THE CMTA Report

SUMMER 2018

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SPOTLIGHT ON RESEARCH

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OUR MISSION: *To support the development of drugs to treat CMT, to improve the quality of life for people with CMT and, ultimately, to find a cure.*
OUR VISION: *A World Without CMT.*

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Community fundraisers raise awareness, funds to drive the CMTA's mission forward.

See page 18

THE CMTA Report

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info@cmtausa.org

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The opinions expressed in the newsletter are not necessarily those of the Charcot-Marie-Tooth Association. The material is presented for educational purposes only and is not meant to diagnose or prescribe. While there is no substitute for professional medical care for CMT disorders, these briefs offer current medical opinion that the reader may use to aid and supplement a doctor's treatment.

A Message from CEO Amy Gray

DEAR FRIENDS,

Summer is often a time to slow down, relax and rest. But, here at the CMTA, we find ourselves working harder than ever to best serve the 2.8 million people around the world who are living with CMT. As you dive into this issue of *The CMTA Report*, you might notice something new. To best meet your needs, we have organized our content into three main sections—research, living with CMT and community engagement. We hope this will give you a clearer picture of where we're headed with research, how you can live your best life now and how you can get involved in our community.

On the research front, we're celebrating 10 years of discovery with our Strategy to Accelerate Research (STAR) program. The CMTA has engaged the top 25 scientific experts in the field and is now working with more than a dozen pharmaceutical and biotechnology companies, including Genzyme (a Sanofi Company), Ionis Pharmaceuticals, InFlectis BioScience and Acceleron Pharma, just to name a few. Through STAR, the CMTA currently has more than 25 active research projects and has invested more than $8 million. In this issue of *The CMTA Report*, you'll learn more about these partnerships and projects, as well as how you can participate in research.

While we look toward the future, we also know how important it is to offer resources that will help you manage your symptoms and live your best life now. In this issue, you'll find articles written by experts on breathing and hearing issues that can affect people living with CMT. We hope you will find them helpful and informative.

One of the most important functions of the CMTA is to provide a community in which people feel embraced, engaged and supported. In this issue, you'll discover how the CMTA remains committed to this community by offering more than 70 branches, 20 Centers of Excellence and other support programs. Every summer, we also host Camp Footprint—a camp created especially for children. We encourage you to take advantage of the opportunities near you—from Walks 4 CMT to educational sessions to group meet-ups, there is truly something for everyone. There will be a lot of events occurring in September during CMT Awareness Month, so get involved!

As we honor our commitments and priorities, we also want to take time to thank you for your financial contributions, as well as the gifts of your time, talent, enthusiasm and knowledge. We truly couldn't do everything we do without your support.

With thanks,

Amy Gray

AMY GRAY, Chief Executive Officer

STAR:

THE CMTA'S PLAN FOR FINDING TREATMENTS AND CURES FOR CMT

OUR VISION IS BOLD. We want to create a world without CMT. In fact, we have a strategy that is working, and we believe our vision is realistic. To better understand why we are confident, it is important to understand our Strategy to Accelerate Research, also known as our STAR program.

Launched in 2008, STAR brings together researchers with pharmaceutical and biotechnology companies to find treatments for CMT. We currently have more than 25 active research projects with top labs worldwide, and we have formed robust alliances with more than a dozen leading companies.

In this research section of the newsletter, you will learn more about one of our exciting projects and get to know two of our key alliance partners. Because we know that our research is powered by our community, we are sharing information about how CMT patients can get directly involved with research through our Patients as Partners in Research program.

While the challenge ahead of us looms large, we are confident that with our committed community, amazing partners, scientific know-how and unrelenting drive, we will someday live in a world without CMT.

CMTA RESEARCH SPOTLIGHT:

Alliance Research Partners

For the last decade, the CMTA has led the way with its Strategy to Accelerate Research (STAR) program. As mentioned at left, we now have partnership agreements with more than a dozen pharmaceutical and biotechnology companies across three continents including Genzyme (a Sanofi Company), Ionis Pharmaceuticals, InFlectis BioScience, Acceleron Pharma and many others. They are leaders in the latest genetic and neurological technologies such as CRISPR, gene therapy, gene silencing, and axon and muscle regeneration and are working with the CMTA to accelerate the development of treatments for CMT.

NEW CMTA ALLIANCE PARTNER INFLECTIS BIOSCIENCE ANNOUNCES PHASE 1 CLINICAL TRIAL

The CMTA recently announced a new strategic research partnership with InFlectis BioScience, a drug discovery company committed to the development of innovative therapeutics in CMT and other diseases.

The early focus of the collaboration has been on pre-clinical studies, clinical planning and understanding the impact of the disease on patients.

"This is the CMTA's Strategy to Accelerate Research in action," CMTA CEO Amy Gray said. "We develop world-class research tools and provide access to expertise that attracts partnerships with companies like InFlectis BioScience. The goal of these partnerships is ultimately to develop therapies that have the potential to treat people living with CMT. We are very excited that InFlectis BioScience has received approval to begin Phase 1 clinical trials. This is one of many examples of how the CMTA's research strategy is indeed moving us closer to a world without CMT."

THE CMTA FORMS ALLIANCE WITH ACCELERON PHARMA TO ADVANCE CMT TREATMENT OPTIONS

The CMTA has announced a strategic partnership with Acceleron Pharma, a Cambridge-based biopharmaceutical company dedicated to developing medicines to treat serious and rare diseases, including CMT.

The CMTA's Strategy to Accelerate Research (STAR) connects top CMT clinicians and academic researchers with pharmaceutical partners committed to developing treatments, and ultimately, a cure for CMT. Strategic alliances with corporate partners like Acceleron support innovative drug development with a goal to rapidly deliver therapies to CMT patients.

"We are excited to establish this partnership with Acceleron as they work to advance their lead neuromuscular therapeutic candidate, ACE-083, through an ongoing Phase 2 trial in patients with CMT," said CMTA's CEO Amy Gray. "Strategic research partnerships with great companies are proof that the work done by CMTA researchers is paramount to understanding the disease and developing innovative medicines. In the 10 years since establishing the program, STAR has generated important findings for advancing the science of CMT and attracting pharmaceutical companies to work on CMT. We share Acceleron's enthusiasm and passion for developing life-changing treatments for patients."

The partnership with Acceleron will include preclinical research and close collaboration with the broader CMT community through the CMTA's Patients as Partners in Research initiative aimed at raising awareness for the thousands of patients living with CMT. The partnership will seek to incorporate the voice of the patient in drug development and future clinical trials through patient initiatives such as focus groups and surveys. Learn more about the Patients As Partners in Research Focus Group that was recently hosted with Acceleron on page 6.

"It's a privilege to partner with the CMTA in this important initiative, as our missions are in complete alignment," said Ravi Kumar, PhD, Chief Scientific Officer of Acceleron. "Both organizations are deeply committed to scientific innovation, but developing transformative therapies cannot occur within the vacuum of a laboratory setting. Ongoing input from the patients we're working to help is essential for success in these pursuits, and this arrangement will help ensure that CMT patients are heard."

ACCELERON

CMTA RESEARCH SPOTLIGHT:

Patients as Partners in Research Program

The CMTA is pleased to announce the launch of our Patients as Partners in Research initiative! The goal of Patients as Partners in Research is to advance the work of our STAR program and to enroll the patient community in the critical work of furthering the development of treatments and ultimately a cure for CMT.

