FALL 2022

THE CMTA REPORT

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THE CMTA'S INCREDIBLE EXPANDING UNIVERSE

CAMP FOOTPRINT: IN PERSON AGAIN AND GROWING

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THE CMTA REPORT | FALL 2022

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A MESSAGE FROM THE CEO

Dear Friends,

The CMTA has seen meteoric growth in 2022. Rather than dribbling out the announcements one at a time, we decided to gather them in one issue to present a clearer picture of the CMTA's flight path. We hope this view of the CMTA brings the picture of our growth into sharper focus, just as the Webb telescope brought the galaxy into sharper view.

There are several new stars in the CMTA firmament. Jon Pastor brings to the Board of Directors experience leading consulting teams focused on operations improvement and growth strategies for Fortune 500 companies, along with an MBA from the Harvard Business School. He has CMT1A.

The CMTA recently welcomed Dr. Katherine Forsey to the newly created post of chief research officer, a move necessitated by the explosive growth of the CMTA's Strategy to Accelerate Research (STAR). Forsey, a research biologist by training, will play a critical role as clinical development efforts heat up, particularly with the biopharma industry and leading gene therapy labs around the globe. Forsey, who has CMT1A, will play a critical role in advancing CMT research, especially as many of our alliance partners prepare for clinical development efforts.

We also have three new members on our STAR Advisory Board—Dr. Bruce Conklin, Dr. Vera Fridman and Dr. Diana Bharucha-Goebel. They bring a wealth of knowledge to the CMTA with expertise in gene editing, natural history studies and disease-focused outcome measures.

On the programming side of things, we are extremely excited about our first-ever CMT Clinical Summit to educate health care professionals about CMT on Nov. 4. Please be sure to let your physicians know.

We have two exciting announcements about our youth programming. There's a new support and advocacy group, called COMPASS, aimed at creating a home for those who have aged out of the CMTA's Youth Program, which provides services to those 8 to 18. And Camp Footprint will open a second location on the West Coast in 2023.

We are so excited to share our expansion plans with you, our community. We couldn't undertake these life-changing explorations without you.

Warmly,

Amy Gray

Amy Gray
CMTA Chief Executive Officer

CONGRATULATIONS DR. SHY! As we went to press, CMTA Board Member Dr. Michael Shy was awarded an endowed chair by the University of Iowa for his work on CMT. He now holds the Carver College of Medicine Endowed Chair in Inherited Peripheral Neuropathies. Outside of the chair in neurology, this is the only other endowed chair in the Department of Neurology. The CMTA is so proud to work with this leader in the field and offers Dr. Shy our most heartfelt congratulations and appreciation.

CMTA WELCOMES SOFTWARE ENTREPRENEUR JON PASTOR TO BOARD OF DIRECTORS

The CMTA Board of Directors voted June 21 to appoint entrepreneur and software executive Jon Pastor to the board.

Jon was most recently the chief product officer of RealPage, a publicly traded real estate software company that bought his start-up. He began his career at McKinsey & Company, leading consulting teams focused on operations improvement and growth strategies for Fortune 500 companies. He has a BA in chemistry from Case Western Reserve University and an MBA from the Harvard Business School. He and his wife Marni live in Pittsburgh, Pa. with their two children. Multiple generations of his family have CMT1A.

Jon said he "is honored to join an organization that is doing so much for patients, as well as aggressively and efficiently deploying resources to advance research."

CMTA Board Chair Gilles Bouchard said, "Jon is a terrific addition to our organization, bringing youthful energy and inquisitiveness, combined with a sharp mind and a broad and successful business experience."

Growth in STAR Portfolio Leads To New CMTA Position

The CMTA welcomed Katherine Forsey, PhD, to the newly created post of chief research officer on June 2. The move came in response to the explosive growth of the CMTA's signature research program, the Strategy to Accelerate Research (STAR). CMTA-STAR is funding more research projects than ever before and expanding its network of leading CMT researchers across the globe.

Dr. Katherine Forsey

Forsey brings to the CMTA a rich and varied portfolio of work experience in both for-profit and nonprofit sectors, along with specializations in science communication, education and program leadership.

"I am honored to join the CMTA as chief research officer. The CMTA is making huge strides toward a world without CMT. With the momentum we have, and with the many talented and passionate people we have working with us, I am confident we will deliver much-needed advances for patients in the years ahead," Forsey said.

Forsey, a research biologist by training, holds a doctorate in biology from the University of York in the area of expression, activity and localization of metabolic enzymes during preimplantation embryo development. During her industry-funded PhD, she spent time at the University of Cambridge, UK; in Sygen-sponsored research laboratories and at the Veterinary Research Institute, Brno, Czech Republic. She also earned her BSc Hons. in molecular cell biology from the University of York. During her undergraduate degree, she completed a year in industry undertaking a genetic toxicology research project at Syngenta to develop in vitro alternative assays.

CMTA CEO Amy Gray said of the appointment, "As STAR continues to gain unprecedented momentum, particularly with the biopharma industry and leading gene therapy labs around the globe, Dr. Forsey will play a critical role in advancing CMT research, especially as many of our alliance partners are preparing for clinical development efforts."

Forsey, who has CMT1A, lives in Yorkshire, England with her husband and two children. She will be the CMTA's only staff member outside the United States, giving the organization the opportunity to expand its footprint with industry and researchers worldwide.

Three New Members Added To STAR Advisory Board

The CMTA named three new members to its STAR Advisory Board recently, adding expertise in gene editing, natural history studies and disease-focused outcome measures.

The STAR Advisory Board comprises three distinct entities. The Scientific Advisory Board (SAB) provides scientific input for ongoing or proposed projects. The Therapy Expert Board (TEB) evaluates the translational quality of ongoing and proposed projects. Finally, the Clinical Expert Board (CEB) provides expert guidance and support to the CMTA's alliance partners, helping to ensure the success of clinical trials.

DR. BRUCE CONKLIN is joining the SAB. He is a senior investigator at the Gladstone Institutes, professor at the University of California San Francisco (UCSF) and deputy director of the Innovative Genomics Institute founded by Nobel Prize winner Jennifer Doudna, PhD.

The Conklin Lab focuses on biomedical applications of CRISPR gene editing technology with an emphasis on motor neuron diseases, including CMT. After decoding human genetics, models of CMT are developed that can be directly treated with gene correction or targeted drug therapy. Editing out the disease-causing parts of the DNA sequence using CRISPR gene editing could provide a cure for CMT.


Dr. Vera Fridman


Dr. Bruce Conklin

Conklin's lab uses induced pluripotent stem cells (iPSCs) collected from adult CMT patients to create model motor neuron cells. They use these model cells to test the effects of the CRISPR gene editing treatments they are developing. If the treatment is found to be effective in the human model cells, a treatment for CMT patients is one step closer.

The CMTA has funded Conklin's research on CRISPR therapies for CMT2A, CMT2E and CMT2F. The promising findings from his initial research suggest that this approach could also be used for many other types of CMT.

