a high risk for diabetes in the future and be able to change the outcome. Another large cohort of volunteers will come from the Veterans Administration. The benefits will be both better health and less expense.

"The power of precision medicine is not simply to apply top-line technologies to previously unsolvable medical problems, but to create the most cost-effective solutions for our patient populations who disproportionately need health care resources," says Dean Li, MD, PhD, associate dean for research at University of Utah Health Sciences. Li leads a public-private coalition dedicated to quickly translating information gathered by the Initiative into appropriate research.

Patients with rare diseases, often caused by unknown and multiple genetic mutations, bear a disproportionately heavy health burden. Currently, there are no drugs available to treat 95 percent of patients with such diseases. Though by definition each rare disease strikes no more than 200,000 people, as a group these conditions affect 10 percent of the U.S. population.

Find out more: **www.nih.gov/precision-medicine-initiative-cohort-program/scale-scope**.

All in the family: Hereditary IBM

TMA has members with hereditary inclusion-body myopathy (hIBM) and, at first glance, it's hard to distinguish them from people with sporadic IBM (sIBM). Hereditary IBM is much more rare than sIBM, and those with both diseases face many of the same challenges.

Hereditary IBM is a unique form of chronic neuromuscular disease that involves slowly progressive muscle weakness and muscle cells that show rimmed vacuoles and filamentous inclusions, the same as are typically seen in sIBM. Unlike sIBM, however, hIBM does not result from an autoimmune inflammatory process. Rather, it is caused by a genetic mutation, often affecting individuals of Middle Eastern descent.

While onset is often earlier for hIBM (20s-40s versus 50s-60s in sIBM), symptoms and prognosis are similar to sIBM. Early signs may include foot drop, difficulty running or walking, loss of balance, and tripping. Weakness and muscle wasting progresses over time and may involve the hand, shoulder, and neck muscles. The weakness and severity can vary from person to person, but like sIBM, it often leads eventually to marked disability.

It was noted in the recent live discussion with Dr. Andrew Mammen (**www.myositis.org/your-myositis-community/live-discussions/517-inclusion-body-myositis**) that several questions came from people diagnosed with sIBM who had a family history of the disease. Dr. Mammen suggested, however, that if there's a familial pattern involved, the disease is probably not sIBM, which does not run in families.

Neurologist Margherita Milone of the Mayo Clinic is a current member of the TMA Medical Advisory Board and has studied the electromyographic and clinical differences between hIBM and sIBM. She will speak about hIBM at TMA's Annual Patient Conference in New Orleans. Former TMA MAB members Drs. Anthony Amato, Alan Pestronk, and Tahseen Mozaffar are also involved in researching this rare form of IBM, as are a number of former TMA research grantees, all of whom are working to discover treatments and a cure.

The Myositis Association

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There's still time!

Be sure to check TMA's website (**www.myositis.org**) for the most current agenda and conference updates.

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