SUMMER 2022

THE CMTA REPORT

www.cmtausa.org

MEET YOUR CMTA "PIT CREW"

LOOKING FORWARD TO CAMP FOOTPRINT

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THE CMTA REPORT | SUMMER 2022

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Charcot-Marie-Tooth Association

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Charcot-Marie-Tooth Association

Dear Friends,

A MESSAGE FROM THE CEO

When retired BlackRock portfolio manager Dan Chamby first considered joining the CMTA Board of Directors, he was struck by the engagement and energy of everyone in the organization. The difference between mediocre organizations and great ones, Dan said, is that the latter have energy and enthusiasm and a strong sense of mission and collaboration. Dan saw that sense of mission and collaboration in the CMTA, which he compared to BlackRock for its ability to “swarm” a problem, with everyone putting everything they have into solving it.

In this issue, we bring you profiles of the energetic and enthusiastic members of the CMTA staff. Our team brings a wealth of education and experience to the organization, operating as an extraordinarily efficient “pit crew” to enable the CMTA to keep delivering the cutting-edge research and community services that are our hallmark.

We hope that the article will give you a greater understanding of every staffer’s responsibilities and how they work together. At the very least, you should come away knowing who to contact with questions on any given subject.

Also in this issue, we report on two of the CMTA’s latest research projects—a recently approved biomarkers project for Type 2F and Dr. Stephan Zuchner’s “Distant Cousins Project” at the University of Miami, which is trying to identify the “culprit,” or causative, genes for heretofore unidentified Type 2 cases.

Research and personal experience come together in an article that links one man’s genes, genetics and genealogy in a fascinating CMT detective story that goes back four generations.

Finally, because it’s summer, we bring you David Tannenbaum’s sage advice on traveling with a companion who isn’t considerate of their travel partner’s CMT. And since fall will quickly follow, we bring you two school stories for parents whose kids have CMT—one dealing with 504 and IEP support plans and the other with teaching students about rare diseases.

It’s an issue full of information and inspiration, and I hope you’ll learn and enjoy.

With warm regards,

Amy Gray

Amy Gray
CMTA Chief Executive Officer

Finding Your Culprit Gene:

THE DISTANT COUSIN PROJECT

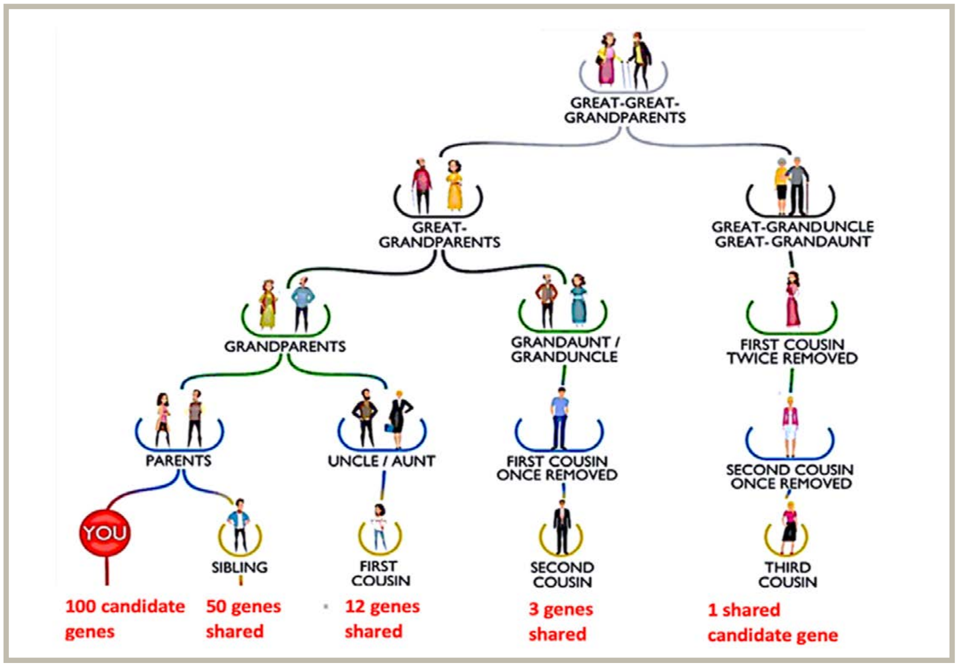
Effective treatments for genetically caused rare diseases are within sight, making the effort to identify genes that cause rare diseases more important than ever.

Unfortunately, more than half of individuals diagnosed with Type 2 CMT have not had their “culprit,” or causative, gene identified, and it’s impossible to fix a gene or treat a genetic disease without it. The CMTA’s Distant Cousin Project, supported by a generous donor who lives with an unknown Type 2, is working to speed up the rate at which Type 2 CMT culprit genes are identified.

Dr. Stephan Zuchner’s team at the University of Miami is spearheading the effort. The researchers recently identified a heretofore unknown CMT gene, along with a potential cure for the variant. Almost overnight, patients with this culprit gene went from not knowing what gene caused their disease to knowing that there is a potential treatment.

It might seem like sequencing one’s genome along with the genomes of family members with the same disease would lead to the culprit. To some extent, that’s true. The problem is that researchers can readily identify hundreds of potential culprit genes (variations of unknown significance), and everyone shares too much DNA with close relatives to narrow the genes down to just a few possibilities. However, by locating a distant cousin with the same causal gene, the number of potential culprits can be reduced to one or two. For example, fourth cousins share about only about one-fifth of 1 percent of their DNA. If two fourth cousins share the same culprit gene, there is a very good chance that researchers could locate it.

Dr. Stephan Zuchner



COUSIN GRAPH ILLUSTRATING THE POWER OF FAMILY RELATIONSHIPS TO IDENTIFY CMT GENES

The Distant Cousin Project will pay for sequencing the first 10 eligible families with an unidentified form of CMT. The genomes will be sequenced as part of Dr. Zuchner’s research studies and analyzed by the Genesis Project database, an all-volunteer, scientist-managed academic foundation dedicated to fostering genetic discovery.

The success of the study will depend on finding CMT patients with an unidentified culprit gene. Eligible families include those:

- Whose cause of CMT is unclear despite genetic testing.
- Where one parent has CMT.
- Where there is a distant cousin, or another distant relative on that parent’s side, who also has an unidentified form of CMT. This is the most difficult part of the challenge and will require patients’ creativity and determination.
- Who are willing to have a specialized physician at a CMTA Center of Excellence confirm the CMT diagnosis, if they have not done so already.

Anyone who thinks they may qualify for this study should contact study coordinator Lisa Abreu directly at l.abreu@med.miami.edu. She will obtain consent from the patient and distant family members via phone or Zoom, ask for a pedigree and organize a blood draw, either at their doctor’s office or at home.

GENES, GENETICS AND GENEALOGY:

THREE ROAD MAPS FOR CMT

BY BRUCE HARRIS-LANGLOIS

Each of us has our own CMT story.

Mine started at the age of 54 when I was walking in my neighborhood and it suddenly struck me that my gait—a high step followed by a slap on the ground and swinging my foot somewhat to the outside—was exactly like my mother's. Later I began to notice the aching in my legs, the sprained ankles, the numbness and tingling in my feet, my very high arches and the odd wear pattern on the soles of my shoes.

"I have CMT," I told my doctor in 2001.

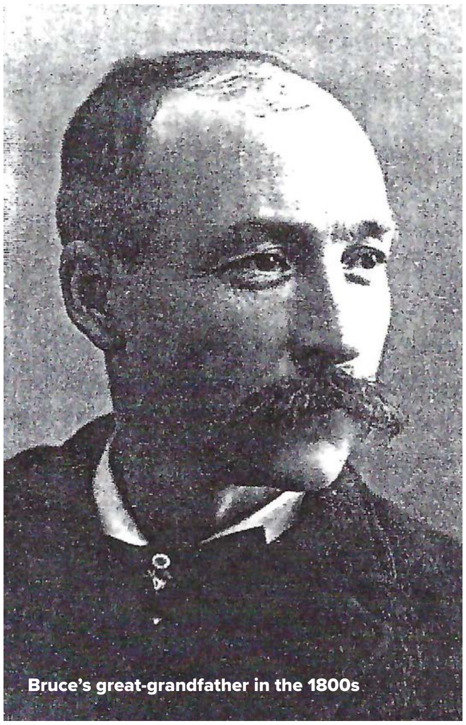
"What's that?" he asked.

"It's genetic, but I don't know the name of the gene," I replied.

When my mother was a young woman, my grandmother told her that her feet looked like those of my great-grandfather, a carpenter and joiner who lived from 1828 to 1893. People called him a "cripple." In 1871 his daughter wrote in a letter to one of her nine siblings, "[F]ather falls every day... he grew old very fast."

About that time, my great-grandfather wrote he could no longer do his job. "I have not been able to do any work for five years. I have not dressed nor undressed in over three years without someone to help me [sic] my hands and arms are drawn out of shape [sic] that I cannot feed myself.... Some days I can walk around considerable, again I could not turn round to save my life for two or three weeks."

As a child in the 1950s, I remember my grandmother being feeble, walking with a cane and wearing funny-looking shoes. As my mother reached middle age she stumbled and fell a lot and her hands got gnarly and painful. She thought her symptoms were a holdover from suspected polio as a teenager.


Bruce's great-grandfather in the 1800s

My mother was clinically diagnosed with CMT Type 2 in 1993 before genetic testing was well developed for Type 2 subtypes. Even now fewer than 50 percent of people with Type 2 know their subtype, a problem the CMTA is working on solving. It seemed to me like an unbroken autosomal dominant genetic line from my great-grandfather to my grandmother to my mother to me. But I wanted it confirmed by a clinical diagnosis plus genetic testing.

I had taken several genetic tests at Dr. Michael Shy's CMT Clinic at Wayne State University in Detroit, but my gene had not yet been discovered. Then, in 2017, Dr. Richard Lewis at the CMT Clinic at Cedars-Sinai in Los Angeles submitted a blood sample to GeneDx for testing, which identified a variant on the MME gene. This was the likely cause of autosomal dominant CMT Type 2T, characterized by late-onset axonal CMT, usually starting around age 50. Finally, I knew—2T.