Dr. Katherine Forsey called Conklin a leader in the development of genome surgery approaches to treat CMT, adding, "His knowledge and expertise will help guide and inform the CMTA's Strategy to Accelerate Research and enable us to drive progress towards effective treatments for all types of CMT."

DR. VERA FRIDMAN is joining the CEB. She is an associate professor in the Neuromuscular Section at the University of Colorado's (CU) Anschutz Medical Campus, clinical director of the CMTA Center of Excellence at the University of Colorado Hospital and associate director of Medical Student Education for CU Hospital's Department of Neurology.

Fridman's research focuses on removing barriers to effective clinical trials by performing natural history studies, identifying the best measures of disease progression and investigating new biomarkers and therapeutic targets. In collaboration with the Inherited Neuropathies Consortium, she carried out three major natural history studies for CMT, including the largest cross-sectional natural history study in various forms of CMT, the largest longitudinal study of CMT1A and the recently completed longitudinal study of CMT1B.

In collaboration with Dr. Florian Eichler, Fridman conducted a clinical trial evaluating the effects of high-dose L-serine supplementation in patients with Hereditary Sensory Autonomic Neuropathy Type I (HSAN1). In one of the first biologically rational therapies for a hereditary nerve disease, the trial demonstrated clinical improvement of neuropathy in response to treatment.

Forsey said, "Dr. Fridman's expertise, knowledge and close links with the CMT patient community will be essential to the CMTA's strategy to accelerate research and the effective development of clinical trials as we move treatments into the clinic."

DIANA BHARUCHA-GOEBEL, MD, PHD, a pediatric neuromuscular neurologist and clinical director of the CMTA Center of Excellence at the Children's National Hospital in Washington, D.C., is also joining the CEB.

Dr. Bharucha-Goebel is director of the neuromuscular program and associate professor in the Division of Neurology at Children's National Hospital in Washington, D.C., and the Division of Pediatrics and Neurology at the George Washington University School of Medicine & Health Sciences. She

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NEW STAR ADVISORY BOARD MEMBERS

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is a clinical research collaborator in the Neuromuscular and Neurogenetic Disorders of Childhood Section in the National Institute of Neurological Disorders and Stroke at NIH.

Quickly translating scientific research and discoveries into practice is Bharucha-Goebel's overall aim, with a particular focus on the evaluation and identification of disease-focused outcome measures in rare disease. She led the clinical development of a Phase I first-in-human gene transfer trial for individuals with giant axonal neuropathy, while concurrently performing a parallel natural history study to assess outcome measures in this disorder. She is also interested in viral mediated gene transfer treatment approaches, studying total vector genome dose, parallel preventative immune system modulation and immune system monitoring following gene transfer.

In the clinic, her team provides comprehensive and specialized care for pediatric CMT patients, with expertise in genetic and electrophysiologic diagnostics and specialized family-centered care in orthopedics, physical and occupational therapies, physical medicine and rehabilitation, neurology, nutrition and bone health.

"Dr. Bharucha-Goebel's pediatric specialty, dedication to scientific progress and passionate patient care will help to inform our Strategy to Accelerate Research, particularly in the areas of clinical trial readiness and the safe roll-out of gene therapy approaches," Forsey said.

Dr. Diana Bharucha-Goebel

FIRST CLINICAL SUMMIT TO EDUCATE HEALTH PROFESSIONALS ABOUT CMT

The CMTA isn't just expanding its numbers, it's expanding its services. On Nov. 4, the CMTA will host the first-ever CMT Clinical Summit to educate health care professionals about CMT. The summit will include sessions on:

- CMT neurology/patient diagnostic journey
- Genetic testing
- Clinical studies/trials and patient recruitment
- Surgical intervention: When is it time to see an orthopedic surgeon?
- Physical therapy
- Occupational therapy
- CMT-related breathing impairments
- 504/IEPs–getting help in school

The goal of the summit is to improve the diagnostic journey and patient care by providing world-class professional medical training to health care professionals, including general practitioners, neurologists, genetic counselors, nurses, physician's assistants, physical therapists, occupational therapists, foot and ankle specialists, pulmonologists and social workers. Each session will be accredited, and CME (Continuing Medical Education) units will be awarded to attendees. Sessions will be led by experts from CMTA Centers of Excellence.

According to Laurel Richardson, director of community outreach for the CMTA, "The feedback that we hear most often from the community is that their physician and/or care team does not know what CMT is. We want to be at the forefront of solving this problem by being education leaders for CMT. In addition to finding a treatment for CMT, our mission is to improve the quality of life for people living with CMT, and we won't rest until that happens."

Katherine Forsey, the CMTA's chef research officer, said, "Clinicians are on the front line of CMT patient care. With rapid advances in the field, the identification of novel genetic causes, and the arrival of clinical trials, the CMTA's Clinical Summit provides a unique opportunity for health professionals to update and advance their CMT knowledge."

The CMTA is asking community members to let their health care providers know about the clinical summit.

The CMTA is also sponsoring a virtual Patient & Research Summit on Oct. 1. More information about both meetings can be found at https://summit.cmtausa.org/.

CMTA STARTS COMPASS GROUP FOR YOUNG ADULTS

The CMTA's Youth Program, which has grown exponentially in the three years since it was formally established, is expanding again. COMPASS is a new support and advocacy group aimed at creating a home for those who have aged out of the CMTA's Youth Program, which provides services to those 8 to 18.

COMPASS
NAVIGATING CMT
AS A YOUNG ADULT

Jonah Berger, national youth programs manager, says COMPASS is for young adults with CMT between the ages of 19 and 30, "a time of transition in any life, from youth to college to employment and beyond. COMPASS hopes to unite our young adults and empower them to navigate the waters of CMT and adulthood."

Eighteen young adults attended the inaugural meeting for COMPASS in June. They decided to meet monthly to socialize and connect. Each meeting will also include an advocacy and educational component on topics like dating, college, bracing, exercise, driving, employment, accommodations and mental health.

"Every stage of life brings its own unique gifts and challenges," Berger said, and "the CMTA is always working to support our community through all the stages of life. If the group that came together in June is any indication, the COMPASS group is going to be another amazing piece of support in the journey through life with CMT."

Questions about COMPASS? Email Jonah at jonah@cmtausa.org.

Camp Footprint To Add West Coast Site in 2023

In other expansion news, plans are afoot for a West Coast summer camp for kids with CMT, starting in 2023.

The original Camp Footprint, started in August 2016 near Pittsburgh, has been an unbridled success. For one week a year, campers who have never met another kid with CMT find each other in what they call The Tribe of the Funky Feet. Camp Footprint (motto: One Step at a Time) gives campers the chance to connect with other kids with CMT, master their environment, participate in activities planned just for them and celebrate their abilities.

But to families west of the Mississippi, "Camp Footprint East" was geographically challenging. And families whose children attend schools that start in August needed an early-summer alternative to the late-summer East Coast camp. In a survey of West Coast families with kids who have CMT, some 90 percent expressed interest in a closer camp option.

