THE OUTLOOK

THE MYOSITIS ASSOCIATION

Annual Patient Conference Page 10

Education, research, and support

FALL 2015
Quarterly Newsletter

Corner Office

Bob Goldberg, Executive Director

The mission of The Myositis Association is to:

- *Provide support to myositis patients and their families*
- *Provide connections between the Medical Advisory Board and the general medical and patient community*
- *Increase funding to support myositis research*

Dear reader,

Periodically, we are asked to list in *The OutLook* those who have myositis and have died recently. We do not do this because, in some cases, there are members of TMA who die and we do not learn of their death until months later. Rather than slight anyone and because of the limited space of this newsletter, we choose to not list those from among the TMA membership who have passed away.

However, there were two deaths of members that occurred this past summer that I do want to acknowledge because of their outstanding contributions to TMA.

Andrea Langworthy died on June 5, 2015 at age 67. She had inclusion-body myositis. Many of you may recognize the name because Andrea was a columnist for this newsletter the past year as well as leader of TMA's support group in the Minneapolis-Saint Paul area. Andrea was a trailblazer and strong advocate for those with limitations. In 1976, she became the first full-time car saleswoman in the Twin Cities, and she was a columnist for the Rosemont Town Pages beginning in 2004. Andrea was diagnosed with IBM at age 55 and continued selling cars until she was no longer physically able to navigate the showroom and car lot. She took over leadership of TMA's support group in Minneapolis several years ago, and most recently began writing interesting, challenging articles for *The OutLook*. She will be greatly missed by those who knew her. An example of how she touched the lives of so many is that until her death Andrea handwrote notes on her holiday cards every year to all those on her holiday list — over 1,000 friends!

Another member who passed away this summer is Roger Stevens. Roger was a self-made man of wealth who owned several construction companies in the New York City area. He was a patron of the arts in the Westhampton Beach, NY area where he lived. However, Roger will be most remembered at TMA as someone who had inclusion-body myositis and was the driving force behind the follistatin gene therapy trial now being conducted at Nationwide Children's Hospital in Columbus, OH. Roger was diagnosed with IBM in 2002. He contacted TMA in 2003 and challenged the organization to raise more funds for research. Roger sponsored the Matching Research Challenge the following year, offering to match all research donations for one year. The result was nearly an additional $500,000 for research. Some of those funds were used by TMA to fund the follistatin gene therapy trial and Roger contributed directly to Nationwide Children's to see that the trial, which cost over $1 million, got started. Roger and his family also hosted a benefit concert by a Broadway performer in Westhampton Beach in 2005 that raised nearly $100,000 for TMA.

Roger was an environmentalist and an animal lover. He was as generous to those who knew him as he was to the causes he cared about. TMA would not be where it is today were it not for the selfless contributions Roger Stevens made to this organization.

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Philanthropy: Giving is a family value for Thorpes

A. Skidmore ("Skid") Thorpe had many careers over his long, productive life. He was an architect, a stockbroker, and a real estate developer. Daniel Thorpe, born when Skid was in his 50s, doesn't remember a time when his father didn't have inclusion-body myositis. "He didn't let it affect his attitude in the least," Daniel said.

Skid designed a home that would accommodate him and, after retirement, looked after the family interests. He was a wonderful dancer and lifelong sportsman. Friends noted at his death in Wayzata, Minnesota in 2012 that he played golf until he could no longer grip a club.

Skid married Daniel's mother, Edith Davenport, in 1975. She also had many interests, including golf and singing. But really, said Daniel, her profession was philanthropy. Edith was president of the Thorpe family charity, the James R. Thorpe Foundation, for 25 years. "That was where her heart really was," Daniel said, "and she made sure the rest of us knew the importance of charity." The Foundation continues to help countless numbers of deserving youth and seniors. Edith led the board from 1983 to 2008, emphasizing the importance of site visits, as well as directing the development of a website and a succession plan for the board.