Some examples of ways you may be able to get involved in the CMTA's Patients as Partners in Research include:

- Completing surveys about your symptoms and experiences with CMT
- Participating in focus groups with the CMTA and our strategic partners in the biotechnology and pharmaceutical fields
- Enrolling in CMTA-funded research studies with our clinical and scientific partners
- Joining clinical trials for which you may be eligible

We have already created two opportunities for people with CMT to get directly involved with research:

Acceleron Pharma Focus Group

One of the first projects undertaken through our new research initiative was a Patients as Partners in Research Focus Group held in early June 2018. In partnership with the Cambridge, Massachusetts-based biopharmaceutical company Acceleron Pharma, the CMTA brought together patients to participate in a focus group. The group's aim was to develop a greater understanding of CMT and to gather information that will help advance the field and inform the development of new therapies. Topics discussed included specific muscle weaknesses, CMT subtypes, healthcare utilization and current/developing treatment options.

CMT2A Breathing Study

The second CMTA Patients as Partners in Research opportunity is a new study with the University of Iowa CMT Clinic and CMTA Center of Excellence to look at pulmonary function for people who have CMT2A. You are eligible for this project if you are 15 and older and have a diagnosis of CMT2A. If you decide to participate in this study, the University of Iowa will evaluate your symptoms, and you will have pulmonary function tests to assess any problems with your breathing. This may then lead to a nighttime breathing treatment similar to CPAP for sleep apnea which could be tried to see if it would help with your breathing. The University of Iowa will follow up over time, so after the first visit they will see participants back at three months, six months and 12 months to see if this works in people with CMT2A.

If you are interested in learning more about participating in this study, please contact Emilee Gibson with the University of Iowa CMT clinic at 319-356-3894 or emilee-gibson@uiowa.edu. ★

BECOME A PARTNER

WE ALL DREAM OF A WORLD WITHOUT CMT.
Now there are even more ways for you to get involved!

- ★ **Complete surveys about your symptoms and experiences with CMT**
- ★ **Participate in focus groups with the CMTA and our strategic partners in the biotechnology and pharmaceutical fields**
- ★ **Enroll in CMTA-funded research studies with our clinical and scientific partners**
- ★ **Join clinical trials for which you may be eligible**

Please visit www.cmtausa.org/patient-partners for more information on these and upcoming opportunities for you to become a partner.

be the breakthrough in their lives

Thanks to your support, Charcot-Marie-Tooth Association's STAR research program is where it is today – on the verge of delivering treatments for CMT, the progressive neuromuscular disease that affects more than 2.8 million people worldwide.

BUT OUR WORK IS FAR FROM DONE.

In the next three years, the CMTA will need $10 million in funding from people like you to maintain the quickened pace of its research. With these funds, the CMTA will be able to:

- Conduct clinical trials on drugs already identified to treat CMT, poising them for Food and Drug Administration approval.
- Continue the search for other treatments for 1A, 1B, 2A, 2E, 1X, 4C, and other types of CMT.
- Continue to pursue every promising avenue toward a cure until we reach our goal of ending CMT.

More than most people, you know what a drug treatment for CMT will mean. You undoubtedly have your own living example. Give today, because there are 2.8 million reasons to end CMT.

Your gift is welcomed and appreciated and is tax-deductible as allowed by law.

Please donate online at www.cmtausa.org/cmtbreakthroughs or complete the form below and mail to:

CHARCOT- MARIE-TOOTH ASSOCIATION
PO Box 105 • Glenolden, PA 19036

Yes, the CMTA can count on my contribution to be the breakthrough in their lives and accelerate research for a treatment for CMT!

☐ $75 ☐ $150 ☐ $300 ☐ Other: $____________

☐ I am interested in learning about leaving a legacy gift to the CMTA.

☐ Check enclosed, payable to the Charcot-Marie-Tooth Association, or

Please charge my: ☐ Visa ☐ MasterCard ☐ American Express

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BIOMARKER PROJECT SPOTLIGHT:
Developing Critical Tests to Accelerate Treatments

BY JOHN SVAREN, PHD,
U. OF WISCONSIN-MADISON

Because clinical trials involve a large investment of both time and funding, many conversations with CMT pharmaceutical partners about potential therapies focus on how to design clinical trials that will quickly address a new medication's efficacy. Consequently, one of the most urgent needs in the CMT field is to find better ways to assess the dysfunction of the peripheral nerves in patients with CMT.

Building upon the neuropathy score developed by the CMTA's Centers of Excellence in conjunction with the Inherited Neuropathy Consortium, a collaborative project that is funded in part by the CMTA and directed by CMTA board member Dr. Michael Shy, several efforts have been launched to develop additional measures that will provide an assessment of neuropathy. Because CMT is a slowly progressive disease, developing these measures is a challenge.

Some recent progress toward this goal has come from magnetic resonance imaging (MRI) studies of calf muscle. Initiated in the United Kingdom under the direction of Dr. Mary Reilly, a member of the CMTA's STAR Advisory Board, and independently validated in collaboration with Dr. Shy at the University of Iowa, these studies show that the measurement of muscle mass can be a sensitive measure of neuropathy progression. Moreover, recent studies from Dr. Reilly and Dr. Alexander Rossor in London indicate that blood samples can be used to measure a protein called Neurofilament L that is released from CMT nerves.

Since the focus of several CMT1A therapies is reducing the expression of the *PMP22* gene that causes neuropathy, the collaboration of Dr. Shy with Dr. John Svaren at the University of Wisconsin has turned to the analysis of both blood samples and skin biopsies. There are nerves present in the skin, so the affected Schwann cells—the cells in the peripheral nervous system that produce the myelin sheath around neuronal axons—can be assessed by sensitive gene detection methods to determine the level of PMP22. This type of analysis was used in the recently published study of antisense oligonucleotides in rodent models of CMT1A.This study was published in December 2017 by scientists at Ionis Pharmaceuticals, in collaboration with the CMTA.

In a recently awarded project by the CMTA, Drs. Shy and Svaren will collaborate to measure PMP22 and other genes in skin biopsies from CMT1A patients. They also will see if similar gene expression changes can be measured in the other major forms of CMT: CMT2A, CMT1B and CMT1X. In addition, blood samples will be used to identify changes in not only nerve proteins, but also other proteins and RNA substances that are released from Schwann cells that are affected in CMT1 neuropathies.

The goal of these studies is to establish more sensitive ways to determine the earliest indications of whether therapeutic agents are improving neuropathy in people with CMT. We hope to find blood tests that may be specific to the aforementioned subtypes of CMT, as well as some that could apply generally to multiple types of CMT. Given the progress the CMTA and its strategic partners are making toward clinical trials, the development and validation of these tests is critically important and urgent, and it will provide a more comprehensive battery of tests that can be used to assess whether new therapies have the intended effect in clinical trials. ★

CMTA REMEMBRANCES

Your gift to the CMTA can honor a living person or the memory of a friend or loved one. Acknowledgment cards will be mailed by the CMTA on your behalf. Donations are listed in the newsletter and are a wonderful way to keep someone's memory alive or to commemorate happy occasions like birthdays and anniversaries. They also make thoughtful thank-you gifts. You can participate in the memorial and honorary gift program of the CMTA by completing the form below and faxing it with your credit card number and signature or mailing it with your check to: CMTA, PO Box 105, Glenolden, PA 19036.

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In honor of (person you wish to honor)
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Send acknowledgment to:
Name: ______________________________
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Occasion (if desired):
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Charcot-Marie-Tooth Disease & Pulmonary Care

BY ASHRAF ELSAYEGH, MD, FCCP, PULMONARY/CRITICAL CARE, CEDARS-SINAI MEDICAL CENTER

Charcot-Marie-Tooth Disease (CMT) is a spectrum of disorders (CMT1A, CMT1B, CMT2, CMTX, CMT3, CMT4, and CMT5-7) that affect peripheral nerves that carry motor and sensory information to and from the brain. These include the phrenic nerves, as well as other peripheral nerves that control the function of breathing. This produces symptoms of peripheral nerve dysfunction, which can result in difficulty breathing. Although respiratory dysfunction is not a very common hallmark of CMT patients, it can occur.