Four generations of my family have had CMT symptoms consistent with the MME gene and Type 2T. In a few decades, we progressed from anecdotes in family letters to discovering a new subtype, thanks in part to the CMTA's Strategy to Accelerate Research (STAR).

Could our genealogy tell us even more? My mother had researched genealogy for her and my father's families for years, but she wasn't looking for hereditary diseases. While autosomal dominant and recessive inheritance patterns remain something of a mystery to me, I wondered if I could understand them better through the lineage of grandparents, aunts, uncles and cousins.

My mother's ancestors were northern Europeans, with names like Harris, Bronson and Burke. But my father's family was almost entirely French, with names like Langlois, LaFlamme and Doucet. My grandmother was a Langlois, born in 1888. Her father was French Canadian, born in 1843, and his parents were born in 1798 and 1805, all in Quebec. Another generation back, her parents Daniel and Marguerite were second cousins once removed, third cousins once removed, half third cousins, fourth cousins once removed, half fifth cousins and half sixth cousins—more complicated than an L.A. freeway interchange.

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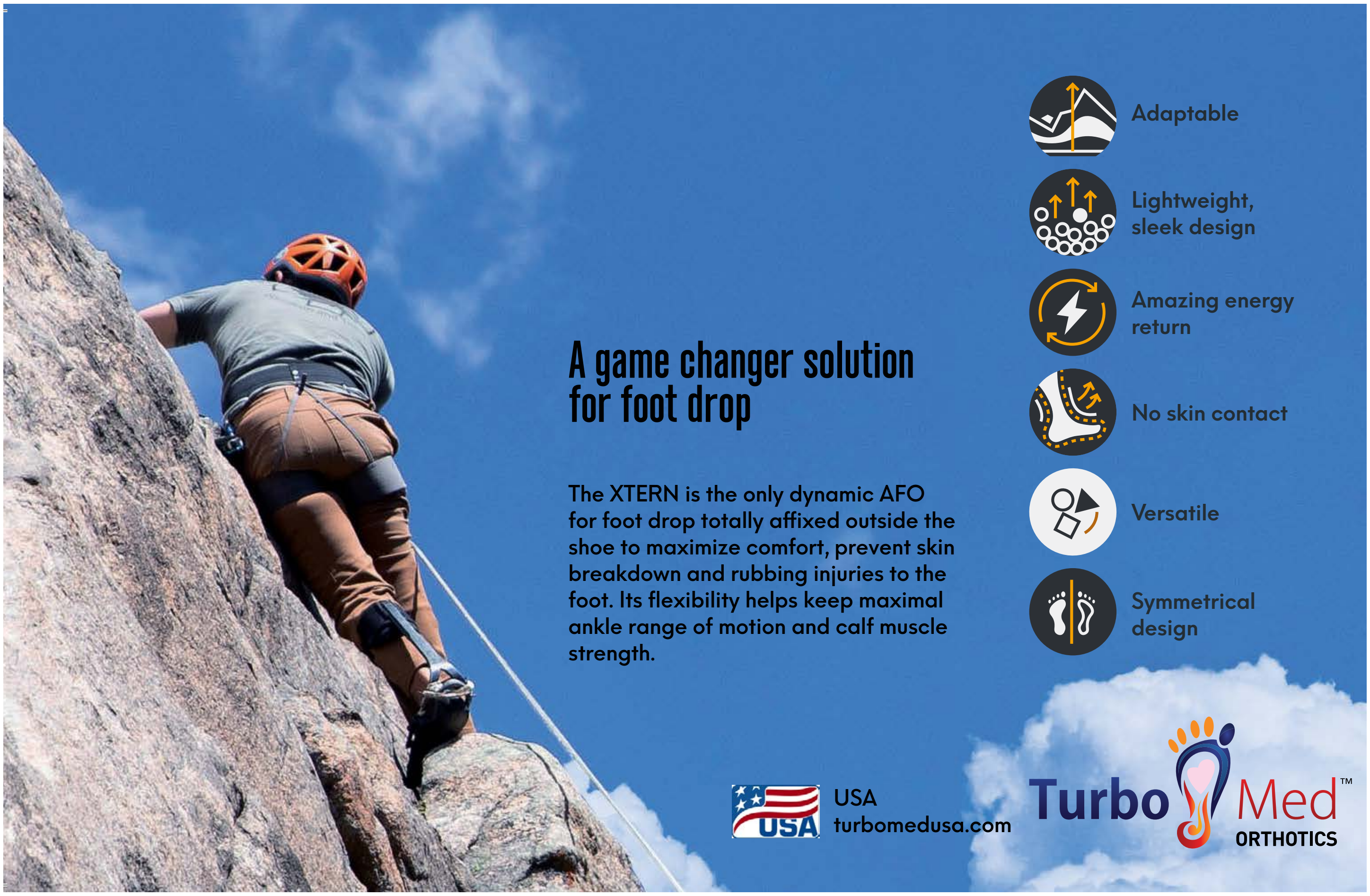
Bruce and his grandmother in the 1950s



Registration Now Open for 9th Annual Cycle 4 CMT

Whether you are a cyclist, walker, supporter or sponsor, this event is for everyone!

CMTA's Signature In-Person Cycle 4 CMT Event

Sunday, August 28, 2022 • 8 am – 3 pm
The Old Lantern Inn and Barn,
Charlotte, Vermont

Register or Donate today: cycle4cmt.com

Can't make it to Vermont in August? No problem! Participate in one of our nationwide virtual rides!

For more details, go to cycle4cmt.com or email CMTA board member Elizabeth Ouellette at elizabetho@outlook.com

The in-person Vermont event includes a fun after-party on the scenic grounds of the Old Lantern Inn and Barn with silent auction, live music, fully catered meal, Vermont-brewed beer, cider or non-alcoholic beverages. Come and meet CMTA staff, community members and CMT experts.

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These people are known as the Acadians. In the 1600s, Acadia was located in what is now New Brunswick, Nova Scotia and Prince Edward Island. Around 1755 many Acadians were forced to move to England, France, Quebec and the American colonies, in particular Louisiana. (The word Cajun is derived from Acadian.) Acadians were a closely knit people and kept to themselves in small communities, which led to a higher than normal frequency of genetic disorders. CMT is one of the so-called Acadian diseases. I wondered if my genealogy could help explain where my CMT came from.

At the CMT Clinic at Cedars-Sinai, I met with Licensed Certified Genetic Counselor Tara Jones, MS. I asked whether I could have inherited a recessive genetic form of CMT from my father's side in addition to the autosomal dominant MME gene from my mother's side. Finding out if I am a carrier of the recessive gene would be valuable knowledge for my children and grandchildren, especially since my children are approaching 50, the age when my mother and I were noticeably affected by CMT. The genetic pattern would give my children a starting point in diagnosing any infirmities that suggest CMT.

Bruce's mom in the 1970s

Tara Jones advised me that the likelihood of having a recessive Acadian gene was slight for three reasons. First, the fact that CMT is seen more often in the Acadian genetic line does not mean that all Acadian descendants have CMT.

Second, I am not aware of any French-Canadian grandparents, great grandparents, aunts, uncles or cousins who experienced CMT symptoms. We don't know or suspect that any of them had CMT, and neither of my two living cousins on that side can recall any foot, gait, walking, tripping or falling issues in their families.

Finally, though MME can cause CMT with both autosomal dominant and autosomal recessive inheritance patterns, Tara confirmed that my specific genetic change has only been reported to have autosomal dominant inheritance, which follows my maternal lineage of a relative with CMT in every generation.

My gene is MME, the genetics are still beyond my comprehension and my genealogy is a road map in the rearview mirror. I will keep going to the CMT Clinic at Cedars-Sinai because the best chance for a world without CMT is more STAR-funded research, more clinical trials, more CMTA patient and family support, more patients going to the CMTA's four dozen Centers of Excellence and more genetic testing and interpretation (www.cedars-sinai.org/programs/neurology-neurosurgery/clinical/nerve-muscle-als/experts.html).

Looking forward to the road ahead, I want CMT to stop with me.

Bruce Harris-Langlois, 75, lives in California, He uses his two grandmothers' names here to protect the privacy of his family members.

BOARD OF DIRECTORS APPROVES BIOMARKERS PROJECT FOR CMT2F

On April 19, the CMTA Board of Directors approved a $292,099, two-year project for the development of biomarkers to be used in clinical trials for CMT2F. The approval moves the research one step closer to clinical trial readiness.

CMT2F, an axonal neuropathy, is caused by mutations in the HSPB1 gene. It affects up to 4 percent of individuals with CMT.

Several candidate therapies have been proposed for 2F, using either small molecules or therapeutic genome editing employing CRISPR-Cas9. Because CMT typically progresses slowly and does not shorten lifespans, sensitive biomarkers are needed that can show that a therapy is reaching its target within a single year. While participants in the Inherited Neuropathy Consortium follow several patients with CMT2F, the disease biomarkers critical to clinical trials for 2F are lacking.

Principal investigators Bruce Conklin, MD, and Luke Judge, MD, PhD, of the Gladstone Institutes and UCSF Departments of Medicine and Pediatrics will be using a state-of-the-art approach to CMT2F biomarkers like that used for CMT1A with promising preliminary results. That approach was also recently approved for 1X, 1B and 2A. They will be measuring progression in 25 patients with CMT2F, using a variety of outcome measures—plasma biomarkers, including NFL, calf muscle MRIs and a nanostring platform for skin biopsies.

The researchers will be extending existing biomarker/outcome studies to CMT2F patients at Dr. Michael Shy's clinic at the University of Iowa and additional sites within the Inherited Neuropathy Consortium, including leading CMT clinics in London (Dr. Mary Reilly), Milan (Dr. Davide Pareyson), the University of Rochester (Dr. David Herrmann) and the University of Pennsylvania (Dr. Steven Scherer).