Edith had grown up believing in her obligation to participate in this type of charity. For 20 years she was also a trustee of the Surdna Foundation in New York, founded by her great-grandfather, John E. Andrus. The private foundation supports innovative ideas for sustainable communities in the United States, guided by social justice, with healthy environments, strong local economies, and thriving cultures.

Besides the obvious benefits to the community, his mother's passionate beliefs serve as a constant reminder to family members of their responsibilities, Daniel said. In fact, he said, family members have always staffed the James R. Thorpe Foundation; and members of each new generation are expected to work to make sure the foundation's philanthropy is as effective as possible.

At Skid's death, the family directed memorials to The Myositis Association, but Edith also wanted the family to make personal donations. She asked the children — which includes Daniel's five step-brothers as well as himself — to follow the family example of personal charity. "I'm not sure 'asked' is the right word," Daniel said, "we were 'strongly encouraged.'"

Edith died a few years after Skid, leaving a large bequest to TMA; but the family's belief in philanthropy continues, Daniel said, with a new foundation formed as a way for some of the younger nieces and nephews to get involved in philanthropy.

The generosity of the Thorpe family and many others enabled TMA to triple its funding for research in 2015. ●

Where do I turn?

Finding and paying for long-term care

By Karyn Rizzo

In this issue we introduce the first in a series of occasional columns by Karyn Rizzo, author of *Aging in America*. Karyn will talk about negotiating the confusing world of public and private insurance benefits. She spoke at TMA's 2015 Annual Patient Conference. Watch for a video of her session to be posted at www.myositis.org.

Long-term care is on the minds of many people, regardless of their current health situation. Deciding what direction to go when choosing a long-term care provider can be very confusing. Now that more people are living longer, we're all facing decisions that our parents and grandparents never had to make in the past. What programs are available to help pay for long-term care? In a nutshell, unless you are able to benefit from a long-term care policy purchased early in life, the national and state programs are your best options to pay for care.

If you are disabled

Once a person is receiving disability benefits, or is 65 years and older, Medicare can offer skilled nursing and physical, occupational and speech therapies in wherever a person calls home. This does need to be ordered by a primary care physician, and is one way to receive the care needed at no charge.

To apply for Social Security disability online, go to **www.ssa.gov/disabilityssi**.

There are four steps in the online disability application process:

This process takes about two hours.

1. **Background Information**
2. **Disability Information**
3. **Medical Release**
4. **Confirmation**

If you are a veteran

For those who have served in the military, and have been honorably discharged, there are some low-to-no-cost benefits and programs available. The "Aid & Attendance" benefit and many other benefits may cover private duty help in the home and assisted living when needed. There is an asset cap to these programs; however, if a veteran or spouse has low or little assets, the County Veteran Service Officers can help apply for benefits at no charge.

To find your Veteran Service Office, go to the National Association of County Veteran Service Officers at **www.nacvso.org/find-a-service-officer**.

To find a low-cost service to help with the application of benefits, contact an elder veterans legal aid group at 800-878-2149. If there are over $80,000 in assets, you may not be eligible for this service. To find an elder law attorney in your area, go to **www.naela.com**.

For information on the VA Health System and to meet with a benefits officer, go to **www.VA.org**.

To apply for Medicaid

Many states offer long-term care through Medicaid, and this can be an option for many needing private duty assistance in the home and/or assisted living. In some states a "personal needs trust" can be established by an elder law attorney, thus qualifying for Medicaid benefits to pay for needed care.

If Medicare recipients are living strictly on their SSI or SSD income, they most likely qualify for Community Medicaid and other state programs. To apply for Medicaid, the application can begin online. Go to **www.medicaid.org** and enter your state.

TMA has resources for veterans. See the presentation included in the Conference proceedings at **www.myositis.org**, linked from the home page; and TMA's Veterans' Facebook and forum pages. If you are a veteran and have specific questions, email **tma@myositis.org**. ●

Researchers review IBM best practices

The Cochrane Collaboration is an independent, non-profit, non-governmental organization made up of more than 37,000 volunteers in 130 countries. The collaboration was formed to organize medical research information in a systematic way to inform the choices that health professionals, patients, policy makers and others face in health interventions according to the principles of evidence-based medicine.