DIAPHRAGM DYSFUNCTION:

Although breathing is controlled by multiple muscles, it appears that the diaphragm plays the most significant role. The diaphragm is a muscle at the bottom of your chest cavity controlled on each side by the right and left phrenic nerves. If the phrenic nerve becomes affected or damaged, the diaphragm will not be able to contract properly, resulting in shortness of breath, also called dyspnea. An early sign of diaphragm dysfunction is dyspnea while lying flat. Patients will have obvious shortness of breath or difficulty catching their breath when in a supine position—lying facing upward—usually relieved by sitting upright or sleeping at an angled position.

Diaphragm dysfunction may be diagnosed using multiple testing. The simplest test is pulmonary function, which tests the strength and efficiency of breathing. A full pulmonary function test (PFT) is not typically recommended in CMT patients, but could be performed if there are any symptoms of breathing problems. If abnormalities are detected on initial testing, then this can be followed by just the spirometry portion at least twice a year. Many clinics have this available in the office. Given that respiratory decline is rare and slow to occur in CMT, spirometry is usually not indicated. The major components of the PFT that are helpful in patients with neuromuscular weakness are the Forced Vital Capacity (FVC), which tests the total volume of air one can breathe out, and the Negative Inspiratory Force (NIF), which measures the force with which one can take a deep breath in. Data is not available to prove superiority of FVC versus NIF in monitoring respiratory decline; therefore, it is important to monitor both for a more accurate gauge of respiratory dysfunction.

Another relatively easy test to evaluate diaphragm dysfunction is a SNIFF test. This test requires the patient to sniff while undergoing fluoroscopic imaging. This will measure the movement of the diaphragm. In general, this does not need to be performed unless surgical intervention on the diaphragm is required, which is discussed below.

Finally, phrenic nerve conduction and/or diaphragm EMG may also be performed to evaluate diaphragm function. The phrenic nerve study is similar to the nerve conduction study done for the diagnosis of CMT except the phrenic nerve is stimulated in the neck and the recording is done with sticky pads on the chest wall and lateral rib. It is safe, and no more uncomfortable than routine nerve tests. The diaphragm EMG requires a needle in the area of the diaphragm and carries some small risks. More recently, ultrasound to evaluate the diaphragm has been utilized in some centers. Again, these are usually not necessary to

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[1] NIV is a portable noninvasive ventilator. Trilogy and Astral are two common brand names. CPAP and BiPAP are extremely uncomfortable for neuromuscular patients with respiratory problems. When working with an NIV, the physician can program many more variables (rather than just a simple pressure), so it becomes much better tolerated by the patient. When treating sleep apnea, neuromuscular patients have a higher percentage of central sleep apnea (rather than obstructive). A CPAP would worsen central apnea, so NIV is again the device of choice.

perform unless surgical intervention is needed.

When respiratory decline is discovered, early management is necessary. The mainstay of therapy is noninvasive ventilation (NIV). Continuous Positive Airway Pressure (CPAP) therapy is not recommended in neuromuscular disease. Although Bilevel Positive Airway Pressure (BiPAP) therapy is better than CPAP, there are now more sophisticated forms of NIV that are much more comfortable for and much better tolerated by patients. Also, as the respiratory function declines further, the newer forms of NIV are far superior in managing the respiratory complications. They are superior in maintaining the respiratory system, as well as maintaining a proper carbon dioxide (CO_2) level. If the patient has reached the point of retaining CO_2, he or she should already have been placed on noninvasive ventilation. Therefore, monitoring blood gases to follow the CO_2 level is usually not indicated. It is a painful invasive test that will not add much information or contribute to changes in management. The exception is if the patient has signs of hypercapnia—high CO_2—such as altered mental status, headaches or difficulty to arouse. At that point, measuring CO_2 levels with a blood gas will help in adjusting the NIV settings. The recommendation is to wear the NIV at night while sleeping to start. If needed, NIV may be used during the day if dyspnea occurs.[1]

In CMT, as with other neuromuscular disorders, the weakness in breathing usually does not affect oxygen levels, but as mentioned above, may increase CO_2 levels because of the inability to blow CO_2 out of the body. Therefore, supplemental oxygen is rarely needed in CMT and may actually be harmful in some patients.

If the respiratory decline has reached a point that the patient is requiring NIV 24 hours a day or NIV is no longer benefiting the patient, a tracheostomy may be considered at that point. The patient would then be attached to a portable ventilator through the tracheostomy. This situation is exceedingly rare in CMT.

Diaphragm pacing always comes up when discussing respiratory dysfunction in neuromuscular patients. Diaphragm pacing has equivocal results at best in neuromuscular patients and has never been tested specifically in CMT

(continued on page 14)

WHAT'S ON YOUR MIND? *Ask David.*

Write to David at info@cmtausa.org.

David Tannenbaum has an LCSW degree and has been a psychotherapist in New York City for the past 30 years, specializing in helping others with the task of growing emotionally and spiritually through physical challenges.

Dear David,

Growing up, I watched my dad miss out on a lot of family fun because he was too self-conscious to use a cane as he got older. He was grumpy a lot and hardly ever spoke of his CMT. As a young boy, I was determined not to follow in his footsteps. Although I'm happy to be walking well with my new braces, I am a little self-conscious and worry that wearing braces separates me from others. At 37, I would like to meet someone to share my life with, but I fear that my disability will make that impossible.

David replies:

My dad was stubborn as well, and he also deprived himself of many pleasurable experiences. Although he had mobility issues that were non-CMT-related, he saw using a cane or wheelchair as a sign of weakness. I failed to change his mind. So, he passed at 87 and spent the last years of his life feeling like his body had failed him.

At some point in our lives, hopefully sooner than later, we all need to accept that we are indeed a little different from others. That difference does not have to deprive us of most of the joys in life. In fact, for many of us, the challenges of CMT can help us develop qualities like sensitivity, compassion and kindness that will serve us well in life. While there are people who will judge you only by physical appearance, there are others who will appreciate the more human qualities you have cultivated over the years. A very famous philosopher once said that if you have a "why," you can cope with just about any "how."

See your braces or any other equipment that you need as simply the tools to reach your goal. Don't focus on feelings around needing some mechanical assistance. Be practical and focus on what you want to achieve. When I was first diagnosed at 19 and wanted to travel to Europe for the summer, a doctor told me that some of the best trips he took were in a wheelchair. He had polio, and it obviously didn't stop him from leading a full, purposeful life. Of course, I was mortified. This was not what I wanted to hear. Yet many years later, I did need the occasional assistance of a wheelchair and experienced a great trip to Europe. I was sorry only that I didn't do it sooner. I was proud of myself for letting go of feeling self-conscious and was determined not to allow CMT to stand in my way. Instead of being self-conscious, be conscious of your best self, which has less to do with focusing on your limitations and more to do with loving yourself for exactly who you are. ★

CMTA CENTERS OF EXCELLENCE

www.cmtausa.org/coe

CMTA CENTERS OF EXCELLENCE are patient-centric, multidisciplinary CMT clinics where children, adults and families affected by CMT can be assured of receiving comprehensive care by a team of CMT experts. The Centers roughly correspond to the 21 international sites that make up the NIH Inherited Neuropathies Consortium (INC)—a group of academic medical centers, patient support organizations and clinical research resources sponsored in part by the CMTA. The centers will become even more important as the CMTA begins clinical trials, which will depend on how much we know about the "natural history" of CMT—how different types of CMT progress over time and whether novel medications are slowing the course of the disease. Much of that information will be supplied by the Centers of Excellence.