MEET THE CMTA "PIT CREW"


Intrepid CEO Amy Gray

In Formula One racing, a good pit crew can propel a car to victory. What makes a good pit crew?

Mark Gallagher, who worked in senior leadership roles in Formula One racing for 30 years, says the "pitstop mindset" requires that all crew members know their roles, unite around a common goal and continually work to improve performance. Those basics allow the pit crew to "swarm" the race car and fix any problem in just minutes.

According to Gallagher, Formula One racing is a good example of what happens when you equip a team of ambitious, hardworking people with the tools they need to succeed.

The CMTA staff has all the elements of a good pit crew. Each of the seven full-time staffers knows their roles and how to perform them, unites around the goal of a world without CMT and continually tries to do their jobs better. It's a remarkable team and we thought our readers should know more about them and the important roles they play at the CMTA.

AMY GRAY

Chief Executive Officer

Amy is the CMTA's pit crew chief—the one who calls the shots and sets CMT research and community-building up for success. A good pit crew must be knowledgeable about every aspect of operations, whether that knowledge is gleaned from education or experience. Amy has both: After obtaining a bachelor's degree in marketing from St. Cloud State University in Minnesota, she went on to increasingly responsible roles in four different nonprofits—the Muscular Dystrophy Association, where she first met and worked with CMT families, the Crohn's and Colitis Foundation, the Parkinson's Foundation and now the CMTA.

Amy joined the CMTA as CEO in 2017, a decade after the CMTA implemented its "Strategy to Accelerate Research" (STAR). She brought much-needed expertise in fundraising, staff development, financial and operational management, strategic planning and community engagement.

During her tenure, Amy has led the CMTA staff, board, advisers, investigators, pharmaceutical companies and community members in building a foundation to fund more research and attract more companies to the field of CMT. Under her leadership, the organization expanded STAR and its Centers of Excellence network, launched Patients as Partners in Research and completed a rebranding effort and the launch of a new website. Those changes earned the CMTA its third 4-star rating from Charity Navigator with a "Perfect 100" score, indicating that its finance, accountability and transparency are all top-notch. The CMTA is the only organization in the CMT space to be so honored. Of 10,000 rated organizations, only 60 have received that perfect score.

Amy has also worked with the staff and board, branch leaders and advisors, campers and counselors, to grow the CMTA's community outreach programs, including a full array of services for youth with CMT. "The CMTA community is so incredible, and it has been like an extended family in many ways," she says.


Amy at Camp Footprint

Outside of work, Amy enjoys the warm Florida winters after a lifetime spent in Minnesota, which allow her to run and rollerblade outdoors year-round. An adventurous soul, she skydived once for charity. She loves dogs and has two golden doodles—Chewy and Solo, named by her sons, who are Star Wars fans. We think the Force is with her.

Rylee, Jeana, Chris and Haylee Sweeney

JEANA SWEENEY

Chief Engagement and Gifts Officer

Continuing the pit crew analogy, Jeana Sweeney, chief engagement and gifts officer, is the “fueler.” She’s in charge of raising the funds that keep the racecar racing, fitting since she’s something of a speed demon.

Born in Reading, Pa., Jeana began her career as a credit union teller at 17, rising quickly to positions as a loan officer, a branch manager and a business developer. In her first year as a business developer, she tripled the credit union’s commercial accounts.

Jeana’s spontaneous case of CMT1A was diagnosed when she was 15, after many falls and broken bones. A triple arthrodesis was “life-changing” she says, and for the first time in many years, she stopped spraining her ankle and was able to walk without looking down at her feet and falling.

Afterward, though, she was continually told that she couldn’t do things—couldn’t exercise, couldn’t work, couldn’t have kids. She refused to accept the “couldn’ts.”

For years, Jeana thought she was the only person in Johnstown, Pa., with CMT. Then one day she received a flyer announcing that a local support group was forming. “I almost couldn’t believe it,” Jeana says now. “I was so scared to go because I was unsure what to expect—I had never met anyone else with CMT. After that first meeting, branch leader J.D. Griffith took me aside and asked me to get more involved. He said he saw something special in me…. How could I say no to that?”

That first meeting was 20 years ago and Jeana has been creating awareness and raising research funds ever since. When she started at the CMTA, there were only three other staff members. But she saw a lot of opportunities for growth, creativity and possibility. And each new member who joined the staff brought big ideas and “tons of passion” to the mission of a world without CMT.

Today, Jeana says, “My experience has been nothing short of amazing… from the start of the CMTA launching our first-ever CMT Awareness Week (now month!) to creating Camp Footprint.” She’s also been impressed watching the research go from one project for 1A to a growing research platform with the potential to treat most CMT types.

“Sometimes I wish every person living with CMT could see the incredible dedication and hard work each staff and board member puts in for our cause. I wish they could hear how much the STAR researchers want a cure for them,” she says.

Ultimately, Jeana says, her goal is to work herself out of a job by helping bring a cure to everyone with CMT. In the meantime, Jeana enjoys spending time with Chris, her husband of 24 years, and their two daughters, Hayley, 21, and Rylee, 16. (Rylee has a confirmed diagnosis of CMT.) Jeana also likes river fishing for small-mouth bass, riding her bike and rooting for the Steelers.

Laurel Richardson at Virtual Camp Footprint

LAUREL RICHARDSON

Director of Community Outreach

Laurel Richardson, director of community outreach, says the CMTA has been the most rewarding job experience she’s ever had: “The role combines my passion for advancing research with the desire to improve the quality of life for those living with CMT.”

That passion is evident in everything Laurel does—and it’s a lot. She’s responsible for the national branch system, the CMT Patient & Research Summits, the Centers of Excellence and the CMTA’s educational programming and materials.

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MEET THE CMTA "PIT CREW"

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Laurel's education and experience all came together in this job. After earning a degree in journalism with an advertising emphasis from San Diego State University, she worked as a media account executive for Cox Media and WECT and as a client relations manager/regional media buyer for Right Point Media.

Between those jobs, Laurel was a stay-at-home mom for 10 years to Sophie, now 19, and Jack, now 17.

Laurel says she first got involved with the CMTA when she could no longer pretend that she didn't have CMT. In her mid-30s, she developed drop-foot and balance issues and began falling regularly.

Wanting to be part of the solution, Laurel reached out to Jeana Sweeney to learn about branch meetings in San Diego. Jeana responded, "We currently don't have a branch leader for San Diego, would you like to lead it?"

She accepted. After her husband retired from the Marine Corps and they moved to Wilmington, N.C., she started a branch there. Several years later, she was invited to join the CMTA staff, and she'll celebrate her fourth anniversary with the organization in June.

In the past four years, Laurel says she has seen the organization grow STAR and bring more community members into the CMTA family through education and connection opportunities, and by offering more and better ways to fundraise. "It's incredibly rewarding to be part of such a hard-working, passionate team," she adds.

When she's not turning passion into action, Laurel loves to travel, cook and eat. She founded a Girls on the Run chapter at her daughter's elementary school and helped coach the team even though she wasn't a runner. She says that if she didn't work at the CMTA, she would be a dude ranch wrangler in Wyoming.

KIM MAGEE

Director of Finance and Administration

Kim Magee, the CMTA's Director of Finance and Administration, is the CMTA's longest-serving employee. The big-hearted accountant joined what was then a three-person organization in 2007 after her previous job for an eviction company proved too heartbreaking. She decided to look for a job that allowed her to give back to the world.

Kim Magee

Kim found the CMTA the old-fashioned way: She saw an ad for a bookkeeper in the newspaper. The office was on her mailman husband's route and he encouraged her to apply, a decision that she calls the best thing she ever did career-wise. "I work with the greatest people and am part of an organization that makes such a difference in people's lives," she says.

A master of all things financial, Kim handles donations, grants and accounting. She earned an associate's degree in accounting from Keystone Business School and a bachelor's degree in accounting from Neumann University. She earned her degree as a Certified Non-Profit Accounting Professional (CNAP) in 2013 and an advanced certificate in 2019.

Though the CMTA has only seven staff members, Kim says, "The work that we do with the help of the board, consultants and volunteers is on a 'big time' level." She remembers when renowned CMT specialist Dr. Michael Shy began using a machine that could test 100 candidate molecules at a time and it seemed like a big deal. "Now we're using gene therapy and CRISPR. It's amazing," she adds.

Kim sees her role as "a pit crew member—I keep the car running." In addition to her accounting duties, she's the office manager, the HR department and whatever else is needed to keep the organization humming along. "A lot of people don't know my face, but when a tire needs changing or an engine needs tuning, I'm the one doing it."

"We all work toward one goal—to find a cure for CMT disease," Kim says. "In the meantime, we try to make the lives of people who have or deal with CMT easier."

Born and raised in Pennsylvania, Kim still lives there with her husband of 32 years, Danny, and their two children Danny, 29, and Noel, 26, live close by. Doing good for the world even in her off time, she crochets every day, mostly blankets for families in shelters or kids in need.

JONAH BERGER

National Youth Programs Manager

Jonah Berger, the CMTA's beloved national youth programs manager, has the youthful spirit of the kids he leads, backed by years of education and experience.

He earned a bachelor's degree in theater and speech from Frostburg

Jonah Berger with wife Megan and baby Amelia Rose


Jonah Berger

State in Western Maryland, followed by a master's degree in special education from Trinity College in Washington, D.C. He then spent 20 years in Colorado developing a special needs mentoring and employment success business called The Rhythm Within. He also served as camp director for several programs and gave motivational speeches around the country.

For years, Jonah led outings for kids whose parents were attending the CMTA's Patient Family Conferences. When Camp Footprint launched in 2016, Jonah was the natural choice to lead it. The demand for youth services kept growing, and he was brought on to develop and run a comprehensive, year-round youth program in 2020.

Jonah says he has always loved the CMTA and its focus on patients. "Community and cure have always been truly important to this organization, and that's why I got involved. That feeling has grown through the years, and the cure gets closer and the community gets ever stronger."