The group conducts systematic reviews of randomized controlled trials of health-care interventions, which it publishes in The Cochrane Library.

Neuromuscular specialists, including Drs. Michael Rose, Katherine Jones, Kevin Leong, Maggie Walter, James Miller, Marinos Dalakas, Ruth Brassington, and Robert Griggs, reviewed the evidence from clinical trials about the effects of drug treatments for inclusion body myositis (IBM). Their review did not include trials of exercise or management of swallowing difficulties, as these are subjects of other reviews. Researchers report on what they found:

The review included 10 trials (249 participants). One of these trials was completed but has not yet been published; seven trials compared treatment with placebo (inactive treatment): three were trials of intravenous immunoglobulin (IVIg), two of interferon beta-1a; and one each of oxandrolone, methotrexate, and arimoclomol (not yet published). Two additional trials compared methotrexate with combined immunosuppressive therapy and with an agent that destroys white blood cells (ATG); and with azathioprine. In these two trials, participants and investigators knew which treatment participants were receiving, which could have biased the results.

Results and quality of the evidence

For the primary outcome, which was muscle strength, they were only able to combine the results for the two trials of IFN beta-1a therapy versus placebo. This treatment did not appear to offer a benefit in terms of muscle strength. Methotrexate also did not stop or retard loss of muscle strength when compared to placebo. Evidence from these trials was considered to be of moderate quality because the trials were too small to rule out a possible benefit for these drugs. For the other trials, the evidence was of very low quality. Three trials compared IVIg (combined in one trial with prednisone) to a placebo, but were unable to perform meta-analysis because the available data were not suitable. One trial of ATG combined with methotrexate versus methotrexate alone provided very low-quality evidence of an effect on muscle strength in favor of methotrexate plus ATG at 12 months. The other comparisons, of methotrexate versus placebo, oxandrolone versus placebo, azathioprine combined with methotrexate versus methotrexate alone, and arimoclomol versus placebo were reported in single trials that did not provide enough data for analysis of the effect on muscle strength.

Due to their small size and short duration, the trials studied were generally unable to give definitive answers as to whether the treatments tested were effective. All of the interventions studied had some adverse effects and are known to cause potentially serious adverse events. Larger trials of longer duration are needed, using robust ways of measuring the effects of treatments that are meaningful to people with IBM. Agreeing on common trial measurements will also make it easier to compare trial results and assess potential treatments.

The evidence is current to October 2014. The international researchers involved discussed their findings, as well as other strategies for managing IBM, in London on September 30, 2015. ●

Apple and tomato components prevent muscle loss

Scientists at the University of Iowa have discovered the first example of a protein that causes muscle weakness and loss during aging. The protein is a factor that alters a gene, with the result of causing reduction of strength and mass. The Iowa study also identifies two natural compounds, one found in apples and one found in green tomatoes, which reduce the protein's activity in aged skeletal muscle. The findings, which were published online September 3 in the Journal of Biological Chemistry, could lead to new therapies for age-related muscle weakness and atrophy.

"Many of us know from our own experiences that muscle weakness and atrophy are big problems as we become older," says Christopher Adams, MD, PhD, professor of internal medicine and senior study author. "These problems have a major impact on our quality of life and health."

Previously, Adams and his team had identified ursolic acid, which is found in apple peel, and tomatidine, which comes from green tomatoes, as small molecules that can prevent acute muscle wasting caused by starvation and inactivity. Those studies set the stage for testing whether ursolic acid and tomatidine might be effective in blocking aging, identified as the largest cause of muscle weakness and atrophy.

In their latest study, Adams' team found that ursolic acid and tomatidine dramatically reduce age-related muscle weakness and atrophy in mice. Elderly mice with age-related muscle weakness and atrophy were fed diets lacking or containing either 0.27 percent ursolic acid, or 0.05 percent tomatidine for two months. The scientists found that both compounds increased muscle mass by 10 percent, and more importantly, increased muscle quality, or strength, by 30 percent. The size of these effects suggest that the compounds largely restored muscle mass and strength to young adult levels.