CMTA CENTER OF EXCELLENCE	CLINICAL DIRECTOR
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**These Centers of Excellence are not part of the INC.*

INTERNATIONAL

The Children's Hospital (Westmead, Australia)	Dr. Manoj Menezes
The National Hospital for Neurology & Neurosurgery (London, England)	Dr. Mary Reilly
C. Besta Neurological Institute (Milan, Italy)	Dr. Davide Pareyson
University of Antwerp (Edegem, Belgium)	Dr. Jonathan Baets

NJ/NYC PATIENT/FAMILY CONFERENCE A SUCCESS

Energy was high and smiles abounded at the New Jersey/New York City Patient/Family Conference in June. Our 135 participants arrived early and stayed late, eager to learn from the outstanding speakers and build friendships with fellow families in the CMTA community.

Highlights from the action-packed instructional weekend include Dr. David Herrmann's presentation on measuring the efficacy of treatments for CMT in clinical trials, Dr. Louis Weimer's presentation on "Diagnosis, Genetic Testing, and Neurotoxic Medications", Kate Lair's helpful orientation to applying for disability insurance, and exercises for mental wellness from psychologist Dr. Elizabeth Misener, as well as information on orthoses and occupational therapy.

You can see footage from some of the speakers on our YouTube channel! **www.youtube.com/user/CMTAssociation**

Our next Patient/Family Conference and Youth Outing will be here before you know it—
SAVE THE DATE TO JOIN US IN SEATTLE ON OCTOBER 20TH!

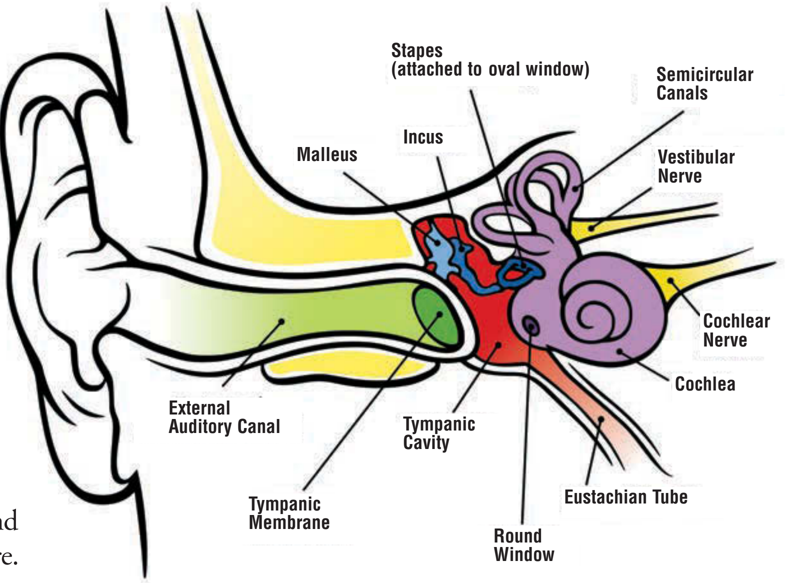
Hearing Loss & CMT

BY MARIO A. SAPORTA, MD, AND TIFFANY GRIDER, MS, CGC

The association between hearing loss and CMT has long been recognized, with the first published work on the subject dating back to the 1970s. This association is not surprising, because our sense of hearing relies on nerves—called cochlear nerves—to connect our inner ear sound detection system to the part of our brains that interprets sound. Just as the nerves in our legs and arms can be affected by CMT, so can the cochlear nerves. Interestingly, the rate of incidence of CMT-related hearing loss varies by CMT subtype.

The Inherited Neuropathies Consortium (INC) Natural History Study, the most comprehensive effort to date to characterize the way the different subtypes of CMT progress over time, studied the number of patients with various types of CMT who complained of hearing loss. Approximately 15 percent of patients with CMT1A, the most common type of CMT, complained of hearing loss, underscoring the need to better characterize and understand this relatively prevalent complaint.

An even larger proportion of patients with CMT1B and recessive forms of CMT (CMT4) noted the same issue (28.2 percent and 30 percent, respectively). Other CMT subtypes associated with hearing loss include hereditary neuropathy with liability to pressure palsies (HNPP), CMT1X and CMT2A. CMT-related hearing loss usually affects both ears equally, interferes with mid and high frequency sounds and ranges from mild to severe.



A common complaint among patients with CMT-related hearing loss is difficulty understanding others when in an environment with loud background noise—a party or concert, for example. Hearing loss can significantly affect the quality of life of people with CMT, including impairing the communication and learning development of children with CMT, especially if left undiagnosed for a long period of time.

Hearing screening tests and active discussions between patients and physicians are important and helpful strategies to identify and cope with CMT-related hearing loss. The use of hearing aids also can be very useful. New technology using dedicated mobile apps allows for customized adaptations to the hearing aid system in response to specific environments and social circumstances. ★

Our sense of hearing relies on the cochlear nerves that connect the inner ear to the part of the brain that interprets sound. These nerves can be affected by CMT.

PULMONARY DISEASE

(continued from page 11)

patients. Patients who are interested in diaphragm pacing should discuss the procedure with a center that performs it. At that point, if diaphragm pacing is a consideration, SNIFF testing and diaphragm/phrenic EMG are required.

Diaphragm dysfunction also plays a role in coughing. Again, although coughing is controlled by multiple aspects, having a weakened diaphragm can diminish the ability to cough. This may lead to secretion build up in the chest cavity. The increased secretions can also contribute to dyspnea.

As of now, there is no good test to measure cough function aside from clinically watching the patient cough. If coughing is affected, there are two main forms of airway clearance devices that may be tried. First, there is a high frequency chest wall oscillator. This helps break down secretions, making them easier for the patient to expectorate. Second, there is a cough assist that can actually help expectorate the secretions for the patient.

The key to proper pulmonary care is a durable medical equipment (DME) company and a respiratory therapist who specializes in neuromuscular patients. They will help in day-to-day management and adjustment of the noninvasive ventilator and will be an excellent resource for the patient on simple questions involving the equipment.

VOCAL CORD PARALYSIS:

Although relatively rare, some patients with CMT can have vocal cord paralysis. In general, it is usually more problematic in children than in adult patients. This may present with unilateral or bilateral vocal cord involvement. Unilateral vocal cord paralysis rarely presents with symptoms. Chronic aspiration of gastric content may occur with vocal cord paralysis resulting in repeated pneumonias. Bilateral vocal cord paralysis can result in stridor and difficulty breathing.

If patients develop chronic multiple pneumonias secondary to aspiration, vocal cord paralysis should be considered. This may be evaluated easily with a laryngoscopy. If paralysis is discovered, no significant intervention is usually warranted. Aspiration precautions should be taken. If vocal cord paralysis becomes problematic with hoarseness and difficulty breathing, a tracheostomy is indicated. These situations are rare, but again, tend to be more common in children than in adults.

SLEEP DISORDERS:

As with any neuromuscular disease, sleep apnea may occur in CMT patients. Both obstructive (OSA) and central (CSA) sleep apnea may present in these patients. Also, restless legs syndrome (RLS) and periodic limb movements (PLMs) may occur.