Jonah inherited CMT1X from his mother. He says his CMT journey was eased by the support of relatives who also have it and by his lifelong connection with the CMTA. His 3-year-old daughter also has it. Her attitude—and those of every kid in the youth program—will undoubtedly be shaped by Jonah's: "I do not let CMT hold me back. I strive to find the benefits and hidden gifts in CMT. I am thankful for the perspective it gives me and the unbelievable people whom I have met as a result of the CMTA. We can do

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SWARMING THE SUMMIT

To give just one example of the CMTA pit crew in action, take the CMT Patient & Research Summit scheduled for Oct. 1.

About a year in advance of the summit, the team began brainstorming and building consensus around the goals for the summit. The summit's goals are to educate the CMTA community about research progress, encourage patients to get involved in research studies and stress the importance of funding to deliver STAR's promised acceleration. This year, the CMTA is adding more breakout sessions to cover research across all types of CMT and providing more custom content to the community.

PIT CREW CHIEF AMY builds the STAR agenda for the event, extending invitations to researchers and biopharmaceutical partners to showcase the CMTA's overarching research strategy, key research projects and important partners.

LAUREL wears the "event manager" hat for the summit, working closely with the entire team to achieve those goals. She issues speaker invitations and coordinates all speaker engagement logistics. For in-person summits, she also works with the team to choose the best city and the best conference venue, then manages all negotiations with the hotel. She also leads the 2022 Summit Steering Committee, which shapes the morning sessions of the summit on managing and living well with CMT.

SARAH G. heads up the technical aspects of the virtual event, partnering with a top-notch virtual event platform to offer an immersive and engaging summit experience to CMTA community members. The platform is set up to provide community members the opportunity to participate in virtual vendor booths, discussion tables and interactive presentations that include polling, leaderboards, Q&A and live transcription for multiple languages. Pre-summit, Sarah G. provides platform training and presentation design support to clinicians and researchers. Following the conference, Sarah G. ensures that conference materials are added to the summit video session library on the CMTA's website. Focusing on digital inclusivity and accessibility, Sarah G. says, ensures that STAR updates and helpful patient-centered care tips reach each member of our community.

SARAH K. continuously transmits creative advertising to the community about the event and strategizes about how to reach a larger audience. She encourages members of the digital community to sign up through social media, email and the CMTA website. Sarah K. is also responsible for communications on the day of the summit and afterward, sharing quotes, recordings and statistics with the community.

JEANA provides input on how best to deliver STAR research updates and secure corporate sponsorships from biopharmaceutical partners for the event. She also encourages the CMTA's donors to tune in to see how their gifts are impacting research. Kim keeps track of the budget and donations, while Jonah helps out with day-of logistics and invites members of the Youth Council to chat with attendees about youth programs at the CMTA.

After big events like the summit, the team debriefs, talking about what went well and what can be improved. Taking the time to reflect and learn helps to improve performance, Laurel says. The pit crew continually challenges each other to reach more people and to share information about CMT that will improve the quality of life for all community members.

MEET THE CMTA “PIT CREW”

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anything we want to do; we just have to find our way of doing it!”

Never one to stay still, Jonah has written and published two books and completed two triathlons. Like the kids he leads, he fuels those activities with pizza and says he can eat more than three average adults.

SARAH GENTRY

Technology Manager

Every pit crew has a technologist, and Sarah Gentry (Sarah G.) fulfills that role for the CMTA, where she is responsible for the strategic development and advancement of technology and cybersecurity.


Sarah Gentry

She attended the Rochester Institute of Technology, where she earned a bachelor’s degree in applied arts and science and a master’s degree in security technology management. She has co-written books on cyberbullying, cyberstalking and online gaming. Before joining the CMTA, Sarah was an instructional technologist at the University of South Florida, assisting faculty, staff, and students with instructional technology, professional development, online course design and review support.


Sarah G. with wife Jessica and Daphne Olivia Gentry or D.O.G

Sarah, who has Type 2, was first introduced to the CMTA in 2015 when she attended a local branch meeting. At the time, the CMTA was seeking counselors for Camp Footprint and Sarah signed up. It was an amazing experience, she says: She felt at home immediately and strangers quickly became family.

In addition to serving as a camp counselor in 2015, Sarah has been a branch leader, a walk leader for the Tampa Bay Walk-n-Roll and a volunteer for youth outings. She plays a key role in setting up the patient research summit (see page 11). Outside of the summit her focus includes reviewing the CMTA’s current digital landscape and figuring out ways to enhance our infrastructure to improve efficiencies and better support our growing CMTA community.

Outside of work, Sarah likes to spend time with her 9-year-old Lhasa Apso Havanese mix Daphne Olivia Gentry, or D.O.G. She loves to plan vacations that involve going to a Wegmans Supermarket and will pick accommodations based upon the distance to Wegmans. She’s also a major Syracuse basketball fan and says that Syracuse winning a March Madness game on your wedding day is good luck. She should know—it happened to her.

SARAH KAIDER

Digital Marketing Manager

Sarah Kaider (Sarah K.) is the CMTA’s newest employee and a welcome addition to the pit crew. As digital marketing manager, she is responsible for the CMTA’s social media, email marketing and website. When something happens in the world of CMT, Sarah gets the word out.

Sarah earned a bachelor’s degree in mass communications with a focus in advertising and public relations from Towson University in Maryland. While in school, she interned with the Cystic Fibrosis Foundation. Post-graduation, she has spent her entire career working with health-based nonprofit organizations and says that as an empath, she derives the most fulfillment in her life and career when she can make a difference in the lives of others.


Sarah Kaider with fiancé David

After graduating, Sarah began working as the social media coordinator for the Epilepsy Foundation of America (EFA). She was soon promoted to social media manager, and shortly after, communications manager. She was working at the EFA when she started dating her fiancé David. On their first date, he shared with her that his hands were shaking while he was eating because of his CMT, not nerves.


Sarah K. with Theo

After researching CMT online, Sarah began volunteering with the CMTA. When the digital marketing manager position opened up, she was thrilled to be able to meld her expertise in marketing and passion for helping others with her drive to support her fiancé.

Sarah is the proud mom of a Cardigan Corgi named Theo and an African Pygmy Hedgehog named Regulus (Reggie). When she’s not penning social media posts, she writes science-fiction/dystopian stories and her dream is to be published by HarperCollins or Penguin Random House.

REGISTRATION OPENING IN JUNE

2022 CMT PATIENT & RESEARCH SUMMIT

Charcot-Marie-Tooth Association

VIRTUAL • SATURDAY, OCTOBER 1, 2022

Sessions 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!

Sponsored by APPLIED THERAPEUTICS

NEW PATIENT SUPPORT FUND FOR TRAVEL TO CEDARS-SINAI FOR FOOT SURGERY EVALUATION

Thanks to a generous donor, the CMTA has established a Patient Support Fund for patients being evaluated by a foot and ankle surgeon for CMT surgery at Cedars-Sinai Medical Center in Los Angeles. The fund will also provide financial support for those traveling to Cedars-Sinai Medical Center for second opinions. Cedars-Sinai is a CMTA Center of Excellence with a skilled surgical team.

Patients are expected to make every effort to finance their care. The fund is intended to provide supplemental assistance if they fall short. It can provide up to $5,000 in financial support per patient over the course of treatment. The fund will pay for one or two people to travel to Cedars-Sinai for a consultation or surgery.

Patients must submit all necessary paperwork to Jeana Sweeney at jeana@cmtausa.org or CMTA, Patient Support Fund, PO Box 105, Glenolden, PA, 19036, along with a patient verification letter from Dr. Glenn Pfeffer, CMTA Advisory Board member and director of the foot and ankle surgery program at Cedars-Sinai. The CMTA will make a decision within 14 days of receiving the application.

To apply for support, go to www.cmtausa.org/patientsupportfund.

Dr. Glenn Pfeffer

Cedars Sinai

INNERVATORS HONOR ROLL

ACCORDING TO THE DICTIONARY, an innervator is a nerve stimulator. According to the CMTA, an Innervator is an action-oriented donor who gives monthly to the organization, sustaining STAR (Strategy to Accelerate Research), our premier research initiative, as well as important community initiatives like Camp Footprint, Patient/Family Conferences and educational programs. All of these programs require steady, reliable support from committed donors.

Please join the Innervators today!

www.cmtausa.org/cmta-innervators

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> *"One of the best things my involvement with the CMTA has brought me is acceptance. I've learned to live comfortably in my skin, as imperfect as it is, with grace and resilience. I am grateful for all the incredible research that is leading to a cure, and I hope it will help my children and grandchildren live their lives without CMT. For me, the challenge of living with a slowly degenerative disease is made easier because I've been able to improve my mental health and confidence by exposure to all the positivity in The CMTA Report. Knowing that I am not alone and that there are many others with CMT living good, full lives is inspiring. That's why I'm an Innervator."* **—DIANE COVINGTON**

The Oaxaca-Thomas family and friends fundraiser

Bailey's Beat the Bite 5K Walk Raises $60,000 in Six Years

More than 30 people came out March 26 for Bailey's Beat the Bite 5K Walk in Tucson, Ariz., raising a total of $10,435 for CMT research this year. Over six years, the event has raised a total of more than $60,000.

Born on the 4th of July 2009, Bailey Roestenburg had a typical early childhood, running around, dressing up like a princess and wearing heels too big for her feet. When she was 3, her mother, Vanessa, noticed signs that she might have the same disease her father did—her feet turned inward, she fell several times a day while walking, and eventually, she was dropping to the ground because her ankles couldn't support her. By the age of 4, Bailey was wearing AFOs to try to keep up with her peers.

In 2017, members of her family launched Bailey's Beat the Bite 5K to raise money for medical research so that one day no one will have to live with CMT.