"Based on these results, ursolic acid and tomatidine appear to have a lot of potential as tools for dealing with muscle weakness and atrophy during aging," Adams says. "We also thought we might be able to use ursolic acid and tomatidine as tools to find a root cause of muscle weakness and atrophy during aging."

Adams' team investigated the molecular effects of ursolic acid and tomatidine in aged skeletal muscle. They found that both compounds interfere with the activities of the genes involved in aging muscle atrophy. They concluded that ursolic acid and tomatidine allow skeletal muscle to recover from effects of aging, says Adams, who is also a staff physician with the Iowa City Veterans Affairs Medical Center.

DISCLOSURE: The study was done in collaboration with Emmyon, Inc., a University of Iowa-based biotechnology company founded by Adams, which is now working to translate ursolic acid and tomatidine into foods, supplements, and pharmaceuticals that can help preserve or recover strength and muscle mass as people grow older.

Research funded by "ice bucket challenge" may benefit inclusion-body myositis research

National media outlets following up a year after the ice bucket challenge interviewed Professor Philip Wong at Johns Hopkins University, who is researching a protein, TDP-43, in connection with ALS, also called Lou Gehrig's Disease.

In defending the huge monetary outpouring in response to media attention to ALS — like IBM, a rare disease — Dr. Wong said research into the protein may show that it's a candidate for curing patients with other diseases such as Alzheimer's Disease and IBM, both of which are progressive diseases related to aging.

In more recent ALS research at St. Jude Children's Research Hospital, scientists have discovered evidence of a mechanism at the heart of amyotrophic lateral sclerosis (ALS) and related degenerative diseases. The research appeared in the September 25 edition of the journal *Cell* and highlights a possible new treatment strategy for IBM as well as ALS, researchers said.

The study focused on usually short-lived compartments called stress granules that form in cells under stress. Stress granules are just one type of the membrane-less structures or organelles that assemble as needed to handle various cell functions and then rapidly disperse. Until now, however, the mechanism underlying stress granule formation was poorly understood.

Stress granules are tied to degenerative disorders like ALS and IBM. Genes encoding the protein components of stress granules are often mutated in patients with ALS and other diseases. These same proteins accumulate in thread-like deposits called amyloid fibrils in the nerve and muscle cells of patients with ALS, frontotemporal dementia (FTD) and inclusion body myopathy (IBM). Scientists believe that this is a clue that a similar mechanism is involved in these disorders. ●

A difficult journey

By Nancy Harber

Nancy Harber, a registered nurse, was the long-time caregiver for Charlie Harber, an inclusion-body myositis patient. She serves as an informal consultant to patients and caregivers struggling with the challenges of myositis. Although her experience was specifically with IBM, many of her solutions will apply to other forms of myositis as well.

For this article, I don't want to talk about the mechanics of getting into, off of, up or down from something, or how to outsmart gravity and physiology. I want to talk a little about the difficult journey we are all on and how horribly frustrating it can get. And about the guilt that we all take on ourselves when, being human, we get impatient and tired.

The first TMA conference that Charlie and I attended was in 2001 in San Diego. Charlie was the facilitator for two KIT groups at the time and we had been asked to assist with the conference. The theme that year was "You Are Not Alone." Little did I know just how meaningful that phrase would be to us during the following years and how much it continues to be for all of us today.

While it is true that these diseases are "rare" in the general scheme of things, unfortunately there are too many of us battling these challenges. And while that is sad, it is also a blessing. It means that there are others on the same journey that you are on, and if you look, you can find someone just a little bit further down the road than you are. Learn from them!!! The old saying, "Don't re-invent the wheel" has never been more true. This is one of the really best things about support groups. You can almost always find someone who has fought the problem you are just now starting with and who can share with you what worked for them.