Sleep apnea is cessation of breathing—or reduction of respiratory effort—during sleep. Obstructive sleep apnea is secondary to obstruction of the upper airway, whereas central sleep apnea is caused by receptors in the brain. Symptoms of sleep apnea include poor sleep, frequent nighttime awakenings, daytime fatigue, and morning headaches. Snoring may or may not occur. Witnessed apneas may also be a seen by the patient's sleep partner.

Restless legs syndrome is pain or discomfort in the legs (that may be unilateral or bilateral) occurring during periods of inactivity usually at night. The pain is relieved by movement of the leg. Periodic limb movements consist of the jerking of limbs during sleep, resulting in sleep disturbance.

OSA, CSA and PLMs may all be diagnosed with an overnight sleep study. A screening sleep study is not indicated; however, if patients have any of the above symptoms, an overnight sleep study (Polysomnogram/PSG) would be indicated. Treatment is warranted if the PSG is positive for sleep apnea. Again, the newer forms of NIV are recommended rather than CPAP for neuromuscular patients. Also, CPAP is generally not appropriate for central sleep apnea. Therefore, NIV for all patients with neuromuscular disease and sleep apnea is recommended.

The only caveat to the above recommendation is for patients who may have already received an NIV for respiratory dysfunction. If the patient has NIV already ordered, they generally do not also need a sleep study since the NIV will treat the sleep apnea automatically. In addition, most PLMs improve with treatment of the sleep apnea, and NIV is usually all that is needed.

Restless legs syndrome is a diagnosis that is made clinically, and no testing is needed. Multiple pharmacological treatments are available when the diagnosis is made.

Pulmonary complaints are very rare in CMT patients. When problems occur, they are usually slow in onset and slow in progression; however, if they do occur it is a good idea to have a plan. See a pulmonologist who specializes in neuromuscular patients, as well as get acquainted with a center that specializes in CMT patients. In addition, if equipment is ever needed, use a DME/respiratory therapist that specializes in neuromuscular patients. ★

NOW IS THE TIME TO PUT THE CMTA FUNDRAISING FRENZY IN HIGH GEAR—PLAN YOUR WALK 4 CMT TODAY!

CMTA
Charcot-Marie-Tooth Association
WALK 4 CMT

Each year, thousands of people participate in Walks 4 CMT across the country to create awareness, renew hope and generate a community of support, all while raising funds for the CMTA and CMT research.

Many of the Walks 4 CMT are organized by 35 of the more than 70 CMTA branches, so if your branch is planning a walk, please check with your branch leader to see what you can do to help recruit participants and sponsors. If your branch hasn't done a Walk 4 CMT yet, now is the time to get started!

No CMTA branch near you? Not a problem! We're making it easy for you to bring a Walk 4 CMT to your neighborhood. Just contact CMTA National Events Manager Andi Cosby at andi@cmtausa.org or 1-800-606-2682, ext. 111, and she will help you get started and set up a webpage where your friends and neighbors can register for your walk and/or make a donation to the CMTA.

Whether your event is big or small, you will have 100 percent support from the CMTA and tons of fun in the process!

Learn more about Walk 4 CMT at www.walk4cmt.org.

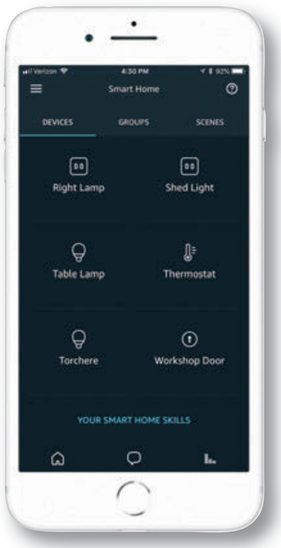
Alexa, Fetch My Slippers

BY DANA SCHWERTFEGER

Well, okay, so Alexa isn't able to do that yet—or make the perfect martini, but Alexa does have an amazing repertoire of skills that is being expanded every day. Together with Vera, my Smart Home controller, and my TuyaSmart app, I can tell Alexa to turn lights on and off, lock and unlock doors, turn on my Bose speaker and adjust my thermostat.

I actually met Vera before Alexa. Shortly after moving into my new home in Arizona a couple of years ago, I became increasingly annoyed that I had to use a key to shoot the deadbolt every time I left my house. I've had some hand involvement from my CMT for about 15 years, and while I can still use a key to lock and unlock my doors, as long as I was doing some renovating, it seemed like a good time to take a closer look at smart locks.

I can say, "Alexa, turn on Table Lamp," or I can use the Alexa or Vera apps on my phone to turn lights on and off, lock and unlock doors, control my thermostat--even turn my Bose SoundTouch speakers on and off.



I already had Schlage locks that could be rekeyed, so I bought three new smart locks at $180 apiece that work on Z-Wave technology, which is similar to Wi-Fi or Bluetooth in that you have to connect or pair your Z-Wave devices with a controller. I looked at Wink, Hue and several other controllers, but I settled on VeraPlus because there was no monthly fee, and it was just a one-time purchase of about $150. Vera also had an app for my iPhone, so once I connected my locks, I could see their status and lock or unlock them right from my phone.

I could also set a PIN for each lock or the same PIN for all of them, so I could unlock my doors by entering a code. Locking them on the way out was super easy, too. All I had to do was press the padlock icon on the keypad to engage the deadbolt. I still carry a key, but I hardly ever use it.

Customers who bought this item also bought....

Of course, the website where I bought the smart locks also very helpfully showed me other Z-wave products that would work with Vera. I wound up buying a Z-Wave thermostat, a garage door opener and countless light switches. You can buy switches with a rocker that replace conventional switches, as well as wall outlets. Leviton also makes plug-in switches. Just plug a device into a wall outlet and plug a lamp or other appliance into the device. Connect it to your controller, and you can turn lights on and off without having to fumble with those pesky knobs.

VeraPlus ($150, no monthly fee) is just one of many controllers that can be used to control Z-wave, Zigbee and Bluetooth devices. Buying open-box or refurbished items is a good way to lower the cost of creating a Smart Home.

Here an Echo, there an Echo, everywhere an Echo....

Several months after setting up my devices with Vera, I bought my first Echo from Amazon. As I was going through the list of all the things Alexa could do, I discovered that Alexa and Vera did not get along, and I could not use voice commands to control my devices. The Vera developers quickly caught up and now Vera and Alexa work well together. At last count, I now have six Echos and one Echo dot, so I can issue commands to Alexa from just about every room in my house as well as the patio and garage. I have two second-generation Echos, but I bought all the rest refurbished for about $70 each. Buying refurbished and open-box items is one way to keep the cost of a smart home within your budget.

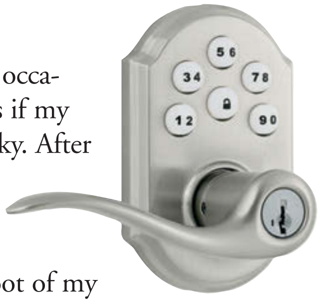
Moscow on the Hudson....

Remember that classic scene where Robin Williams, playing a Russian defector, goes into a supermarket to buy coffee and is completely overwhelmed by the number of brands to choose from? Buying smart home products can be a bit like that. The technology is advancing so rapidly, and so many manufacturers have entered the market, that the number of products and the variety of options has become mind-boggling. From Alexa and Google Assistant to Siri, Cortana and Bixby, virtual assistants are taking command of everything from our coffeemakers to our washing machines to our bathroom mirrors.