Starting a West Coast camp will add new community to the CMTA, increasing the number of youth and families served and spotlighting future leaders.

The camp will follow the East Coast pattern closely, replicating existing plans and infrastructure. It will start small and grow, as Camp Footprint East did.

Planners have already identified a possible home for Camp Footprint West—B'Nai B'rith Camp in Lincoln City, Ore. Two hours from the Portland airport and five minutes from the Pacific Ocean, the camp offers the same activities as the Pennsylvania camp, is close to emergency services and is ADA compliant.

The bottom line, Camp Footprint Director Jonah Berger said, is that the tools to start Camp Footprint West are in place and, "The time is now." Registration for both camps opens January 5, 2023. Contact Jonah with any questions about either camp at jonah@cmtausa.org.

UNDER THE MICROSOPE

In our new column, Under the Microscope, CMTA Chief Research Officer Dr. Katherine Forsey will take a closer look at all issues related to the CMTA's Strategy to Accelerate Research, from investigators to contract research organizations to Centers of Excellence and university labs. In her first column, she explains how our preclinical testing alliance partnerships work.

Accelerating Research Through Alliance Partnerships: ORTHOGONAL NEUROSCIENCE

The CMTA is also expanding its universe by collaborating with leaders in the field of drug development to form preclinical testing alliance partnerships. Orthogonal Neuroscience is one such partner, working with the CMTA to accelerate its research into the human monoclonal antibody ORT247.

As Dr. Michael Ahlijanian, the company's chief scientific officer explained, ORT247 prevents axonal collapse and preserves the motor neuron's connection to the muscle, thereby maintaining function and strength. Three mechanisms could benefit CMT patients:

1. **Protecting connections** by maintaining existing motor neuron connections with, and preventing retraction from, the muscle
2. **Restoring connections** by promoting axon regeneration in motor neurons that have died
3. **Growing new connections** by stimulating neighboring axons to extend their reach into areas where connections have been lost

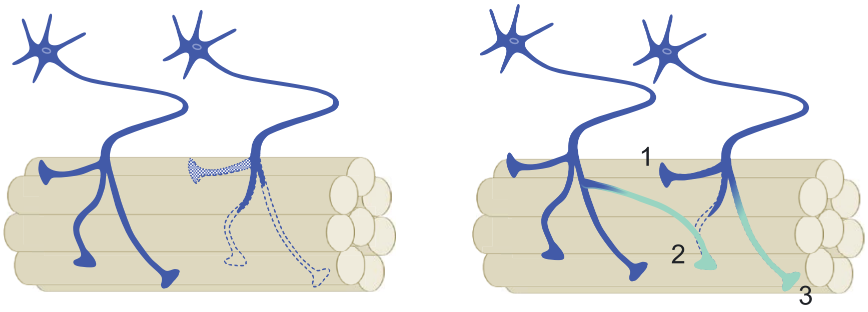
Through the CMTA's Preclinical Testing Alliance, Orthogonal gained access to the CMTA's service research providers and preclinical testing network. The CMTA provided bespoke support around the selection of appropriate models, design of the experiments, data interpretation and guidance on what future experiments will be needed to take the project forward. The CMTA also made introductions to key clinicians and thought leaders to facilitate future clinical trials in CMT patients.

The CMTA Preclinical Testing Alliance facilitated ORT247 tests in CMT1A (demyelinating) and 2E (axonopathy) mouse models and the results show an improvement in several disease endpoints. In both models, improvements in gait analysis and neuromuscular junction function (shown through electrophysiology) and a reduction in blood serum biomarkers of axonal damage were seen. The CMT1A model also showed improved grip strength. In the CMT2E model, balance beam performance improved, and neuromuscular junction innervation was fully restored.

Orthogonal followed these positive results with a standard toxicology program to demonstrate treatment safety, a three-month and a six-month study in non-human primates, and an investigational new drug (IND) application to the U.S. Food and Drug Adminis-

THERAPEUTIC HYPOTHESIS:

EPHA4 RECEPTOR INHIBITION COULD IMPROVE NEUROMUSCULAR JUNCTION FUNCTION IN CMT



Dying motor neurons retract axon terminals from synapses that innervate muscle fibers

Three mechanisms of ORT247 action could benefit CMT patients by:
1) Protecting connections
2) Restoring connections
3) Growing new connections

Dr. Michael Ahlijanian

tration—all crucial steps before the commencement of a human trial.

In June, the first study of ORT247 in humans started with normal healthy volunteers. It will run to at least the end of 2022. Dr. Ahlijanian's hope is that trials commence in CMT patients by mid-2023.

The hope is that ORT247 treatment will provide stabilization or an outright improvement in strength and muscle function along with reductions in key biomarkers for CMT patients, not just in CMT1A and CMT2E, but in all types of CMT. ORT247 treatment could also work alongside any future gene-targeted therapies to potentially restore muscle function.

Speaking about the benefits of the Preclinical Testing Alliance, Dr. Ahlijanian said, "This CMTA Alliance partnership ensured Orthogonal performed the right experiments in the right models, driving the research forward through provision of expert testing and analysis capacity."

He added, "We are working very hard to develop therapeutics that will provide benefit for CMT patients. Progress can be frustrating at times, but that only increases our incentive and drives us to work harder."

Dr. Mark Scheideler, chair of the CMTA Therapy Expert Board, said, "The CMTA Preclinical Testing Alliance program has allowed Orthogonal to join our STAR consortium network as a working partner. This has provided a means for Orthogonal to explore if its novel antibody therapy approach can be used to treat CMT and reduce the burden of the disease on patients."

CMTA BOARD APPROVES $206,018 GRANT TO INC FOR ADDITIONAL PREP WORK FOR CLINICAL TRIALS

The CMTA Board of Directors approved a $206,018 grant to the Inherited Neuropathy Consortium (INC) June 21. The grant funds additional work to prepare for clinical trials, including outcome measures and biomarkers, at four of INC's 20 sites.

INC is an integrated group of academic medical centers, patient support organizations and clinical research resources dedicated to conducting clinical research on different forms of CMT and improving patient care. Funded primarily by the National Institutes of Health (NIH), with supplemental funding from the CMTA and the Muscular Dystrophy Association, INC plays a key role in developing the infrastructure necessary to evaluate CMT therapies.

INC is currently working with pharmaceutical and academic partners on multiple clinical trials that are under way or in the planning stages, including treatments for CMT1A, CMT1B, CMT2A, CMT1X and other rarer forms such as SORD recessive neuropathy.

Outcome measure and biomarker studies are "fundamental to bring clinical trials to CMT," grant reviewers said, adding that "longitudinal assessments in the ongoing studies are critical, and completion of these studies in a rigorous fashion is a very high priority for translational development."