When we have a new person show up at one of our groups, I am always struck with the questions and concerns they share with us. They are the same ones we heard from the last new person who joined us. And I am SO grateful that they found our group, took a deep breath and braved an unknown group of people to come and ask, "What's going to happen to me and how can I live with this?"

None of us like to admit we are frightened by what is happening to us physically and it isn't easy to share those fears. But you can bet that many in the room have "been there, done that" at some time in the past. And you can also bet that nothing makes someone dealing with a myositis disease feel more useful than to be able to share his or her story with a newly diagnosed person.

We can't cure these diseases yet. We can only learn to live with them and find the best way to cope. To save someone else from struggling with problems if we have found a better way to deal with them: This is what I think life is all about.

As caregivers, you have a huge responsibility on your shoulders. But you also have a glorious opportunity to share your knowledge and your strength with someone coming on the road behind you, while also learning something new from those slightly ahead of you. You can make a real difference in people's lives — not just for the one you care for, but for all of those who touch your world.

And yes, it gets scary, tiring and often brings tears, but when someone says, as happened recently to me, "I tried what you suggested about tilting my head forward to swallow pills and for the first time, didn't choke," you know it has been worth it.

"You are not alone."

TMA invites all caregivers and former caregivers to help caregivers for the newly diagnosed in a number of ways. Visit our forums and our Facebook pages, or volunteer with your local support group. For ideas, email **tma@myositis.org**. ●

A global effort drives myositis research and patient support

Nobody who has attended The Myositis Association's Annual Patient Conference or followed myositis research could have failed to notice that TMA has become increasingly multinational. That's because myositis experts as well as patients come from all over the world. Since TMA changed its name from the Myositis Association of America (MAA) in 2002, it has acknowledged the contributions of its far-flung associates by awarding research grants to researchers from almost every continent; by supporting patient groups in other countries, and by inviting internationally-known researchers to serve on its medical advisory board. Most recently, TMA sent the first of what will be an annual edition of the "International Outlook" to all email addresses outside the United States. Following are some highlights of that issue. If you'd like a copy, email TMA at tma@myositis.org.

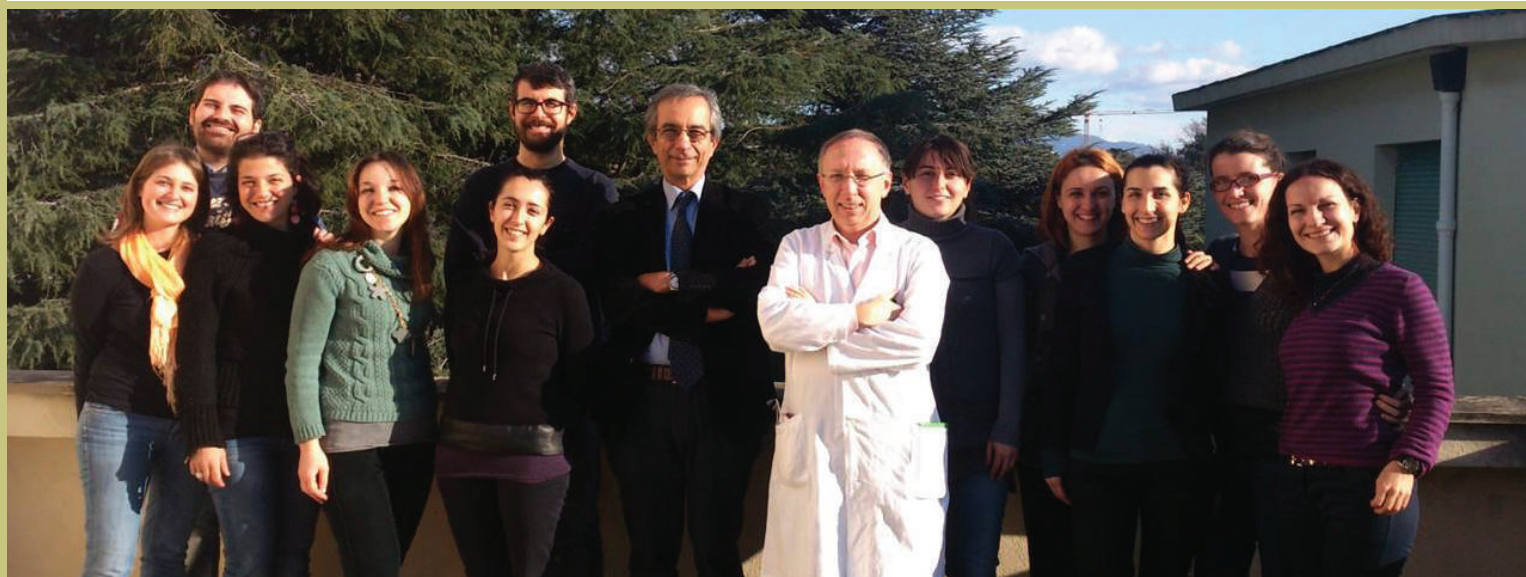
Worldwide cooperation makes research faster, better