So, to be smart about smart home devices, start with a plan. Make a list of everything you want to be able to control. You don't have to do everything at once—start with door locks and maybe some lights—but be sure that your system can be expanded to include additional devices. And be sure that the devices for what you want to control are available and fully compatible. An example: When I bought a thermostat that was Z-Wave compatible, the product description said, "works with Vera, etc." It did, but I soon discovered that not all of its features were fully supported by Vera. I can turn my heat or AC on from my Vera app and set the temperature, but I can't program wake and sleep settings. I should have consulted Vera before buying the thermostat to see which models were in Vera's skill set.

Dumb and dumber....

A lack of compatibility isn't the only glitch I've encountered. During what was supposed to be a routine firmware update, my $150 Vera controller became a brick, a $150 Vera doorstop, and much of my smart home suddenly became very dumb.



The same thing occasionally happens if my Wi-Fi goes wonky. After half a dozen support calls, they replaced my Vera. A reboot of my router usually fixes the wonky Wi-Fi, but it is a bit irritating when I say, "Alexa, turn on desk lamp," only to have Alexa respond, "I'm sorry. The hub desk lamp is connected to isn't responding."

Enter my TuyaSmart app. Tuya works via Wi-Fi and an app on my iPhone, and the plug adapters for Wi-Fi are less expensive than Z-Wave adapters. I have a few lights connected via Tuya, which also has an Alexa skill, so when Vera gets grumpy and takes an unscheduled vacation, I can still turn some lights on. Of course, if my internet goes down, all bets are off. Period. So, it's still a good idea to have a light or two you can still turn on the old-fashioned way, and don't throw away your house key just yet.

Back to the Future....

The Clapper—remember the Clapper, the sound-activated control device that debuted in 1985, around the time Marty McFly was traveling back to the future? It's still around, but it's like something from the Stone Age. And yet, thinking about it in comparison to what gadgets can do today, it helped me remember that just because I can buy a washing machine I can control with Alexa doesn't mean I need to.

There was a time when light switches and door locks posed no problem for me. But, as my CMT has progressed, I am grateful for the smart home devices I have installed. They do make living with CMT easier. But some things, like doing my laundry, I can still manage very well on my own without Alexa, thank you.

Smart Plugs, about $10, are an easy way to control lights or fans and work through Wi-Fi and apps like TuyaSmart, which also works with Alexa.

Smart locks ($175), Z-wave switches and plugs ($30-40), work with Vera and other controllers, many of which also work with Alexa and Google Home.

Oh, and about those slippers....

I swear I did not know about this when I chose the title of this article, but Nissan has developed "self-driving slippers." No kidding … when you press a button, little wheels come out and the slippers go and park themselves at the door. The catch? For now, the slippers are available only at the ProPILOT Park Ryokan, a pop-up hotel in Hakone, Japan. ★

GRASSROOTS/COMMUNITY FUNDRAISING

Have you ever wondered what you can do to help cure CMT? The families and community members you're about to read about have figured it out! The CMTA relies on personal donations and community fundraising to fund our mission. These amazing stories of community fundraising events will inspire you and remind you that you can make a difference. Large or small, everything our community does to generate dollars powers the CMTA's ability to invest in treatments and therapies.

Remember, when you support the CMTA, you are helping to create a world without CMT.

ANNUAL WESTCHESTER, NEW YORK BRANCH LUNCHEON

Seventy people attended the Westchester, New York CMTA Branch annual fundraiser for STAR. This year's luncheon was held at Banchetto Feast in Nanuet, New York.

Donated lantern centerpieces adorned each table, and seasonal candy added to the festive feeling. After a delicious luncheon a selection of home baked cookie platters was placed on each table. A really nice surprise was a sheet cake which read "We'll Succeed" decorated with the CMTA logo.


The Perrone fanily fundraiser drew a crowd of more than 180 people.

With an auction of gift cards and gift baskets and a 40-20-20-20 raffle, it was a really great event, raising over $15,000, making it the branch's most successful fundraiser ever.

PERRONE FAMILY FUNDRAISER

The Perrone family wanted to have a fundraiser where they could kick up their heels and have a great time, so with the help of great family and friends, they organized a dinner dance. They spread the word on social media, and before they knew it, they had more than 180 people attending! The theme was "What can we do?" and the answers were: Continue to support people who have CMT and other rare diseases, continue to spread the word and create awareness and continue to fundraise to support research and therapies. But, most importantly, continue to celebrate differences. They raffled fun-filled baskets and had a 50/50, which brought in more than $700. The winner graciously donated the entire amount back. By the end of the event, they had raised approximately $12,000!


The excitement at Bingo Night was on full display!

THE STRASBURG BINGO NIGHT

The first annual CMTA Bingo Night fundraiser was on May 20 at the Strasburg Firehouse. It was a big success! Event coordinator Amy Keller was overwhelmed with joy by the number of people who came out to support her fight for a cure for CMT. The event raised more than $8,000 and filled the room above capacity. Thanks to family, friends, customers and GetSmok'd BBQ for all the support that made the bingo night a success!

FISHING FOR A FIX

The 3rd Annual Fishing for a Fix was held in Harrisburg, Pennsylvania on Sunday, April 29. It was another successful event, raising more than $5,000! This year had the highest turnout of participat-

FOR A CURE

ing anglers with 48 children competing for the first fish of the day, first palomino trout of the day and top three trout by length. Prizes included donated trophies, as well as fishing rods, nets, tackle and more!

As in previous years, all prize items, raffle baskets, the kayak for raffle and materials used were donated, allowing all money raised to be donated to the CMTA! Food and drinks were also donated this year from local grocers, and there was even a food truck! Get Smok'd BBQ grilled up hamburgers and hot dogs as well as their traditional BBQ menu and donated all proceeds to the event. Many branch members also contributed this year by donating, volunteering or attending the event!

With each year, the event has grown and provided lessons on what to add or how to improve. Last year, the youngest branch member raised more than $2,000 on his own at the age of 12. Branch leaders were so impressed they decided to create an award in his honor! The first ever "Jordan Mermelstein Fundraising Award" was given to the highest individual fundraiser, Devon Schaeffer! The branch hopes to see this continue and that it helps to teach the young participants and community about the focus on the mission of supporting the CMTA.

The Harrisburg, Pennsylvania branch of the CMTA really pulled together this year and collectively created another special day! Branch members can't wait to do it again next year!

HOPS FOR HOPE

There was a great turnout for the first ever Hops for Hope! The love and support from CMT members, families and the community was felt and seen by all! Congratulations to the silent auction winners and a huge thank you to Swamp Cabbage Brewing Company, Ludicrous Foods truck, and the Belgian Waffle food truck for providing awesome food and beverages! The event raised more than $1,000! ★

Hops for Hope Silent Auction

THE OXFORD FUNATHLON RAISES $100,000

The Oxford Biathlon—now the Oxford Funathlon—was bigger and better than ever this year. Organized by CMTA Board member Steve O'Donnell with assistance from CMTA Advisory Board member Clark Semmes and CMTA staff member Andi Cosby, the fifth iteration of this popular fundraising event featured 28 swimmers, 61 bikers and 32 walkers. Participants included leading CMT researchers Drs. Michael Shy, Steven Scherer and Mark Scheideler.

Our thanks to the 282 people who donated to the event and to the 117 people who enjoyed a wonderful buffet lunch at Doc's Sunset Grill. Once again, the event raised more than $100,000 for the CMTA!

The event kicked off, as always, with an optional one-mile swim across the Tred Avon river in beautiful Oxford, Maryland. The United States Coast Guard shut down all river traffic on the historic Tred Avon as volunteer kayakers and paddle boarders escorted the swimmers across the one-mile passage. For the first time this year there was even a drone, deftly operated by Chris Sweeney, soaring overhead and taking snapshots of swimmers.