Noting that INC is the only group capable of performing the proposed projects, CMTA reviewers said it is "highly probable" that the grant will lead to a patent, license, external funding through biomarker development, new gene discoveries and therapeutic clinical trials.

With previous CMTA support, INC enrolled almost 7,000 patients into its registry and developed CMT-specific clinical outcome assessments (COA) to measure disability in adults and children with CMT. INC also identified several biomarkers, including MRI imaging, skin biopsies and plasma markers, and applied wearable sensors to obtain sensitive gait and balance data during activities of daily living, yielding a "digital biomarker" with enhanced responsiveness to change. INC's earlier work has reduced the typical clinical trial risks of not achieving expected outcomes or lacking the statistical power to obtain meaningful results.

Ongoing biomarker studies funded by the CMTA (for CMT1B, CMT1X, CMT2A, and CMT2F) depend on the INC infrastructure. Reviewers said that while the scope of the proposed plan is large, INC has a track record of success and will recruit personnel to spearhead many of the wide-ranging initiatives: The grant will fund at least six full-time trainees to become independent investigators in CMT-related research; expand the number of full-time trainees and provide training to additional postdoctoral fellows and students.

Mayo Clinic Researchers Find Surgery Increases Grip Strength in 1A, HNPP Patients

In the first surgical procedure shown to help with grip strength, researchers at the Mayo Clinic in Minnesota found that nerve decompression surgeries led to symptom improvement in CMT1A and HNPP patients with carpal and cubital tunnel syndrome.

Carpal tunnel syndrome (CTS) affects the thumb, index and long fingers, while cubital tunnel syndrome (CuTS) affects the small and ring fingers. Both are common among patients with CMT1A, which results from duplications of the PMP22 gene, and hereditary neuropathy with liability to pressure-palsies (HNPP), which results from deletion of the PMP22 gene.

Led by Dr. Christopher J. Klein, the study included a group of 112 patients—60 with HNPP and 52 with CMT1A. CTS was identified in 32 HNPP patients and six CMT1A patients, while CuTS was identified in 26 HNPP patients and six CMT1A patients.

As the researchers noted in a paper to be published in Muscle & Nerve, poor hand function contributes to reduced quality of life in both CMT1A and HNPP patients. In fact, they said, "Quality of life and disability measures in CMT have been found to be equivalent to that of stroke patients when upper extremity hand function is included in the disability assessments."

After CTS surgery, pain, sensory loss, and strength improved in 4 out of 5 CMT1A, and 6 out of 10 HNPP patients. One CMT1A and one HNPP patient improved with CuTS surgery, while two HNPP patients worsened.

In the future, the researchers wrote, incisionless thread release and ultrasound-guided carpal tunnel release should be investigated, because they are predicted to pose less of a surgical risk. "Until genetic therapies become available, this approach provides a meaningful therapeutic option to help patients."

MORE THAN ONE-THIRD OF 1A PATIENTS REPORT BEING DIAGNOSED WITH DEPRESSION

More than a third of 2,800 patients with CMT1A reported that they have been diagnosed with depression, according to results from the CMT&Me study presented at the most recent annual meeting of the Peripheral Nerve Society (PNS).

"The fact that the diagnosis of depression is high in this study population is not surprising for a disease with this symptom burden; however, depression itself as a co-morbid condition represents significant disease burden and can affect treatment and outcomes for CMT1A," researchers said. They added that this connection warrants further analysis and exploration.

Using a digital app called CMT&Me, participants used their smartphones to complete surveys at their convenience. The app immediately submits the information to a central database. A Scientific Advisory Board oversees the CMT&Me study, and includes clinicians who care for CMT patients, PAO representatives, and experts in patient-reported outcomes (PRO) and data management. All patient data remains anonymous, and researchers who access the CMT&Me study database will not be able to identify patients.

Funded by the French company Pharnext, CMT&Me launched in October 2018 both in Europe and the United States, to collect self-reported data from patients with CMT on a regular basis. This study is managed by the company Vitaccess in collaboration with patient advocacy groups and key opinion leaders in the field. The app is available in English, German, French, Italian and in culturally adapted Spanish language versions for Spain and the United States.

In another study presented at the PNS annual meeting, the authors found that patients with 1A reported a high level of symptom burden, including physical limitations, cramps, pain and fatigue. Almost half of participants rated the cramps they experienced as either moderate or severe with 8 percent reporting very severe cramps. The reported symptom burden was mostly similar across countries, although there were some considerable differences in the ranking of symptoms. The authors said that the difference in symptom ranking could drive variation among countries in health-related quality of life, healthcare resource use and costs.

Finally, the study found CMT1A had a "significant" impact on participants' work status. Twenty percent of participants weren't working due to disability and those who were working reported issues such as choice of job, number of days taken as sick leave and having to work part-time because of their CMT. Researchers said that while the database is still not "mature enough" to make extrapolations on an annual basis, a mean 1.4 days of work missed every two weeks could translate to 36 days of work missed per year. Further research is needed to better explore the indirect costs associated with these impacts and manage 1A's impact on patients' work lives.

In an earlier CMT&Me study, participants also reported extreme fatigue from their CMT, rating it 6.8 on a scale of 10. Spain and Italy had the lowest scores, while German and British participants had the highest.

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CMTA

CMT PATIENT & RESEARCH SUMMIT

VIRTUAL SATURDAY, OCTOBER 1, 2022

TOPICS INCLUDE:

STAR Research, Path to a Cure
Is CMT a Multi-System Disease?
Live Your Best Life Today
Balance Work & Fall Prevention
Hand Functionality & Mobility
New CMT Surgical Outcomes
Cutting-Edge Bracing and More!

Hear Keynote Speaker Kenneth Hill Jr.'s inspiring story about being a Martial Arts Hall of Fame Inductee and CMT Warrior

REGISTER TODAY AT SUMMIT.CMTAUSA.ORG

IT'S CMT AWARENESS MONTH
ROCK & ROLL INTO A WORLD
CMTA
WITHOUT CMT

AWARENESS MONTH 2022:

ROCK AND ROLL INTO A WORLD WITHOUT CMT

Grab those old records off the shelf and dig out your vintage T-shirts–it's time to Rock and Roll into a World Without CMT. This September, the CMTA is celebrating our 12th CMT Awareness Month, working with the rockstars in our community to educate newly diagnosed community members, friends, families and the public about the disease.

CMT Awareness Week was born in September 2010, as the CMTA community began to educate others about CMT. Over the years, one special week morphed into a full month of special activities, from interactive webinars to in-person Walks 4 CMT.

Awareness Month 2022 celebrates the rockstars in the CMT community, including CMT patients, families, advocates, clinicians and researchers. We're asking everyone to take part in fun social and digital challenges and activities.

For example, the TikTok and Instagram Rockstar challenge invites participants to play an instrument with oven mitts or socks on their hands to demonstrate the difficulties community members have with fine motor skills. They then post videos and challenge others to do the same.