In the past six years, the Oaxaca-Thomas family not only planned a 5K walk but also came up with aother creative ways to fundraise and raise awareness about CMT. Every year, Bailey, now 12, challenges her aunt, Kristen Oaxaca, to a Facebook Live duel: They throw pies in each other's faces or pour food on each other's heads for donations. During the pandemic, the family traveled all over Tucson planting pink flamingoes in yards to get some buzz for their virtual event.

Back in person this year, the Oaxaca-Thomas family again hosted a Facebook Live challenge, "flocked" the local community with pink flamingoes and held a fundraising night at a local bar. The 5K was accompanied by a raffle, with more than $3,000 of in-kind donations from local businesses. The family would like to give special thanks to sponsors Global Construction Management, QMA Patios, Audacity Graphic Design, Supporting Strategies - Tucson, Ariz., and Chloe Preparatory Academy.

"We started out by doing this for Bailey, but we come back every year to raise money for everyone with CMT," Bailey's Aunt Kristen says.

LOOKING FORWARD TO CAMP FOOTPRINT

BY BRIDGET SAVANT

"I promise it all grows back," veteran camp counselor Casey Cardone said as we drove past bare trees down the road to Camp Kon-O-Kwee in Fombell, Pa. It was March, and it was freezing, but the warmth in our hearts grew as camp came into sight. It had been two and a half long years since we'd been back, but virtual Camp Footprint was over and it was time to plan Camp Footprint 2022, scheduled for August 8 to 14, in person.

While camp was missing its summer greenery, counselors and campers, it triggered many happy memories and fresh ideas. The program staff spent the weekend in a creative think tank, brainstorming how to make the seventh year of Camp Footprint the best yet. We are expanding in multiple ways: We are expecting 125 campers, a 25 percent increase over last year, and we're adding an extra day to the week. The older kids are coming back as counselors; the younger kids are full-blown teenagers now; and a whole new group will join the tribe. Our excitement over seeing everyone face to face for six days is through the roof.

Entrance to Camp Footprint

Now, a bigger, longer camp calls for some adjustments. Fortunately, this group is VERY experienced in making modifications. We analyzed every activity on the schedule to figure out how to offer plenty of time for all to participate. We analyzed past feedback, prioritized quality and fine-tuned our flow. We went down the rabbit hole with our music theme so it weaves throughout our days. We really thought this through, people! No more feeling rushed; we made sure there will be plenty of time to just enjoy our precious days together. Especially in the pool. We LOVE the pool.

After many laughs, deep discussions and pulling inspiration from our favorite Camp Kon-O-Kwee locations, the program staff left feeling exhausted and full of joy. This is what this magical place does to you. There's nothing like some fresh air, limited electronics and the best of the best people who totally get your CMT to remind you of what life is all about. While it was incredibly lovely to have a weekend planning retreat (thank you CMTA!), we can't WAIT for the full tribe to be back together again.

Campers should get ready for more time to hang with friends, good food (yes, they totally revamped the meal plan!), a safe place that welcomes everyone and a wonderful life-changing week. Our campers and staff are in the very best hands, CMT and all. Stay tuned for camp teasers and more info coming your way in the next few months. August can't come soon enough!

Paddleboarding at Camp Footprint

Bridget, a San Diego choreographer, leads the chair dancing that begins every day at Camp Footprint.

To sign up for the CMTA's Youth Program, scan here:

CMT Community Education Provides Opportunities to Learn and Connect

A critical part of the CMTA's mission is to improve the quality of life for people who live with CMT. That includes educating community members and their families about CMT and providing opportunities to connect with others who live with the disease. Our educational programs focus on a wide variety of topics that provide a deeper understanding of CMT—genetic testing, where to find a Center of Excellence, what braces work best, surgical options for correcting the foot, help for kids at school, youth programs, fundraising events and much more. Wherever you are in your CMT journey, education can be part of your path.

To receive email announcements when registration opens for these meetings, sign up for eNews at cmtausa.org/enews. To learn more, simply scan this QR Code: All events are listed on our calendar at cmtausa.org/get-involved/events.

HERE ARE FOUR GREAT OPPORTUNITIES TO LEARN AND CONNECT IN 2022:

- **Monthly education meetings** (registration typically opens three weeks before this virtual meeting) and branch meetings: cmtausa.org/get-involved/events/ Scan the QR code at right to learn more.
- **CMT 4 Me Podcast:** cmtausa.org/living-with-cmt/find-resources/cmt-4-me-podcast/
- **CMTA Webinar recordings library:** cmtausa.org/living-with-cmt/find-resources/cmta-webinars/
- **CMT Patient & Research Summit:** Saturday, October 1 (virtual): cmtausa.org/living-with-cmt/find-resources/patient-family-conferences/

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, the CMTA Director of Development at 814-269-1319

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

Advocating for a Child with CMT Through the Maze of 504s and IEPs

By Sarah Kesty

While it's the beginning of summer, it's never too early to start thinking about how to make the next school year the best possible for your student with CMT. CMT is a disability that qualifies your child for support at school, but whether that's achieved through an IEP (Individualized Education Plan) under the Individuals with Disabilities Education Act (IDEA) or a 504 plan under the Rehabilitation Act of 1973 is a matter of impact. Both 504 and IEP laws are written in the spirit of ensuring access to a "free and appropriate public education" (FAPE), yet they differ in many ways.

In general, 504 plans are best if the support your child needs is similar to what they'd need out in the world, mostly through accommodations. IEPs, on the other hand, are more specific to education supports; they include services like physical therapy (PT) and occupational therapy (OT) and address the aspects of your child's disability that impair their access to learning.

Your school will likely discuss the options with you. However, it's important to go into meetings prepared. Remember, the school psychologist, administrator, and teachers have held hundreds of similar meetings and may rush through. It can also be a bit overwhelming to be at the table with so many others, receiving lots of information about your child.

To help you prepare, here are some reflection questions to get started. If you already have a 504 or IEP, these questions can help you prepare for the next update meeting, to ensure your child's plan covers every aspect of the school experience:

- How does CMT affect my child?
- What devices or assistive technology does my child use?
- Do these devices require the classroom to be physically changed in order to use them? (Think wheelchairs or mobility devices.)
- How does CMT impact my child's movement throughout school?
- How does CMT impact my child's written work? Fine motor skills?
- Does my child need/have services like speech, OT or PT in order to progress in school?
- Do grading policies need adjustments to allow for extra time?

If your reflections indicate that changes to the school environment—called accommodations—are needed, then you're likely best served by a 504 plan. Your child's 504 plan will document the accommodations and possible modifications the school can make to ease your child's school experience

continued on page 19

The Mystery of Rare Diseases:

A LESSON PLAN FOR STUDENTS

Explaining CMT to others can be tricky; its symptoms vary widely and its impact is often invisible to outside observers. Explaining it to children is especially challenging. How do you walk the line between empowering students with information and overwhelming them with pathology? How do you elicit understanding without engendering pity?

The Mystery of Rare Diseases is a lesson plan for grades 6-8 created by CMTA Advisory Board Member Sarah Kesty of Castle Park Middle School in Chula Vista, Calif. It was selected as the best lesson about rare diseases in a contest hosted by Sarepta Therapeutics. Sarepta launched the competition in order to promote the development and implementation of rare disease education in K-12 classrooms.

The Mystery of Rare Diseases guides students through the concepts behind rare disease manifestations and diagnosis. They learn that rare diseases can be difficult to diagnose and the importance of research. Students match a patient's symptoms with potential diagnoses like CMT, Neimann Pick, Weaver Syndrome and acoustic neuroma using the investigative process.

Following discussions, students come to understand that in real life, millions of people are impacted by rare diseases and require making inclusive practices for individuals with rare diseases significant. What seems easy at first becomes complicated, as symptoms overlap and correlate with disease-caused challenges. For example, someone with Weaver Syndrome, acoustic neuroma or CMT may have trouble with balance; the causes are different but the symptoms are the same.

Find Sarah's lesson and more on rare disease awareness at sharemylesson.com/teaching-resource/mystery-rare-diseases-329456.

Walk 4 CMT

When You Walk with Us, You Walk for Them!

Walk 4 CMT is an opportunity to walk, run or roll towards a treatment and ultimately a cure for Charcot-Marie-Tooth Disease. Join thousands of community members across the country and the globe by visiting www.cmtausa.org/walk to find an event near you. If there is no event in your area, please consider starting your own. We would love to help you help the CMT community!

ALL WE NEED IS YOU!

CMTA
Charcot-Marie-Tooth Association

WALK 4 CMT

For more information about participating in, or starting a Walk 4 CMT, please email Mary at mary@cmtausa.org

504s continued from page 17

and ensure their disability doesn't prevent access to learning.

For example, if your child's CMT makes gross motor movements challenging, they may receive a 504 plan to modify PE with alternatives to runs or long walks and provide transportation or extra time between classes so they can move at their own pace. Accommodations for fine motor challenges could include permission to type instead of write, extra time on written work or tests, or removal of penalties for "messy" handwriting.

504 plans are not technically special education because they fall under a different section of law. But let's be real. It's going to be the same team at school, usually, that runs the show and updates the plan.

If your reflections indicate that your child needs services and accommodations, then an IEP may be a better fit. IEPs can include accommodations, just like 504 plans. They can also include services from specialists to help your child advance with specific skills. They are meaty documents that describe what the school proposes to support your child in the coming year.

CMT does not impact intelligence, nor is it known to cause learning difficulties, making significant academic support less likely. Instead, your child's probable best setting will be in a general education class with some services like a resource specialist program (RSP), OT or PT. A RSP teacher may coordinate your child's school experience and modify schoolwork or physical education to meet their needs. These services can happen in class or in a separate setting at school.

In short, 504s are accommodation-based and more about access, while IEPs focus on more specific education support and can also include accommodations.

If you're still not sure which one's right for your child, don't fret. Try reaching out (in writing, always) to your school psychologist, explaining your child's needs and requesting a meeting. That starts a timeline for their response and will get the ball rolling.

Sarah Kesty is a board-certified advocate in special education and a member of the CMTA Advisory Board. She is also an executive function expert, an international speaker, a published author and an autism specialist with the state of California. For more information, visit her website at IEPOMG.com or check out the IEPOMG podcast.