Once the swim was concluded, bikers took the Tred Avon ferry across the river to Bellevue and rode 20 miles to Easton and then back to Oxford. While the bikers were out, Andi Cosby led the walkers around beautiful and historic downtown Oxford with a stop at the Highland Creamery, the best homemade ice cream stand on the entire planet.

After the athletic portions of the event, everyone met at Doc's Sunset Grill for a fabulous buffet featuring Maryland crab cakes, burgers and Caesar salads.

At an after-event party at the historic Robert Morris Inn in downtown Oxford, Chris O'Donnell seized the microphone from a local troubadour and led diners in rollicking renditions of Van Morrison's "Brown Eyed Girl" and Neil Diamond's "Sweet Caroline." It was later remarked by some that it was a wilder time than the historic inn had seen in many a year.

For those who wish they had attended the event, please consider joining next year. The Funathlon will continue until a cure is found for CMT!

Beating CMT—*and Enjoying Every Minute of It*

BY BOB BELZ

From high school sports to college wrestling to bull riding and marathon running, I have been active in sports all my life. I thought CMT had missed me, but in my 60s, I began to show signs of CMT. I went from orthotics to a cane and finally to a walker. My major problems are balance issues and leg weakness.

Golf was my last sport—I have played most of my adult life—but at 82, it was a challenge. I could not swing a golf club without falling down, could not walk the hills and could not get in or out of a sand bunker. I did not want to sit around all day, so I did some research and found the SoloRider golf car (www.solorider.com). SoloRider is a specially designed golf car for people without legs or an inability to use the legs as intended. The seat swings out manually, then electronically stands the driver up. The driver is held in place by a belt around the waist and the chest, if necessary.

The car is designed so the user can drive onto the teeing ground and on the green so the user can putt. Regal Research gave me the names of golf courses in my areas that had SoloRider golf cars to rent so I could try one out. I took one to the driving range several times before I decided I needed one. I now play golf at least three times a week and go to the driving range and the practice putting green a couple days a week—not bad for an 82-year-old man! I need help teeing up the ball and raking the bunkers, but no one seems to mind helping me. Other players are very encouraging and helpful.

We each have to defeat CMT in our own way, and with SoloRider, it looks like I will be playing golf for a long time! ★

Cycle 4 CMT!

Sunday, August 26, 2018
The Old Lantern, 3260 Greenbush Rd, Charlotte, VT

Help support CMT research while enjoying incredible cycling and/or walking amid the breathtaking views of Vermont's Lake Champlain and the Green Mountains.

Not a cyclist or walker? No problem! Register for the event after-party and join us for live music, a delicious meal, great conversation with CMT experts and an impressive silent auction.

Schedule of Events

Cycle 4 CMT (8:00 am – 11:30 am) Ride either 6.5, 15, 25 or 40-mile routes through scenic Vermont towns with fantastic views of Lake Champlain, the Adirondacks and the Green Mountains. Find route details at **www.cycle4cmt.com**.

Walk 4 CMT (10:00 am – 11:00 am) Walk one mile on the beautiful grounds of The Old Lantern and enjoy the scenery of Charlotte's countryside and Lake Champlain.

Event Party (11:00 am – 3:00 pm) Live music by Leno, Cheney and Young • Local Vermont beer and cider, as well as non-alcoholic drinks • Fully-catered meal, silent auction items from local sponsors, and more!

NEW FOR 2018!!
The Cycle 4 CMT Virtual Rider or Walker Program
Can't make it to Vermont this year? No worries. Anyone can join the fun from anywhere! Register to ride or walk virtually anytime between now and August 26. Invite friends to join in on the fun. We'll send your Cycle 4 CMT swag upon completion of your ride/walk.

Register or donate today at www.cycle4cmt.com

CMTA Branches

Most CMTA Branches can be accessed online at **www.cmtausa.org/branches**

ALASKA
Anchorage Area
Megan Rodgers
907-244-2100

ARKANSAS
Little Rock
Lisa Jones
501-776-5364
Candice Cargile
501-516-5588

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Danielle Metzger
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Los Angeles Area
Alani Price
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Sacramento
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Interested in starting a branch in your area?

Contact CMTA Director of Community Outreach Laurel Richardson at laurel@cmtausa.org.

CMTA BRANCH NEWS

ATLANTA, GEORGIA

Our thanks to the Atlanta branch of the CMTA for welcoming Jeannie Zibrida as its new branch leader.

The group met Saturday, March 24, for an informative talk by David Keane from GeneDx. He spoke about new advances in research and more variants being discovered. GeneDx has a very generous billing process, and they will mail a kit to homes of patients (cheek swab or oral rinse) with a physician's order. David has been very supportive of the CMT community and also has been educating Atlanta-area doctors. The Atlanta branch is very grateful to David and for having GeneDx as a corporate sponsor.

BALTIMORE, MARYLAND

The Baltimore branch of the CMTA had great meetings on Saturday, February 18 and Sunday, May 20.

Guest speakers at the February meeting included CMTA board member and chronic pain expert Elizabeth Ouellette (via Skype) and Jed Newhart, a brace expert at Dankmeyer, Inc. CMTA board member Steve O'Donnell was also at the meeting as were Kimberly Kellner and her new support dog, Tigger.

The guest speaker at the May meeting was University of Pennsylvania neurologist Dr. Steven Scherer. CMTA board member Steve O'Donnell also was at the meeting. There were 28 attendees, including a handful of new group members. The Oxford Funathlon was on Saturday, June 9. The next meeting will be in September 2018.

EASTON, MARYLAND

The Easton branch of the CMTA had a great meeting on Saturday, February 17.

Guest speakers included CMTA board member and chronic pain expert Elizabeth Ouellette (via Skype) and Jed Newhart, a brace expert at Dankmeyer, Inc.

GRAND RAPIDS, MICHIGAN

The Grand Rapids branch met on February 21. The group discussed a new anti-sense drug in a clinical trial that is showing signs of stopping the progression of CMT1A, and in some cases, even reversing symptoms of the disease. Ionis, based in San Diego, is the lab doing the work.

The group also discussed strategies to help with hands, including silver ring splints, theraputty and occupational therapy.

Stephanie, an occupational therapist with Family Tree Therapies, offered a free month of services for someone within the group to check out her approach and report back. The group held a drawing, and Pam Yuhas was the winner.

The group discussed speaker ideas for 2018 and expressed interest in therapists and counselors for grief management and coping skills, a geneticist and a physical therapist. Topics of interest included Centers of Excellence, acupuncture and the Ionis and Acceleron clinical trials.

Fundraiser ideas included a walk, a dinner, tree wrapping, bracelet sales, a motorcycle ride, T-shirt sales and a walk/T-shirt sales/meal combination event. Those interested should take the poll on Facebook to choose an event.

MADISON, WISCONSIN

Twenty people, including new members, attended the March 8 meeting. CMTA STAR Advisory Board Chair Dr. John Svaren presented positive and informative updates on the CMTA and CMT research advances. Following a few announcements, there was a round table discussion that included introductions and a time to share.

In May, 12 members of the Madison branch gathered to hear guest speaker Abby, a certified yoga instructor. Abby showed the group stretches that can be done on the floor or in the bed. The group also discussed what's going well, challenges and how to help others find solutions to challenges.

The inaugural Madison CMTA picnic was on Saturday, July 28 at Deb and Tom's home in Sun Prairie, Wisconsin. Everyone was invited!

Donations in memory of branch member Greg Nametz may be made at www.cmtausa.org/donate/gregnametz/.