"TMA had an early role in bringing scientists together," said Dr. Lisa Rider, a founding member of TMA's medical advisory board, recalling the scientific meetings TMA started in 2000. "It was very important to get researchers from a variety of disciplines in the same room and talking to each other," she said. "I think those meetings helped a lot in beginning to foster interdisciplinary studies in myositis, and researchers from a number of specialties began working together."

Today, it's common for collaborative groups to meet and share information. They've established registries that include patient surveys, case studies, research results and even biorepositories with physical samples.

Dr. Rider counted no fewer than 46 myositis research registries in the Americas and Europe, Asia and Australia. Developed for a variety of purposes, scientists use them for studies perhaps never even dreamed of by the original researcher.

MYOVISION, a joint registry project between TMA, NIH and Cincinnati Children's Hospital Medical Center is by far the largest collection of myositis patient data, with nearly 2,000 patients enrolled. MYOVISION collected environmental factors, patient profiles and symptoms, treatment responses, extent of injury, and quality of life. The registry will provide future investigators with a pool of myositis patients to contact for research. One important working group is IMACS, International Myositis Assessment & Clinical Studies Group. A group of more than 200 rheuma-tologists, neurologists, dermatologists, physiatrists, nurses, statisticians and other myositis experts. It has developed standards for myositis studies, and played a part in success-fully completed trials studying a wide range of myositis topics, with more planned trials currently in progress.



Faces of myositis research

Myositis researchers include scientists on literally every continent. *Here are a few, representing hundreds of others working for better treatments and a cure.*

Dr. Nicolino Ruperto

Multinational study compares juvenile myositis treatments

Dr. Ruperto of Genoa, Italy, was one of the founders (with Dr. Alberto Martini) of PRINTO almost 20 years ago. PRINTO has completed a guide for juvenile dermatomyositis best practices for treating children.

Dr. Guochon Wang

Studying China's growing myositis population

In his lab in China's massive capital city of Beijing, Dr. Wang, a rheumatologist, investigated drug-related myositis and heart disease. Many other projects related to myositis are underway at the China-Japan Friendship Hospital, one of the major myositis centers in China.

Dr. Helene Alexanderson

Promoting the benefits of exercise in myositis

A member of TMA's Medical Advisory Board, Dr. Alexanderson has shared her belief in the importance of exercise with hundreds of myositis patients from around the world.

Dr. Neil McHugh

Focusing on myositis autoantibodies

Dr. McHugh is a professor of Pharmacoepidemiology at the University of Bath. His long-standing interest in autoimmune disease has become more focused on myositis. "In particular," he said, "my research group has a major interest in the characterization of autoantibodies found in adult and juvenile myositis."

Dr. Ignacio García-De La Torre

Establishing resources for myositis patients in Mexico

Dr. Ignacio García-De La Torre became interested in myositis after studying Immunology and Rheumatology in California and Colorado. He started the Immunology and Rheumatology department at Hospital General de Occidente de la Secretaría de Salud in Guadalajara, Mexico in 1980, where he is the director. Resources for those with myositis were not available before that.