If you still have questions about IEPs and 504 plans, the CMTA will present an educational webinar on the topic on Aug. 19 at noon, just in time for back to school. To register for this event, visit https://www.cmtausa.org/events/education-meeting-kids-at-school/

SENIORS WITH CMT: 'INCREDIBLE' HUSBAND, WATER EXERCISE KEEP 87-YEAR-OLD GOING

Jean Haynes cannot be stopped. Her positive attitude, helpful husband and water exercise keep her busy and active, despite her CMT. "Just keep going," she advises, adding, "You can do more than you think."

Jean and Ron

Jean was born and raised in northern Indiana. Her father and three out of seven of his siblings had CMT, but they had no idea what it was. Their ankles were thin, their arches high and they had difficulty walking. They referred to the problem as "funny feet."

Jean and her 90-year-old brother, Bob, had the same signs and symptoms, although his started earlier in life and were more severe. He was the first in their family to be diagnosed. On a visit to Mayo Clinic in Rochester, Minn., he was shown a picture of a patient with CMT. His feet were exactly the same, giving the family a name for what they had. Jean's daughter Cheryl also has Type 2, but her two sons have shown no symptoms.

Other than turning her ankle and falling, Jean had virtually no symptoms in early adulthood. Her husband, Ron, 89, a retired physician, began holding her hand or arm whenever they walked together, preventing many falls.

She began playing tennis in her 30s and played until her 60s when she had to give it up because she fell every time she played. She started using a cane in her 60s and a walker in her 80s. She has owned leg braces for a number of years, but like others, doesn't wear them "because of vanity."

Today, Jean uses a walker all the time and says she does quite well with it if she doesn't have to travel too far. When she does, her legs begin to ache and her feet to drop.

Jean says she is blessed with an incredible husband, who helps with her walker so that they can lead a fairly normal life. They've been married 65 years and still make weekly trips to the farmers' market and the beach to walk and sit on the boardwalk. They also take water aerobics in a heated pool three times a week.

Jean and Ron live in a senior community in Vero Beach, Fla., on the Atlantic Ocean. There are no stairs, making it a true paradise for a senior with CMT.

THE CMTA GRATEFULLY ACKNOWLEDGES GIFTS…

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Verdelli Family
Dan Verdelli

Harriet Weiss – “Happy Birthday!”
Mr. and Mrs. Marilyn Berger

IN MEMORIAM:

Seth Warfield 1943 - 2022

The CMT community lost one of its greatest champions April 16 with the death of volunteer, advocate and fundraiser extraordinaire Seth Warfield.

Seth and his wife Missy worked as a team in the fight against CMT. Missy, his wife of 55 years, had the disease, but Seth was by her side every difficult step of the way. They were inseparable and people said their names as if they were one—MissyandSeth. Together they raised millions for CMT research.

CMTA Board Chair Gilles Bouchard said, “What struck me from my very first meeting with Seth and Missy is how close they were, and how well they complemented each other. As a team, they became a major force behind CMT research with a profound and lasting impact. I will sorely miss Seth’s kindness, curiosity and relentless support.”

CMTA CEO Amy Gray commented, “Seth and Missy’s early support for CMT1A research was critical and provided the foundation for much of what followed. Their support will make an impact that extends far beyond Seth’s lifetime. He was a shining example of the good that volunteers can do. We are all grateful to have known him and for his support of our mission.”

Jeana Sweeney, chief engagement and gifts officer for the CMTA, said, “Besides my husband, I have never met a man who loved and cared for his wife as Seth cared for Missy. Watching them together was like reading a romance novel. On every phone call I made to Missy, Seth was in the background, asking the hard questions, giving advice and quizzing me on numbers—all while getting Missy a cup of hot tea. Seth believed in our work, he supported us and held us accountable out of his love for Missy and his family. He wanted nothing more to find than a cure for them and all living with CMT. He has our promise that one day we will. I will miss him greatly.”

Seth was a graduate of Lehigh University (class of ’65) where he earned a degree in industrial engineering and a master’s degree in management science. After completing his studies, he grew his family’s business from a single roofing company to a roofing supply chain with 24 locations in Maryland, Virginia, Delaware and Texas.

After retiring to the Eastern Shore of Maryland, Seth and Missy indulged their love of sailing, family and volunteerism. They started and led the Easton, Md., CMTA branch. Seth became active in Habitat for Humanity, helping build houses with the “Thursday Crew” and starting the local Habitat Re-Store. He also served on the Vestry, Finance and Endowment committees of Christ Church, Easton.

The couple got involved with the CMTA when their two children were diagnosed with CMT1A, and ramped up their involvement when two of their six grandchildren were diagnosed with the disease as toddlers. They felt an urgent call to encourage more research through donations to find the cure and became dedicated fundraisers and advocates.

A gentle man and a gentleman, Seth will be remembered in the CMT community for his generosity and commitment to finding a cure for CMT.

The family asks that donations in Seth’s memory be made to the CMTA, PO Box 105, Glenolden, PA 19036. Online condolences can be offered at www.fhnfuneralhome.com.

SUPPORT THE CMTA AND HONOR A LOVED ONE WITH AN IN HONOR/IN MEMORY GIFT

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○ **YES, I WANT TO BE AN INNERVATOR—**Please make my gift a monthly donation.

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

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

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My gift is ○ In honor of ○ In memory of: (Name) ____

Please send me CMTA updates via email. My email is: ____

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

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,

My husband and I recently retired. We had always planned to travel more when we stopped working, but my husband tends to plan complicated trips that don't take account of my CMT. I am 65, and although I am not severely affected I do need a cane, and fatigue can be a problem if I overdo it. Every time I try to gently bring up my concerns over his plans, he tells me I'm a worrywart and shuts down any further conversation. It's gotten to the point that whenever he mentions a trip he is planning I start to get anxious. I feel my resentment toward him building, especially when he says that I should be grateful that he's doing the planning. Am I being overly dramatic with my concerns?

David Replies:

The first thing I want to say is that none of us should ever have to apologize for having CMT. We have enough to deal with without having to justify our anxiety at the thought of traveling, especially if we are not sure our destination is accessible. Your husband's irritated response to your concerns could be masking his fears around your increasing vulnerability, or his fears around aging in general. Regardless, his reaction to you warrants a serious discussion. It's one thing when friends are oblivious to what we experience daily, but a spouse's insensitivity is unacceptable. Tell him that if he doesn't want to travel alone he will need to listen to your concerns more compassionately.

Traveling can be a wondrous experience even with CMT, but it does require additional planning. Researching accessible destinations on your own or with a good travel consultant could allay many of your fears. What's more concerning is your husband's response to your anxiety. I often feel that having CMT and anxiety are closely related. Our friends, relatives and even spouses often have no idea of the kinds of situations that make us uncomfortable. It's one thing to manage getting around our homes and other familiar settings, but traveling to less familiar places can be daunting. It's very possible to enjoy traveling with CMT, but it's imperative to have a sensitive travel partner. Be very specific with him about the kinds of situations—like stairs or uneven surfaces—that cause you anxiety. Even the small boats that shuttle passengers from a cruise ship to land can be treacherous for those of us who are unsteady on our feet. Most importantly, let go of any shame or embarrassment about needing extra help. Tell him you need his emotional support if the trip is going to be fun for both of you. A simple statement from him like, "Don't worry sweetheart; I'll be there to help you" goes a long way.

In general, don't be shy about asking for help. If you need wheelchair assistance for airports or museums, get it. Being able to take your time and not worry about falling can make a beautiful difference in being able to relax and enjoy sightseeing worry-free.

Get involved with the planning because you know your body and what you can or cannot handle. Knowing your own energy capacity and planning accordingly can eliminate a lot of stress. I have personally found that I end up exhausted on trips that I overschedule, as opposed to seeing less but enjoying it more. Worrying about keeping up with your husband will cause you to feel irritable and resentful. Keep in mind that you don't have to be tied at the hip: If he wants to see something and you are too tired, there is nothing wrong with his going off on his own for a few hours. You will get some rest and may even enjoy the time apart. His telling you what he experienced upon his return could be a lovely and intimate moment.

Before you go, get an informal commitment from him that when you tell him that you are tired for any reason, he will stop, truly hear you and not judge or criticize. Remember that asking for help does not mean you are helpless. Everyone needs help in some form. Those of us with CMT might need some assistance because of our mobility issues. Your husband needs help in learning to be more compassionate.

Bon Voyage!

Frank's PHARNEXT Diary

EDITOR'S NOTE: Late last year, Pharnext began Phase 3 clinical trials to evaluate PXT3003 versus placebo in CMT1A patient subjects aged 16 to 65. PXT3003 is a novel, fixed-dose synergistic combination of baclofen, naltrexone and sorbitol formulated as an oral solution. Individual components of PXT3003 were selected to downregulate the overexpression of PMP22 protein, leading to improvement of neuronal signaling in dysfunctional peripheral nerves. Frank Stone, an accountant from New Hampshire, is participating in the trials and recounting his experiences on Facebook, from which the following account is excerpted.


Frank Stone and faithful friend Rowdy

Nov. 10, 2021

Day 1 of tests, *then the first dose of PXT3003 for Phase 3 Trial at Massachusetts General Hospital with hopes of slowing down the disease progression. Praying for great results so this can get to market after FDA approval for CMT Type 1A.*

I will keep everyone posted during the journey. Keep up the good fight!

Nov. 14, 2021

Day 4 of trial. *The first two weeks are half a dose am and pm. Treatment is oral, and the roof of my mouth is now tender. Will discuss options with the doctor to improve/prevent. The treatment has stopped my thighs from constant twitching, though my calves still twitch all day.*

In other good news, the treatment appears to be adding strength to my legs as I'm doing multiple flights of stairs with no burning and discomfort in my thighs. I'm walking quite a bit with no pain or fatigue. My feet and ankles are still very weak and I was not expecting these early results. My legs haven't felt like this for 15+ yrs.