Participants are invited to honor the CMT rockstars in their lives by nominating them to the CMTA's virtual Hall of Fame. A $5 donation gets their names on a virtual vinyl record. They're also encouraged to add an Awareness Month frame to their Facebook pictures and to use the following hashtags in social media posts: #CMTAM22, #CMTAwareness, #1in2500.

Let's teach all those we know about CMT, welcome new community members to the CMTA and rock and roll into a world without CMT!

Join the CMTA's Rockstar Challenge on TikTok and Instagram!

SUPPORT THE CMTA IN THE FIGHT AGAINST THE PROGRESSIVE AND DEVASTING EFFECTS OF CMT

YES! I want to support STAR Research with a donation in the amount of:

○ $3,500 ○ $1,000 ○ $500 ○ $250 ○ $100 ○ $50 ☐ Other: $___________

○ Make my gift a monthly donation. ○ Please charge my credit card. ○ Visa ○ MasterCard ○ American Express

○ Check enclosed, payable to the Charcot-Marie-Tooth Association. Donate online at cmtausa.org/donate2star

To give the gift of stock, please call Jeana Sweeney at 814-269-1319

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Complete and mail to the CMTA, PO Box 105, Glenolden, PA 19036

9th Vermont Cycle4CMT Cruises To $2 Million Total for STAR

BY KAREN BROWN


Deb and Bob Haas

My friend Kate Lair and I met up in August for a weekend road trip to the 2022 Cycle4CMT in the Green Mountains of Vermont. It's always a treat to be in the company of someone who "gets" CMT—she's a CMTA Advisory Board member—and despite torrential rain, lightning, hail, and a (surprise!) ferry ride, we rolled into Burlington laughing all the way.

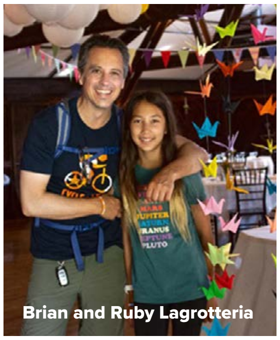
Vermonter and avid cyclist Chris Ouellette co-founded the event nine years ago with his sister Elizabeth to help cure the disease that affects Elizabeth's son Yohan. "The first year, it was mostly family and close friends. I didn't know how to run an event or mark trails, and people who meant to ride 10 miles ended up riding 20. But we raised $60,000 for the CMTA's STAR research program," laughed Chris. In its ninth year, the Cycle4CMT had almost 150 participants from coast to coast and is on track to generate more than $300,000 in new donations this year. Reaching this goal will bring the event's grand total raised for CMT research to $2 million!

The ride itself followed four clearly marked (great job, Chris!) routes of 6.5, 15, 25 or 41 miles through the picturesque Green Mountains. After the ride, there was live music, delicious food and drink, a silent auction and excellent company at the Old Lantern Inn & Barn. Various standard and adaptive bikes, including electric-boost recumbent trikes, were available to test-drive or use for the event, thanks to local sponsor The Ski Shop.

Cycle4CMT also invites walking or rolling and has grown to include a six-month virtual event and organized rides in Wisconsin and California. Last year, I walked 20 miles (not all at once) at home in Virginia. This year I rolled into Vermont in my new wheelchair and took a spin on an adaptive bike. The way you participate is up to you. (It's possible Chris chose "Cycle4CMT" because "CycleWalkRollOrDoWhateverYouWant4CMT" is a bit long for a T-shirt.)

As always, participants came out to support CMT research for different reasons. Dan and John Morgan have been connected to the CMT community for about 20 years through Dan's past work with prominent CMT researcher Dr. Steve Scherer at the University of Pennsylvania. The brothers, who have CMT1X, decided this was the year to participate in Cycle4CMT. When I asked John why they made the trip from New York City and New Jersey, respectively, he laughed. "Dan handed me the flyer and said, 'We're doing this.'" Dan's friend and colleague Rich Meesters joined them, and on Sunday morning the threesome tackled the 41-mile ride with a 2,806 feet elevation change. Team Morgan raised more than $29,000 for CMT research.


Cycle4CMT co-founders Elizabeth and Chris Ouellette (far right) with Dan and John Morgan.


Brian and Ruby Lagrotteria

Father/daughter duo Brian and Ruby Lagrotteria do not have CMT in their family, yet they joined from San Francisco. When I asked Brian why they traveled more than 2,000 miles to participate, he smiled and said, "For CMT. It's important to our friends, so it's important to us." He and 10-year-old Ruby completed the 6.5-mile route, her longest ride to date.

To participate in or organize a ride near you, contact Elizabeth Ouellette at elizabetho@outlook.com.

For more event details and to donate toward a $50,000 matching gift that began Sept. 1, 2022, visit cycle4cmt.com.

Karen Brown is a CMTA volunteer extraordinaire—Facebook Discussion Group moderator, Camp Footprint miracle worker, Southwestern Virginia Branch leader and writer for The CMTA Report.

THE CMTA GRATEFULLY ACKNOWLEDGES GIFTS…

In Memory of:

Warren Beals
Bradley Portin

Steve Cason
Mrs. Ardena Roberts

Thomas P. Cave
Diane and Hal Sheldon

Timothy A. Cave
Heidi DeConcini
Michael and Marylinn Hatch
Diane and Hal Sheldon

Cecilia Clemente
Judy Gostowski
Roseann Lanyon
Kimberly Nussbaum

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Richard Kaufman – "Happy Birthday!"
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Brock Shriver
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Rylee Sweeney
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Natalie Verduin
Pernod Ricard

Missy Warfield
Keith and A. Eletheer Decker

Harrison White
Donna Carlbom
Renee White

Beverly Wurzell – "Happy 80th Birthday!"
Lori Gordon

Mary Cate Zipprich and Aaron Bikel
Barbara Goodwin

Ingrid Zimmerman
Justine O'Brien

Ms. Wheelchair Ohio Has CMT, Advocates for Everyone with a Disability

Krista Allison, Ms. Wheelchair Ohio 2022, has CMT, but she's using her platform to advocate for people with all kinds of disabilities. Since she was crowned on March 12, she has kept up a steady schedule of appearances aimed at making waves in the disabled community.

Krista says it took her years to accept her CMT and get to a place where she could advocate for others. She'd known about the Ms. Wheelchair Ohio organization for many years but didn't apply until last year when she felt like she had fully matured and was in a position to help others.

Toward the end of 2021, Krista saw a post that Ms. Wheelchair Ohio 2022 was accepting applications. She submitted one and waited. On March 12, she competed in a day-long session with workshops, interviews, speeches and on-stage questions. She won and was crowned that day.


Krista Allison

Krista chose community accessibility as her platform to advocate for the entire disabled community. Her crown has given her access to a wide range of opportunities. To list just a few, she has served as Grand Marshall of a Memorial Day parade, met with the chief of the Brunswick, Ohio, police to discuss ways to help disabled citizens, met with the head of Baldwin Wallace University about campus access and attended a training class for puppies for physically disabled individuals.