Dr. Kanneboyina Nagaraju

Myositis key to understanding autoimmune diseases

Dr. Nagaraju, an immunologist with a specialty in molecular mechanisms of target tissue injury in muscle disease says that, "After almost eight years of working with viruses, I realized that it was not exciting to study diseases for which the cause is known. So I decided that I would like to focus on autoimmune diseases, for which the cause is not known." Dr. Nagaraju is a veteran member of TMA's Medical Advisory Board and has been involved in many myositis studies.

TMA: Education, research, advocacy and support at the forefront

For those who couldn't attend the 2015 Annual Patient Conference, TMA has collected videos and PowerPoints from the Conference and made them available on the website at **www.myositis.org**. Check often, as we edit and post new videos on the site. Find Conference coverage at **www.myositis.org/your-myositis-community/annual-patient-conference**, including the live feed from Saturday, September 12:

- **Reports from TMA Medical Advisory Board Members**
- **Managing dermatomyositis**
- **Understanding myositis medications**
- **What your antibodies tell your doctor about your disease**

In addition to the videos and live feed, you'll also find PowerPoint presentations from the Conference on TMA's website.

The Conference included a Myositis Symposium where TMA's medical advisors — neurologists, rheumatologists, dermatologists and rehabilitation specialists — shared information about ongoing and completed research.

TMA's Research Committee met to make recommendations about projects deserving of funding for 2015. TMA's Board of Directors awarded $750,000 for research covering all forms of myositis.

As part of TMA's ongoing advocacy efforts to spread the word about myositis, San Francisco Giants and TMA joined forces to educate the public about myositis. Giants TV announcer Mike Krukow, who has IBM, was honored at a ceremony at home plate before the game against the Arizona Diamondbacks on September 20. Thousands of fans at AT&T Park and at home learned about myositis.

For those unable to attend the Annual Patient Conference in Orlando, TMA and some of its medical advisory board members hosted mini-conferences in the second half of September. These events, which reached nearly 150 people in small regional groups, were informal discussions of diagnosis and treatment of different forms of myositis. The programs were well-attended and TMA plans to hold more of the regional conferences in 2016.

On Myositis Awareness Day, September 21, and the weeks following, regional groups staged events designed to inform the public about the need for better treatments, more research and a cure for myositis. A few of the events:

- **The Bolgers and the Colorado KIT support group held the Annual Myositis Awareness "Picnic in the Park."**
- **The Southwest Florida KIT support group organized a Myositis Awareness Giants Game Watch Party, with myositis bingo and plenty of camaraderie, in Sarasota.**
- **Sal Negron organized a "Jeans Day" Benefit at Katten Muchin Rosenman LLP of Long Island, NY.**
- **Karen Samski led the Los Angeles KIT support group activities with a Myositis Awareness Day potluck.**
- **Katie Weyhrauch used her personal garage sale for myositis awareness as she prepared for a cross-country move from New York City to the West Coast.**
- **In Tucson, Vickie Jahaske and TMA Board Member Bill Prall organized a meeting designed to promote myositis awareness.**
- **In Massachusetts, Dagmar Slaven, with help from the local Delta Masonic Lodge, hosted the annual "Dagmar's Walk in the Park."**
- **Jenny Leonard and other mid-Atlantic support group members hosted the Annual Myositis 5k Run, Walk 'n Roll Event near Baltimore.**
- **The Carolinas KIT support group participated in a local craft fair to educate the community about the diseases.**

A forty-year medical mystery

Former dancer solves long-running family h-IBM puzzle

By Tara Voogel

Imagine you have a very active life as a mother and professional. Then suddenly, inexplicably, something changes. You become weak. Everyday tasks become harder to complete. Moving becomes difficult. Multiple trips to the doctor do not provide immediate answers or solutions. And to make matters even worse, your siblings display the same symptoms.

This is my story.

My family's medical journey began more than 40 years ago. Over the course of that time, my siblings and I suffered from the same symptoms. We set up appointments with our physicians and participated in several tests and studies in search of a diagnosis. The question no one could accurately answer: why is this happening?