So promising!! Screw CMT! Hope this trial is successful for everyone. Other than the very tender roof of my mouth, no side effects!!

Nov. 20, 2021

Day 10 completed. *Leg strength and endurance improving. I'm jogging on a treadmill at 3.7 mph for six minutes and walking for 24 minutes. I haven't been able to handle anything like this since my mid-40s and I'm now 55. I'm not running but maybe someday with this trial. I'm quite sore but it feels good being able to push.*

I had a few days when I felt terrible and skipped all exercise and walking. Day 7 and 8 I was wiped out. I was lightheaded, couldn't sleep and had little appetite. This has passed, thank goodness. The sore mouth has stopped with twice-daily use of Act mouth rinse with brushing.

My golden retriever, Rowdy, pushes me every day...such great therapy.

Dec. 6, 2021

Month 2 of PXT3003 treatment. *Doses have been doubled since the start of week 3 for am and pm treatment. My legs are quite strong compared to the beginning of treatment. I always used a handrail going up and down stairs before treatment, but now I walk up and down with some pace behind my dog Rowdy. He certainly helps drive me. The most amazing thing is the change of approach and mindset. I don't think about my legs during the day. This is refreshing. I'm up to 35 to 40 minutes on the treadmill on the #3 incline. My hands and grip have improved.*

I did push a bit too much four weeks ago and injured my meniscus, which hurt quite a bit for 10 days or so. My lower legs and ankles remain weak but the medicine works its way down the extremities from what I was told.

The first week of double doses was an adjustment dealing with stomach pains. My body has now adjusted and there are no other issues or side effects.

My focus for month 3 will be calves, ankles and feet. I can't remember not thinking about my legs, feet and hands for an entire day and night.

Blessed this trial is available during our time, and I'm thankful Massachusetts General approved me for the study.

March 11, 2022

I've been very active and pleased with this treatment twice a day. My energy is quite high and my legs feel great. I'm wiggling my toes more and can feel the tips of my toes now using my fingers. My hands are not cranky in the morning either, which is awesome. I have more concentration at work along with minimal to zero anxiety given I don't think about my legs anymore. My ankles are still weak and my left calf still twitches all day, but leg strength and balance are vastly improved as I walk daily 3+ miles with my golden retriever on a treadmill or outside. I need to do more on flexibility as I feel much tighter with all the exercise I'm doing to beat this terrible disease. Good news: No more side effects to date except for a mild rash for a few weeks.

To add your profile to the CMTA's Patients as Partners database visit the CMTA website or scan:

BRANCH NOTES

SAN DIEGO, CA

The San Diego branch held its first “hybrid” meeting April 16, with members taking part both in person and virtually. Guest speaker Hampus Hedland of Allard USA shared information about a variety of his company’s bracing options for CMT patients. He also answered questions and provided information about finding the right braces.

SOUTHERN CONNECTICUT

The Southern Connecticut branch welcomed guest speaker David Misener, BSc (HK), CPO, MBA to its virtual meeting Feb. 16. David is a board-certified prosthetist and orthotist and a CMTA Advisory Board member. He shared his own experiences living with CMT and how his bracing needs have changed as his CMT has progressed. He spoke about the various bracing options available and how important it is to see an orthotist for the best fit and to communicate your particular needs. He also answered questions about how to get and pay for braces and the best shoe brands to wear with AFOs.

JACKSONVILLE, FL


Dr. Lamb

Members of the Jacksonville branch welcomed neurologist Dr. Christopher Lamb to their virtual meeting March 1. Dr. Lamb leads the CMTA Center of Excellence at the Mayo Clinic in Jacksonville, Fla. He talked about the CMT Clinic and what patients can expect when visiting. After sharing information about the patient experience, he answered questions from the group.

NAPLES, FL

The Naples branch met on Zoom Jan. 18 to catch up and share updates. Topics ranged from why people should learn their CMT types to practical tools that make life easier living with CMT to the CMTA Centers of Excellence. Members agreed to meet quarterly and expressed a desire for an in-person meeting with virtual capability. Topics for future meetings were discussed, revealing particular interest in the topic of breathing and CMT.

SARASOTA, FL

Twelve members of the Sarasota branch (including a couple of new ones) started their virtual meeting Jan. 22 with hand, arm and leg exercises for people with CMT. After a lively discussion of members’ latest news, participants shared tips on various CMT topics. As often happens at branch meetings, the new members found the experience of meeting others with CMT for the first time both meaningful and helpful.

CHICAGO, IL

Ten members of the Chicago branch met virtually March 24 to talk about CMT gadgets and life hacks to make daily life with CMT easier. Members shared their CMT journeys and gadget recommendations and compiled a list of helpful tips to be sent to all branch members.

BOSTON, MA

The Boston branch met virtually Feb. 8 with 21 people in attendance. New branch leaders from Dallas/Fort Worth and Wisconsin joined the meeting, along with several newcomers. The group heard clinical trial updates from participants in the PXT-3003 and CMT-SORD clinical trials. Recruitment is now closed for the PXT-3003 trial, but Applied Therapeutics is still enrolling CMT-SORD patients. Attendees spent a lot of time discussing where patients newly diagnosed with CMT should go for help. Members recommended Dr. Reza Seyedsadjadi, the clinical director of the CMTA Center of Excellence in Boston at Mass General, as the best CMT expert in the area, and Ken Cornell of Cornell Orthotics as the best person to visit for foot inserts or AFOs.

NEW MEXICO

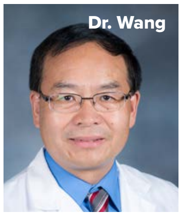
Nine members—including two new ones—participated in the New Mexico branch’s virtual meeting on Feb. 5. The session focused on CMT gene therapy research, and the group viewed the CMT Type I gene research session recorded at the CMTA Patient/Family Conference last November. The group also discussed difficulties in getting genetic testing results and a University of New Mexico neurologist that two group members are seeing. Another member talked about his appointment with Dr. Michael Shy at the CMTA Center of Excellence in Iowa.


Julie Barnett

WESTCHESTER, NY

The Westchester, NY branch welcomed guest speaker Julie Barnett to its virtual meeting April 2. Julie is a fitness instructor/personal trainer who lives with CMT. She moderates the CMTActive Group on Facebook. Julie shared information on what to do to stay as mobile and flexible as possible.

CINCINNATI, OH


Dr. Wang

The Cincinnati branch welcomed neurologist Dr. Zeng Wang to a virtual meeting April 13. Dr. Wang leads the CMTA Center of Excellence at the University of Louisville in Kentucky. He talked about the CMT clinic at Louisville and shared information about the patient experience and what to expect when visiting, then answered questions from the group.

PITTSBURGH, PA

Eight members of the Pittsburgh branch welcomed guest speaker Mary Louie to their virtual meeting on April 9. Mary, the CMTA National Events Manager, talked with the group about its upcoming Walk 4 CMT and shared information about upgrades to the WALK website platform. She also shared highlights about upcoming CMTA educational events and programs.

MADISON, WI

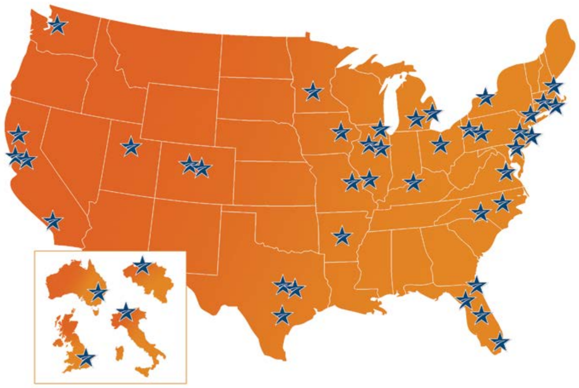
On March 31 the Madison branch welcomed guest speaker John Svaren, PhD, to a virtual meeting. John chairs the CMTA Scientific Advisory Board and lives in Madison. He is a professor in the Department of Comparative Biosciences at the University of Wisconsin and serves as director of the Cellular and Molecular Neuroscience core at the UW Waisman Center, where his lab is located. John shared some promising updates on the latest research happening at the lab, which is part of the global research effort spearheaded by the CMTA. John has given the Madison branch research updates over the years, but this year’s update really stood out and showcased how far research has come.

MANITOWOC, WI

The Manitowoc branch welcomed guest speaker Kenneth Raymond, a CMTA Advisory Board member and patient advocate, to a virtual meeting March 14. Kenny shared information on CMT genetics, which he has studied for years. The group also set the date for its branch fundraiser, a Walk, Bike, Wheelchair 4 CMT event, which will take place Sept. 17 on the Mariners Trail, a paved trail along the shoreline of Lake Michigan between Manitowoc and Two Rivers.

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.