Born in Cleveland with a spontaneous mutation of CMT4J, Krista showed early signs of CMT, but wasn't fully diagnosed until the age of 34. Her symptoms worsened when she was 21 and pregnant with her daughter: By her sixth month of pregnancy, her CMT had progressed to the point where she needed a wheelchair full-time. She hasn't been able to get out of the wheelchair since then but says that she's a lot stronger today than she was right after giving birth.

In addition to her grueling schedule of public events, Krista puts her bachelor's degree in criminal justice and master's in public administration to use in a full-time job with the federal government.

"My journey has been up and down, but I have never let it stop me from accomplishing great things," Krista said, adding, "Don't ever give up on yourself."

CAMP FOOTPRINT MAGIC RETURNS

With First In-Person Camp in Two Years

Camp Footprint 2022 ended Aug. 19, but the memories will live forever. Youth Council members Season Ciriello and Evan Zeltsar, both 17, share what it was like for 100 campers with CMT to spend a week together in the woods of Pennsylvania after two years online.

As the sun fades over Camp Footprint each night, the cabins still bustle with activity. A group playing cards around the table bursts into laughter; a camper and counselor chat about what brand of shoe fits a leg brace best; and one camper helps another to the bathroom. Bunkmates reflect on the day's adventures together before heading to bed, excited about what the next day will bring.

It's hard to put the magic of Camp Footprint into words. Just one week at camp creates a memorable and life-changing experience. The promise of sharing summer fun with other youth with CMT is what draws campers from around the world—both new and old—to Camp Footprint every August. This year, that promise turned into a week of camp even more amazing than ever before.

For many campers, this was their first time experiencing the magic of in-person Camp Footprint. Some have only ever connected with fellow youth online, through social media or on Zoom calls, but there's nothing quite as bonding as summer camp. Pouring your hearts out during cabin time, sharing a creaky bunk bed, spending the day at the nurse together after challenging yourselves a little too much; these are the moments that bring you closer together. You can't feel that same magic through Instagram DMs.

But Camp Footprint isn't just a friend convention; more importantly, it's the perfect place for self-discovery and reflection. You'll be encouraged to say, "I can go a bit longer!" and be fully supported when you've reached your limits.

CMTA
Charcot-Marie-Tooth Association
CAMP FOOTPRINT
ONE STEP AT A TIME

A PARENT'S PERSPECTIVE:

KARI HOLZGANG SHARES THE CAMP EXPERIENCE OF HER DAUGHTER MELIA, 17, IN A LETTER TO CAMP DIRECTOR JONAH BERGER

Hello Jonah,

My daughter Melia came back this weekend with one of the greatest gifts she has ever received, the gifts of acceptance and (more importantly) self-acceptance. I want to thank you and the other volunteers for providing these special gifts to her.

It was evident that you and the other volunteers put together such a wholesome and well-thought-out week of fun-packed activities filled with reachable goals, opportunities to make friends, live outside her comfort zone (but just enough!), and tears and laughter (a necessary balance!) that it has really changed her life.

Until now, Melia has had no peers who understand what she is going through. Other than myself, our family members with CMT are long gone or spread across the US, so she hasn't been able to really understand the disease or see herself in others.

This camp eluded us for many years. We had many excuses. We felt like traveling across country alone was perhaps too complicated for a younger person. But it was evident from your communication, and sharing of travel plans, and seeing all the wonderful updates from parents, that this was easily surmountable. Also in the earlier years, when we first heard of the camp, the timing was never right for us with the beginning of the school year. And to make things complicated, she was in an accelerated program that we believed would be too hard to miss. Now we realize, it was just the opposite. This whole experience, the independent travel, the week of vacation from her worries, camp life, the integration with people she feels are more like her, THIS is the "accelerated program" she shouldn't have missed, and we regret not joining in sooner.

We appreciate you and your tireless efforts to be inclusive and make everyone feel like they can succeed. The fact that you had so many volunteers giving of a week of their time to entertain kiddos who are not their own is also a tribute to you and the wonderful work you do. Thanks to them for providing guidance, enlightenment, and keeping her safe.

She had a wonderful time (the best), made so many friends, and looks forward to the possibility of returning next year, perhaps as a counselor in training. She looks forward to knowing her new CMT family better.

A big hug and thank you forever,

KARI HOLZGANG

WHY SHE GAVE: ALICE REW

We've all heard stories about quiet people working quiet jobs who somehow manage to save a fortune—and then leave it to a worthy cause. Thanks to the hard work and generosity of librarian Alice Rew, who died in May, the CMTA is the grateful recipient of a seven-figure gift.

Alice was born in Atascadero, Calif., in 1938 and lived there her entire life. She was diagnosed with CMT at the age of 3, but it didn't stop her from leading a full, productive life. She was the head librarian for the Atascadero High School for 35 years. She was active in the Atascadero Historical Society for many years and served on the board of the Friends of the Library.

Alice loved books and reading, which led directly to her profession. One day she escaped the mess of a home renovation by going to the library, where they put her to work. Although she didn't have a library degree, the principal offered her a job at the school library on the condition that she get one. She did it in three summers at San Jose State.

Alice first brought technology to the school library. In 1980, she took her own computer to work and used it for recordkeeping. Soon afterward, the school district established computer labs in the classroom.

When she wasn't reading or teaching, Alice loved to cook, sew, quilt and crochet. She also found time for a CMT support group. Of that group, she said, "It makes life easier to have someone to talk to who understands what you're going through."

Alice's friend Sharon Turner recalls that when Alice shared her plans to donate to the CMTA two years ago, she was very happy and a small smile played across her face. As Sharon observed, her legacy will live on in many places, including the CMT community.

Jeana Sweeny, the CMTA's chief engagement and gifts officer, fondly recalled Alice's long-standing commitment to the CMTA: "Every year since 1994, Alice would send a gift to the CMTA to ensure that she received *The CMTA Report* in the mail. With each report, Alice read about the impressive growth of CMTA-STAR research and stories about others living with CMT. She recognized that she could help by leaving a gift to the organization leading CMT research. I feel very fortunate that I was able to thank Alice personally for choosing to leave behind a legacy for the CMTA," Sweeney added.

If you'd like more information about leaving a legacy for the CMTA,contact Jeana at Jeana@cmtausa or 800-606-2682 x106

WHAT'S ON YOUR MIND? ASK DAVID.

David Tannenbaum answers questions from readers in his column "What's On Your Mind? Ask David" regularly in The CMTA Report. *David 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. "My CMT has been my greatest challenge and my best teacher in life," says David.*

Dear David,

I am a 21-year-old woman who was diagnosed with CMT1A a year ago. My father and brother have CMT, so I was not very surprised, but I was still upset. My dad and brother seem to have very mild cases but mine is more severe. I am living at home to save money while attending community college and working part-time. I've been really depressed since my diagnosis and when I tell my dad that I am tired much of the time, he shrugs it off and tells me I'm being too dramatic. I may be a lot of things but being dramatic is not one of them. In fact, I'm generally very quiet and most people have no idea how I'm feeling. I would think that since Dad has CMT himself he would be more understanding and compassionate, but he's not. I have a few girlfriends I can talk to but they have their own lives and not much time to listen to me. My depression is getting the best of me. Help!