The years passed. Our symptoms became progressively worse. My oldest sister received a diagnosis of limb-girdle muscular dystrophy, which turned out to be incorrect. In a heartbreaking turn of events, she passed away.

Then in 2010, finally, I received an answer to the question that had been plaguing us all of these years. I was finally diagnosed with GNE myopathy, which is also commonly known as hereditary inclusion body myopathy (HIBM), distal myopathy with rimmed vacuoles (DMRV), or Nonaka myopathy.

GNE myopathy is a rare, progressive muscle disease caused by mutations in the GNE gene that affects the production of sialic acid. GNE affects the lower and upper extremities, sparing the quadriceps. People with GNE myopathy typically show muscle weakness around 20 to 30 years of age and progressively lose muscle over the course of a lifetime.

Because GNE myopathy is such a rare disease, it is not typically known or understood among the general public or even within the medical community. It is often misdiagnosed as other conditions with similar symptoms, such as Limb-Girdle Muscular Dystrophy, Charcot-Marie-Tooth disease or Miyoshi myopathy.

What began as a diagnosis set a new course of direction in my life. At 58, I'm now an advocate for speedier GNE myopathy testing and diagnosis. My focus is to educate the public about this condition through my personal blog (Tara talks), social media, and through a global online community for other patients living with GNE myopathy. People need to know about this disease and understand that support is available.

Some of the most common symptoms of GNE myopathy include:

- **Foot drop when the muscles in charge of flexing the ankle and toes are weakened in such a way that the person needs to drag the front of the foot and bend the knee to lift the foot higher than usual.**
- **Waddling gait: due to the weakness of the pelvic muscles, the person uses the torso to help move the legs, resulting in an exaggerated waddling movement of the body.**
- **Loss of balance**
- **Difficulty walking up/down stairs**

If you believe your diagnosis with sporadic IBM or PM is in question, talk to your doctor and ask about GNE myopathy. You can learn more at www.gne-myopathy.org.

Additionally, free genetic testing is available to help patients understand how the disease affects them. For more information, please visit www.testingGNEM.com.

TMA also has resources for those with h-IBM. For information, email tma@myositis.org. ●

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Support for myositis patients spans the globe

Myositis Association Australia

"Come as strangers, leave as friends"

Website: **www.myositis.org.au**

From a small list given to Anita Chalmers by TMA in 2002, Myositis Association Australia has grown to hundreds of members scattered throughout the enormous expanse of Australia. Roughly the size of the U.S., Australia has a little less than 10 percent of the U.S. population, so isolation, always a problem with rare diseases, is even more pronounced.

The Myositis Association Australia has a comprehensive website (www.myositis.org.au) which supports as well as educates new members, steers people to appropriate medical specialists, advocates for them on patients rights issues, provides links for a variety of needs, and refers people to trials.

Myositis Canada

A new resource for our northern neighbors

Website temporarily linked to **www.myositis.org**: go to **Community/TMA Community Forum/Canadian Issues** (in French, Preoccupations Canadiennes).

Myositis Canada is in the midst of building a website that will enable Canadian myositis patients to find resources and meet others with myositis.

As the group builds its own website, it currently posts on the Canadian section of the TMA website, as well as on four Facebook Groups (Myositis Canada, Dermatomyositis and Polymyositis Patients, The Myositis Ramblers Keep in Touch, Myositis Support and Understanding).

Myositis UK

(formally Myositis Support Group)

Nearly 30 years of supporting myositis patients in the UK

Myositis UK was set up by Les and Irene Oakley when their daughter was diagnosed with juvenile dermatomyositis.

Myositis UK provides a number of services every day to myositis patients and families: Besides funding research, the non-profit answers enquiries from patients, families, welfare agencies, and medical professionals; provides people with guidance to ensure they get correct advice and information; publishes yearly newsletters and more frequent bulletins; reaches out to health professionals; and plans an annual conference. ●