ARKANSAS

LITTLE ROCK (PEDIATRIC)
Arkansas Children's Hospital
Clinical Director: Aravindhan Veerapandiyan, MD
Appts: 501-364-1850

CALIFORNIA

LOS ANGELES (ADULT & PEDIATRIC)
Cedars-Sinai
Clinical Directors:
Matthew J. Burford, MD, and
Richard A. Lewis, MD
Appts: Tara A. Jones, MS, CGC, 310-423-4268

PALO ALTO (PEDIATRIC)
Stanford Children's Health*
Clinical Directors: John Day, MD, PhD, and Ana Tesi Rocha, MD
Appts: 650-723-0993

PALO ALTO (ADULT)
Stanford Neuromuscular Program*
Clinical Director: John Day, MD, PhD
Appts: 650-723-6469

SAN FRANCISCO (PEDIATRIC)
University of California, San Francisco
Clinical Director: Alex Fay, MD, PhD
Appts: 415-353-7596

COLORADO

AURORA (ADULT)
University of Colorado
Clinical Director: Vera Fridman, MD
Appts: 720-848-2080

AURORA (PEDIATRIC)
Children's Hospital Colorado
Clinical Director: Michele Yang, MD
Appts: Alison Ballard, 720-777-3907

CONNECTICUT

FARMINGTON (PEDIATRIC)
Connecticut Children's Medical Center, Farmington*
Clinical Director: Gyula Acsadi, MD, PhD
Appts: Nanci Stolgitis, RN, 860-837-7500

NEW BRITAIN (ADULT & PEDIATRIC)
Hospital for Special Care
Clinical Director: Kevin J. Felice, DO
Appts: Sharon McDermott, 860-612-6305

FLORIDA

ORLANDO (PEDIATRIC)
Nemours Children's Hospital*
Clinical Director: Migvis Monduy, MD
Clinic Coordinator: Kelly Wydronkowski
Appts: 407-650-7715

GAINESVILLE (ADULT & PEDIATRIC)
University of Florida
Clinical Director:
James Wymer, MD, PhD
Appts: 352-294-5400

JACKSONVILLE (ADULT)
Mayo Clinic Florida
Clinical Director: Christopher Lamb, MD
Appts: 904-953-0853

MIAMI (ADULT)
University of Miami*
Clinical Director: Mario Saporta, MD
Appts: 305-243-9173

ILLINOIS

CHICAGO (ADULT & PEDIATRIC)
Northwestern Memorial Hospital
Clinical Director: Daniela Maria Menichella, MD, PhD
Appts: 312-695-7950

University of Illinois at Chicago
Clinical Director:
Charles K. Abrams, MD, PhD
Appts: 312-996-4780

Rush University
Clinical Director: Ryan D. Jacobson, MD
Appts: Sherri Taylor-Kennedy, 312-942-4500

O'FALLON (ADULT AND PEDIATRIC)
HSHS St. Elizabeth's Hospital
Clinical Director:
Raghav Govindarajan, MD
Appts: 618-641-5803

IOWA

IOWA CITY (ADULT & PEDIATRIC)
University of Iowa Hospitals & Clinics*
Clinical Director: Michael E. Shy, MD
Appts: 319-384-6362

KENTUCKY

LOUISVILLE (ADULT)
University of Louisville
Clinical Director:
Zeng Y. Wang, MD, PhD
Appts: Anson Ashburn, 502-588-4800

MARYLAND

BALTIMORE (ADULT & PEDIATRIC)
Johns Hopkins University*
Clinical Director: Thomas Lloyd, MD
Appts: (Adult) 410-955-2227, (Pediatric) 410-955-4259

MASSACHUSETTS

BOSTON (ADULT & PEDIATRIC)
Massachusetts General Hospital
Clinical Director:
Reza Seyedsadjadi, MD
Appts: Tamika Scott, 617-726-3642

MICHIGAN

DETROIT (ADULT)
Wayne State University School of Medicine and Detroit Medical Center*
Clinical Director: Jun Li, MD, PhD
Appts: 313-745-4275

ANN ARBOR (ADULT & PEDIATRIC)
University of Michigan
Clinical Director: Dustin Nowacek, MD
Appts: 734-936-9010

MINNESOTA

MAPLE GROVE (ADULT)
University of Minnesota*
Clinical Director: David Walk, MD
Appts: 763-898-1000

MISSOURI

ST. LOUIS (ADULT & PEDIATRIC)
Washington University School of Medicine
Clinical Director: Stefanie Geisler, MD
Appts: 314-362-6981

NEW JERSEY

HACKENSACK (ADULT & PEDIATRIC)
Hackensack University Medical Center
Clinical Director:
Florian Thomas, MD, PhD
Appts: Annerys Santos, 551-996-1324

NEW YORK

ROCHESTER (ADULT & PEDIATRIC)
University of Rochester*
Clinical Director: David Herrmann, MD
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NORTH CAROLINA

CHAPEL HILL (ADULT & PEDIATRIC)
University of North Carolina
Clinical Director: Rebecca Traub, MD
Appts: 984-974-4401

CHARLOTTE (ADULT & PEDIATRIC)
Atrium Health Neurosciences Institute
Clinical Director: Urvi Desai, MD
Appts: 704-446-1900

OHIO

COLUMBUS (ADULT & PEDIATRIC)
Nationwide Children's Hospital
Clinical Director:
Zarife Sahenk, MD, PhD
Appts: 614-722-2203

COLUMBUS (ADULT & PEDIATRIC)
Ohio State University, Wexner Medical Center*
Clinical Director:
Clinical Director: Michael Isfort, MD
Appts: 614-293-4969

PENNSYLVANIA

PHILADELPHIA (ADULT)
Hospital of the University of Pennsylvania*
Clinical Director: Steven Scherer, MD, PhD
Scheduling for Clinic Visits:
Shana Millner, 215-662-3606
Scheduling for Research Visits:
Dragan Vujovic, 215-898-0180

PHILADELPHIA (PEDIATRIC)
Hospital of the University of Pennsylvania*
Clinical Director: Sabrina Yum, MD
Appts: Hannah Borger, 215-590-1719

PITTSBURGH (ADULT)
University of Pittsburgh Medical Center (UPMC)
Clinical Director: Sasha Zivkovic, MD
Appts: 412-692-4917

PITTSBURGH (PEDIATRIC)
Children's Hospital of Pittsburgh
Clinical Director:
Hodas Abdel-Hamid, MD
Appts: 412-692-6106

TEXAS

AUSTIN (ADULT)
Austin Neuromuscular Center
Clinical Director: Yessar Hussain, MD
Appts: 512-920-0140

DALLAS (PEDIATRIC)
University of Texas Southwestern
Clinical Directors: Diana Castro, MD, and Susan Iannaccone, MD
Appts: Lindsay Adkins, 214-456-2768

BEDFORD (ADULT 16+)
Kane Hall Barry Neurology
Clinical Director:
Sharique Ansari, MD, MPH
Appts: 817-267-6290, option 4

UTAH

SALT LAKE CITY (ADULT & PEDIATRIC)
University of Utah*
Clinical Director:
Russell Butterfield, MD, PhD
Appts: 801-585-7575

WASHINGTON

SEATTLE (ADULT)
University of Washington Medical Center*
Clinical Director: Michael Weiss, MD
Appts: Gail Schessler, 206-598-7688

WASHINGTON, DC

(PEDIATRIC TO AGE 21)
Children's National Hospital
Clinical Director:
Diana Bharucha-Goebel, MD
Appts: Kathleen Smart, 202-476-6193

INTERNATIONAL LOCATIONS:

AUSTRALIA

WESTMEAD (PEDIATRIC)
The Children's Hospital at Westmead*
Clinical Director: Manoj Menezes, MD
Research Director: Joshua Burns, PhD
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BELGIUM

B-2650 EDEGEM (ADULT & PEDIATRIC)
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ITALY

MILAN (ADULT & PEDIATRIC)
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*** Denotes the CMTA Centers of Excellence that are included in the National Institutes of Health Inherited Neuropathy Consortium (INC). The INC is co-sponsored by the CMTA and the MDA. Worldwide more than 10,000 patients with CMT have been enrolled in protocols, and their data, de-identified to protect patient privacy, is housed in a common repository. As a result of this collaboration, a new CMT evaluation scale for children has been established, along with a new evaluation system for adults, and an infrastructure has been developed to perform natural history studies and clinical trials for CMT.**

CMTA BRANCHES

ALL CMTA BRANCHES CAN BE ACCESSED ONLINE AT www.cmtausa.org/branches

ALABAMA
Northern Alabama
Kimberly Parry
757-235-6260

ALASKA
Anchorage Area
Lisa Hubert
907-223-4566

ARIZONA
Phoenix Area
Pam Palmer
480-236-2445
Christina Fisher
623-742-8921

CALIFORNIA
Antelope Valley Area
Donna Murphy
661-317-6332
Danielle Metzger
661-317-6533
Los Angeles Area
Alani Price
310-710-2376
John Ramos
951-318-5669
Orange County Area
Beth Dorin
949-929-2908
San Diego Area
Kendall Trout
760-632-5654
San Fran/Bay Area
Leader Needed—Call:
800-606-2682

COLORADO
Denver Area
Ron Plageman
303-929-9647

CONNECTICUT
Hartford
Roy Behlke
239-682-6785
Diane Lindsay
860-670-4417
North Haven
Lynne Krupa
203-288-6673

DISTRICT OF COLUMBIA
Washington, DC
Steven Weiss
Kimberly Hughes
301-962-8885

FLORIDA
Central Florida
Leader Needed—Call:
800-606-2682
Destin Area
Ted Spring
850-368-1097
Jacksonville Area
Tim Nightingale
904-504-1953
Stephanie Burkhalter
904-710-3771
Naples
Roy Behlke
239-682-6785
Bill Millar
202-309-5685
Sarasota Area
Rachel Rivlin
941-284-0766
Tampa Bay Area
Edward Linde
813-712-4101
Sarah Gentry
941-706-5348

GEORGIA
Atlanta Area
Jeannie Zibrida
404-307-6519

HAWAII
Honolulu Area
James Cuizon
808-450-1236

IOWA
Iowa City Area
Jeffrey Megown
319-981-0171

ILLINOIS
Chicago Area
Doreen Pomykala
815-351-1328

INDIANA
Fort Wayne Area
Aimee Trammell
574-304-0968
Indianapolis Area
Leader Needed—Call:
800-606-2682

KANSAS
Kansas City Area
Tammy Adkins
314-608-6889
Aron Taylor
913-744-5674

LOUISIANA
Baton Rouge Area
Leader Needed—Call:
800-606-2682

MAINE
Portland Area
Mary Louie
207-450-5679

MARYLAND
Baltimore
Sarah Kaider
301-615-9589

MASSACHUSETTS
Boston
Mimi Works
617-913-4600
Jill Ricci
978-887-1014

MICHIGAN
Central Michigan Area
Megan Berger
517-256-5854
Jonah Berger
303-827-4218

MISSOURI
Kansas City Area
Tammy Adkins
314-608-6889
Aron Taylor
913-744-5674
St. Louis Area
Payton Rule
618-401-4822
Amanda Rule
618-698-3039
Springfield Area
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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 (ddI, 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.