David Replies:

Thanks for having the courage to reach out for help—it's a crucial step in your CMT journey. I'm guessing that your dad is treating your feelings about CMT in the same way he deals with his own feelings, which is to deny them. His rigidity with his own emotional expression doesn't mean you have to follow in his footsteps. Over the years I've encountered members of the same family who never reach out for support to one another. They live in isolation, experiencing their pain privately and missing a powerful opportunity to support each other's need for connection and intimacy. Connecting with each other's shared sadness can be a tender and loving moment. Members of the same family can experience their CMT symptoms very differently. Your dad needs to understand this and stop judging your "complaining." Perhaps he has found his own way of dealing with CMT that works for him, but you need to find your own unique path.

Depression can definitely cause fatigue and affect energy levels, but let's try to get a handle on your overwhelming feelings before you attribute all your tiredness to your CMT. If I've learned anything it's that my anxiety or occasional depression will go directly to my muscles. Often our depression is a wake-up call that screams "I don't want to live this way any longer" and pushes us to find different ways to deal with our illness.

Suffering can either lead to more suffering or it can lead to an awakening. A big part of our awakening is the realization that we are so much more than our limitations. We need the courage to be honest with ourselves and experience the depths of our emotional pain. Accepting what is—and acknowledging the truth of what we feel—allows the feelings to fade away gradually. The phrase "What we resist persists" is true. Open up to everything you are experiencing without self-judgment and claim the right to be your true self regardless of whether your family labels you "dramatic." In making space for all our feelings we begin to realize that we are more than our feelings. We are actually the witness to these feeling states that come and go like passing clouds in the sky. Identify with the sky, not the clouds.

I would also urge you to find a Center of Excellence near you to be fully evaluated so you have a better sense of what is going on with your symptoms. CMT manifests in many ways, and it helps to be seen by CMT specialists before you get carried away by your fears.

Depression is a normal response to many chronic conditions, but it doesn't have to take over your life. A good therapist can give you tools to manage it. You are so much more than your CMT, but depression can prevent us from seeing our true potential. Depression can cause us to feel helpless and take away any motivation to change. I strongly urge you to speak to a counselor who understands chronic illness and can help you navigate through all your feelings to see that you have a right to feel what you feel without judgment or criticism, either your own or someone else's. In time you will experience a bit of sunlight

continued on page 20

Long-Time Drummer for The Outsiders Adapts and Keeps Playing, Despite CMT

Drumming requires the coordination of both fine and gross motor skills and the ability to automate movements. What does a drummer do when CMT impacts his playing? "Adapt," says Rick Biagiola, long-time drummer for Cleveland rock band The Outsiders, best known for the hit "Time Won't Let Me" from 1966.

Over the years, Rick, with the help of his wife Lois, has done just that, finding workarounds that allow him to keep playing. He's changed the way he holds the drumsticks due to hand weakness and wears football receiver gloves that aid with grip. He uses a double pedal because of his foot drop and restricted ankle movement, allowing him to do with two pedals what he used to do with one.

He also taught Lois how to help him set up and adjust his drums and says they have it down to a science, greatly reducing set-up time. While Rick finds it frustrating that CMT has diminished his skills, he maintains a positive attitude, saying, "The fact that you are living with this condition and dealing with it makes you stronger than people know."

Born to a barber father and a mother who put herself through school at the age of 50 to become a teacher, Rick was interested in music from a very young age. His father, who passed away when Rick was 12, was a talented accordionist. As a preteen, watching "American Bandstand," "Ozzie and Harriet" and all the variety shows with big band orchestras inspired Rick to take up the drums. He joined his first band at the age of 13. At 15, he was playing in bars and at teen dances.

Rick was asked to join The Outsiders when he was 17 and drummed for them under the name Ricky Baker. "It was a great experience while it lasted, but eventually, I wanted to come back home," he says. He returned to Cleveland, where he finished school and continued to play through the years, making a very good living at it for a while. He also worked in construction and became an electrician. He's retired now, but still plays with a local band and reformed The Outsiders as a national performing act.


Rick Biagiola

Rick's CMT symptoms didn't begin to appear until around 1989, when he was 41. He recalls: "I was still playing softball and working for the electric company. My ankles would turn while I was performing my job as a meter reader/residential service installer, which also involved climbing poles. That was a tough time. I didn't tell my employer what was going on with me because I was afraid of losing my job. Eventually, I had to retire from the electric company, because the stress of the way I was treated by the union was too great. I didn't realize that at the time I had rights because of the Americans with Disabilities Act. It was a lower-than-low time for me. My wife, Lois, and my faith kept me going, because there were days I didn't think I could take much more."

Dr. Michael Shy from the University of Iowa Center of Excellence diagnosed him with CMT2F in 2016. Rick hasn't been able to find any family history of the disease, either in the United States or in Italy, where his grandparents were from.

Lois and Rick have been married for 41 years and have three adult children and four grandchildren. He used to worry that they might inherit his CMT, but they have all assured him that they will watch for symptoms and deal with whatever comes.

Rick stays healthy and active while he waits and hopes for a clinical trial for his type. Aside from playing the drums and performing, he exercises with weight machines, walks on the treadmill and swims. "Movement is key," he says.

Asked what advice he'd given his fellow CMT patients, Rick was quick to respond: "Get educated and find support, to begin with. Find ways to adjust and compensate, be creative or find someone that can come up with 'outside the box' ideas. Use whatever devices you have at your disposal, whether it's braces, utensils, canes, walkers, orthotics or wheelchairs. Don't let your pride stop you from independence. Accept help and be proud of what you can accomplish.

"Have a sense of humor, and especially have faith," he continued: "You have to develop mental toughness, because, as we fellow CMTers know there is a high frustration level. I had to choose if I was going to give up or decide that I have to work harder to have as normal a life as possible. There are days that I feel down, but the truth is, every day is a blessing."

BRANCH NOTES

ANCHORAGE, AK

The Alaska CMTA Branch met July 23 for an in-person meet and greet. Members shared their CMT journeys and local resources, as well as information about the community programs and resources that the CMTA offers.

JACKSONVILLE, FL

The Jacksonville, FL Branch welcomed guest speaker Mackenzie Meyer from Bremer Brace to a virtual meeting June 7. Mackenzie shared valuable information on treatment goals for bracing, treatment options, who's a candidate, what to expect at a visit and much more. The branch also welcomed newcomers and planned for future meetings and fundraisers.

NAPLES, FL

Seven people attended the Naples, FL branch meeting April 13, all thrilled to be gathering safely in person. Attendees introduced themselves and told their CMT stories. They also shared local resources and discussed the diagnostic journey for new members who needed information on next steps. The branch has decided to switch off between virtual and in-person meetings.