DENVER, COLORADO

There were 14 members at the April meeting, where there was a very educational discussion on the physics of movement and walking, the technology available for orthotics and bracing and what to consider in approaching the various orthotic options. The group is grateful to Chris Cumsille and everyone in the room for their time.

MELBOURNE, FLORIDA

The Melbourne, Florida CMTA Branch had its inaugural meeting on March 3 at the Aquarina Beach Club on Highway A1A in Melbourne Beach. There was a great turnout: more than 20 people attended to hear special guest speaker Jeana Sweeney. The next meeting will be held in early June and will be hosted by Maritza, the new branch co-leader. The group is honored and thrilled to add another branch to the CMTA's growing tree of awareness and action.

NAPLES, FLORIDA

Twenty-four Naples, Florida branch members were treated to a double feature with dinner on March 1. Chris Toelle, area clinic manager for Hanger Orthotics, and Lance Harm, Naples practice manager for Hanger, gave a presentation on "What Device is Best for You." They brought examples of many front- and rear-supported plastic and composite AFOs and explained the features that best met patients' symptoms. They demonstrated a deep understanding of challenges faced by people with CMT—and to top it off, they brought dinner.

The second feature was CMTA Director of Development and Co-Director of Camp Footprint Jeana Sweeney, the wonderful lady who helped Roy Behlke launch the

UNDERSTANDING CMT: A GUIDE FOR PATIENTS, PHYSICAL THERAPISTS AND OCCUPATIONAL THERAPISTS

Having a hard time finding a physical therapist (PT) or occupational therapist (OT) who knows about CMT? Fret no longer! Thanks to our expert clinicians, the CMTA is on the verge of publishing a guide for patients, PTs and OTs outlining the assessment and care of people with CMT. Because CMT manifests so differently from person to person, this guide, based on research, experience and patient input, will optimize collaboration between physical and/or occupational therapists and patients, delivering the best possible care for an individual's specific needs. This well-written document will be available soon, empowering people with CMT and their health care professionals to make safe, educated decisions regarding a rehabilitative plan of action to maintain function and quality of life.

CMTA

UNDERSTANDING CMT

A Guide for Patients, Physical and Occupational Therapists

Who Are Occupational Therapists?

Naples branch five years ago. Jeana started by surveying the attendees' types of CMT—five 1As, two 2As, one 4A and one 1B. The group mirrored the overall CMT population with 60 percent of the group having CMT type 1A. She relayed the good news that CMTA has active research programs on all the types mentioned and has had a recent breakthrough on this type of CMT. Ionis Pharmaceuticals has published a paper on their new research drug showing success in reducing and reversing symptoms of CMT1A in laboratory animals.

Jeana stressed the importance of fundraising, as the next steps leading to clinical trials in humans will require large investments, and she also discussed the success of Camp Footprint. Twelve-year-old Juliette Beliveau attended the camp last summer and told of her new experiences and the fun she had horseback riding, rock climbing, dancing, canoeing and tie dyeing with other kids who have CMT.

★

SACRAMENTO, CALIFORNIA

The Sacramento, California branch had a wonderful meeting on May 19. There were 10 people in attendance, and the group welcomed two new members. Group members enjoyed telling all of their stories and sharing resources. The group talked about the patient side as well as the caregiver side. The social and backyard potluck was July 28.

★

SEATTLE, WASHINGTON

The Seattle branch welcomed several new faces. There was a great turnout of about 40 people, including Dr. Weiss and his crew. Elisa talked about fundraisers that happened in May and June.

Denise and her husband, John, shared their adventure looking at some amazing stair lifts that they found in Fife. They also found many devices to help people with CMT. Shingi and his dad, Robert, spoke about a new device his physical therapist invented that helps him open bags and other hard-to-manage items. The group also discussed four books related to CMT: "CMT Disease," "Conquering CMT," "Running for My Life" and "CMT and Me."

Dr. Weiss discussed nerve testing and answered questions. Many thanks to those who volunteered to do the test.

★

TORONTO

At the January meeting, the group welcomed Shawn Meirovici, ND, a naturopathic doctor who specializes in treating patients with multiple sclerosis, brain injury, spinal cord injury and other neurological diseases. He gave an excellent presentation with slides on many topics: sugar, diet, nerve pain, protecting nerves, cramps and spasms, and available tests, such as for hormones, toxins and food sensitivities. Notes from his presentation will be distributed in a separate document/email. The group is really grateful to Dr. Meirovici for giving up part of his busy Saturday to educate the group!

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THE CMTA Report

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CMT PATIENT MEDICATION ALERT:

Definite high risk (including asymptomatic CMT):

Taxols (paclitaxel, docetaxel, cabazitaxel)
Vinca alkaloids (Vincristine)

Moderate to significant risk:

Amiodarone (Cordarone)
Arsenic Trioxide (Trisenox)
Bortezomib (Velcade)
Brentuximab Vedotin (Adcetris)
Cetuximab (Erbitux)
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Nitrous oxide (inhalation abuse)
Nivolumab (Opdivo)
Pembrolizumab (Keytruda)
Perhexiline (not used in US)
Pomalidomide (Pomalyst)
Pyridoxine (mega dose of Vitamin B6)
Stavudine (d4T, Zerit)
Suramin
Thalidomide
Zalcitabine (ddC, Hivid)

Uncertain or minor risk:

5-Fluouracil
Adriamycin
Almitrine (not in US)
Chloroquine
Cytarabine (high dose)
Ethambutol
Etoposide (VP-16)
Gemcitabine
Griseofulvin
Hexamethylmelamine
Hydralazine
Ifosfamide
Infliximab
Isoniazid (INH)
Lansoprazole (Prevacid)
Mefloquine
Omeprazole (Prilosec)
Penicillamine
Phenytoin (Dilantin)
Podophyllin resin
Sertraline (Zoloft)
Statins
Tacrolimus (FK506, Prograf)
Zimeldine (not in US)
a-Interferon

Negligible or doubtful risk:

Allopurinol
Amitriptyline
Chloramphenicol
Chlorprothixene
Cimetidine
Clioquinol
Clofibrate
Cyclosporin A
Enalapril
Glutethimide
Lithium
Phenelzine
Propafenone
Sulfonamides
Sulfasalazine

WHAT IS CMT?

- More than 2.8 million people worldwide have CMT, which is one of the most commonly inherited nerve disorders and affects the motor and sensory nerves.
- CMT is slowly progressive, causing the loss of muscle function and/or sensation in the lower legs and feet, as well as hands and arms.
- Men and women in all ethnic groups may be affected by CMT.
- CMT is genetic, but it can also develop as a new, spontaneous mutation.
- CMT can vary greatly in severity, even within the same family.
- CMT causes structural deformities such as high-arched or very flat feet, hammertoes, hand contractures, scoliosis (spinal curvature) and kyphosis (rounded back).
- CMT can also cause foot drop, poor balance, cold extremities, cramps, nerve, muscle and joint pain, altered reflexes, fatigue, tremor, sleep apnea, hearing loss and breathing difficulties.
- CMT rarely affects life expectancy.
- Some medications are neurotoxic and pose a high risk to people with CMT, notably Vincristine and Taxols. See full list (at left) of medications that may pose a risk.
- More than 100 different genetic causes of CMT have been identified.
- Many types of CMT can be determined by genetic testing. Please consult with a genetic counselor or your physician for more information (www.nsgc.org).
- Although there are no drug treatments for CMT, a healthy diet, moderate exercise, physical and/or occupational therapy, leg braces or orthopedic surgery may help maintain mobility and function.
- The CMTA's STAR research program and extensive partnerships with pharmaceutical companies are driving remarkable progress toward delivering treatments for CMT, bringing us closer to a world without CMT.