ATLANTA, GA

Fifteen members of the Atlanta Branch met in person on July 23 to hear an informative presentation from David Keane of the genetic testing company Variant. Attendees learned how fast the genetic testing world is advancing and how best to go about getting genetic testing for CMT. Branch leader Jeannie Zibrida shared CMTA updates and talked about the Atlanta Walk 4 CMT set for Oct. 8.

CHICAGO, IL

The Chicago Branch welcomed guest speaker Jeff Seitzer to its May 25 meeting. Jeff, who has a new memoir coming out, shared a bit about his life with CMT, orthotics and how social media and the internet have helped with learning about CMT and meeting others with it. To learn more about Jeff's new book, "The Fun Master," check out jeffreyseitzer.com.

NEW MEXICO

The New Mexico CMTA Branch met virtually on May 7 with nine members in attendance. Guest speaker Dr. Mark Hardison from the University of New Mexico gave an excellent presentation on the science and practice of mindfulness meditation for pain management. He also led members in a 20-minute "body scan" meditation that the group found extremely valuable.

BOSTON, MA


Dr. Reza

Thirteen members of the Boston Branch gathered virtually on July 12 to hear a presentation by Dr. Reza Seyedsadjadi, director of the CMT Center of Excellence director at Massachusetts General Hospital. Dr. Reza shared a PowerPoint presentation with highlights from the recent Peripheral Nerve Society meeting and talked about 12 CMT research projects taking place worldwide. Noting that half of his center's 600 patients still lack a genetic diagnosis, he encouraged listeners who don't have a diagnosis to get re-tested because of recent advances in testing. Dr. Reza thanked members of the Boston Branch for their willingness to get involved in clinical trials for CMT, saying, "It takes great effort on both sides to move forward in science/research, and for new discoveries you need the patients."


Atlanta, GA

GUADALAJARA, MEXICO

The newly formed Guadalajara, Mexico Branch—led by Tomas Luis Lopez Valenzuela and Guadalupe Valenzuela Cazares—held its first branch meeting virtually on July 23 with 12 members in attendance. Guest speaker David Werner, who is bilingual and has been instrumental in helping to get the branch up and running, shared his CMT journey. Attendees introduced themselves and talked about their own CMT journeys and shared CMTA updates and resources–including the newly published "What is CMT?" guide in Spanish. (www.cmtausa.org/cmt-en-espanol)

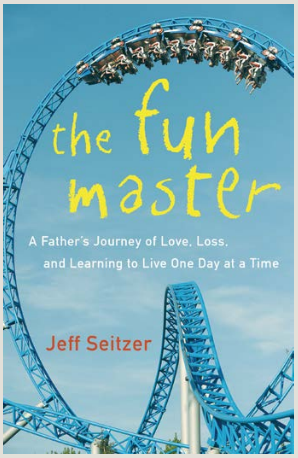
WASHINGTON, DC

The Washington DC Area Branch welcomed guest speaker John Svaren, PhD, to its virtual meeting April 20. John chairs the CMTA-STAR Advisory Board, is a professor in the Department of Comparative Biosciences at the University of Wisconsin (UW) and serves as director of the Cellular and Molecular Neuroscience core at the UW Waisman Center, where his laboratory is located. John shared CMT research highlights, then took questions afterward.

MANITOWOC, WI

Eight members of the Manitowoc CMTA Branch met in April to tell stories of staying positive on their CMT journeys. Members shared valuable suggestions on how to cope with the physical/mental/emotional challenges of CMT, as well as their success stories.

NOW IN PRINT!



We're happy to announce the publication of Jeff Seitzer's memoir, The Fun Master, which was excerpted in the Spring 2022 issue of *The CMTA Report*. Jeff was a self-admitted, "self-involved academic" struggling to cope with CMTX and the residual effects of encephalitis when he took over the care of his son Ethan, who was born in fragile health. Ethan thrived despite multiple surgeries, hospitalizations, serious breathing and swallowing problems, hearing loss, and a challenging social environment. Ethan's instinct for fun proved the perfect complement to Jeff's determination to live life fully. Ethan drowned in August 2010 at age 9, though not before he, his sister Penelope and their mother Janet taught Jeff to find happiness in putting other people's needs before his own—and living in the moment rather than trying to control it.

Find links to booksellers that carry *The Fun Master* at gosparkpress.com/product/the-fun-master/.

DEAR DAVID

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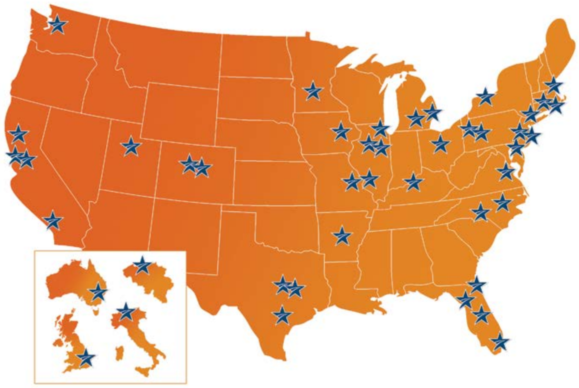
in the darkness and the energy will begin to move, allowing you to see your situation with softer eyes.

Lastly, I often urge younger CMTers to find something to get involved with that gives them purpose. Finding a reason to get up in the morning means everything. You have a healthy brain that will help you eventually find that meaning in life that will move CMT from the foreground to the background of your life. Reach out to the Youth Forum of the CMTA where you will meet others like yourself who know exactly what you are going through. You are truly not alone. I only wish that at 21 I knew what I know now—that even with physical challenges life can be rich and rewarding.

CMTA CENTERS OF EXCELLENCE

CMTA CENTERS OF EXCELLENCE roughly correspond to the 21 international sites that make up the NIH Inherited Neuropathy Consortium (INC)—a group of academic medical centers, patient support organizations and clinical research resources sponsored in part by the CMTA. The centers are becoming even more important as the CMTA begins clinical trials, which 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.



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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)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddl, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Eribulin (Halaven)
Fluoroquinolones
Gold salts
Ipilimumab (Yervoy)
Ixabepilone (Ixempra)
Lefluonamide (Arava)
Lenalidomide (Revlimid)
Metronidazole/Misonidazole (extended use)
Nitrofurantoin (Macrodantin, Furadantin, Macrobid)
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

The medications listed above are potentially toxic to CMT patients. Vincristine has been proven hazardous and should be avoided by all CMT patients, including those with no symptoms. Taxols also pose a high risk to people with CMT. The remainder of the medications listed above present varying degrees of potential risk for worsening CMT neuropathy. Before taking any medication or changing medication, all CMT patients should make sure the treating physician is fully aware of their medical condition.

WHAT IS **CMT?**

More than 3 million people worldwide have CMT. It 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.

People 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 (www.nsgc.org) or your physician for more information.

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-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.