

THE

CMTA Report

WINTER 2020

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BE THE CHANGE

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

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THE **CMTA** Report

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A MESSAGE FROM THE CEO



DEAR FRIENDS,

The New Year is always a time for taking stock. It's also a good time for making changes, and this year it puts us in mind of the great Mahatma Gandhi, who said, "Be the change you want to see in the world." The change we want to see in the world is, of course, the complete and total eradication of CMT. It's what inspires us, drives us and unites us.

At the individual level, change takes many different forms, some of which we're featuring in this issue of *The CMTA Report*. Change might take the form of new, healthier habits and certainly we applaud those. If you're capable, the exercise program that Board Member Steve O'Donnell developed specifically for people with CMT is a great way to start the New Year.

Change can also take the form of an attitude adjustment, and we're also spotlighting two individuals who changed their lives after changing their attitudes. Jamal Hill, the #1 Paralympic swimmer in the United States in the 50-meter freestyle, recounts his road to the 2020 Paralympics in Tokyo and how it began with a change in attitude. Jamal struggled for years with the secret of his CMT1X and says that only when he stopped treating it like a curse did the "blessings and opportunities start flowing."

Like Jamal, Julie Stone was in denial about her CMT for most of her life—until she attended a CMTA Patient/Family Conference in Seattle in 2018. It changed her world, she says, breaking down her own stereotypes about what people with disabilities are like. In a heartbeat, she changed from a CMT denier to a CMT "defier."

The CMTA is changing, too. We added new members to our CMTA Board of Directors and CMTA Advisory Board in 2019, and we fully anticipate that they will help us be the change we want to see. Another person who is helping us change is Mark Scheideler, the expert guiding the CMTA's drug discovery process. You can read about his achievements in his profile on p. 8.

Because technology is ever-changing, we also bring you news in this issue about potentially game-changing new research in gene editing being funded by the CMTA.

We're excited about the changes that the drug discovery process will bring in 2020. We wish you the happiest New Year and a change for the better.

All my best,

A handwritten signature in black ink that reads "Amy Gray". The signature is fluid and cursive.

AMY GRAY, Chief Executive Officer

SWIMMING UPHILL WITH JAMAL HILL

BY CLARK SEMMES

JAMAL HILL is currently the #1 ranked Paralympic swimmer in the United States in the 50-meter freestyle, but athletic achievement did not come easily to the California native.

He struggled for years with the secret of his CMT1X and says that only when he stopped treating it like a curse did the “blessings and opportunities start flowing.”

Born in the Los Angeles neighborhood of Inglewood, Jamal started swimming at the age of 7, before his CMT became symptomatic. At the age of 10, he dislocated his shoulder, which was so damaged that doctors considered amputating the arm. That was followed by a bout of flu that left him paralyzed for a short time, able only to speak and nod. His family assumed his swimming days were over.

Although Hill eventually regained his mobility, his parents and other family members decided to keep quiet about his CMT, not wanting to alter his perception of what was within his reach. His parents acknowledged his CMT, but didn't allow him to use it as an excuse or a crutch.

Jamal eventually recovered and got back into swimming his sophomore year at Junipero Serra High School in Gardena, California. During his sophomore and junior years, he only swam three months per year and did not excel. In the fall of his senior year, he joined a club team and trained really hard, earning a first place in his division.



Post high-school, Jamal earned a small athletic scholarship to Hiram College, a Division III school in Ohio. His coach was a 70-year-old Midwesterner, Jamal was a young black kid from Los Angeles and the two were unable to bridge the gap between them. Jamal performed so poorly his junior year that he decided to look elsewhere for a coach.

He found Dave Salo at the University of Southern California (USC). He called Dave in February of his junior year and said he was leaving Hiram to swim at USC. Jamal returned to California and joined the USC swim team—the Trojan Elite.

After a year without much improvement in his swim times, Jamal decided he needed a personal coach. He found mental performance coach and swim consultant Wilma Wong through another swimmer. She quickly noticed that Jamal's dives needed



PARALYMPIC ATHLETE



Follow Jamal at
www.swimuphill.com/landing
or on Instagram at @SWIMUPHILL.

improvement because his legs weren't functioning. Jamal finally began talking openly about his CMT and now says he wishes he'd started sooner. "When we confront the things that challenge us, we find opportunities to grow," he says.

Wong told Jamal that unless a cure for CMT could be found before the 2020 Olympics, he should consider the Paralympics. For Jamal, making the switch was an emotional journey with many tears and a lot of prayers. He began competing in the Paralympics in fall 2018 and quickly rose to #13 in the world in the 50-meter freestyle. The experience forced him to reconsider some of his preconceived notions. He realized that there was nothing to be ashamed of, that the Paralympics are just as competitive as the Olympics and that Paralympic athletes are just as dedicated and driven as other athletes.

Jamal's goal is to win four gold medals at the Tokyo Paralympic Games in 2020. He also hopes to be a contender for Paris 2024 and Los Angeles 2028, when the Paralympic Games will be held in his own backyard. "It all comes back to legacy," Hill said. "I think that's what my life is about—trying to do something that will outlast me."

Hill is also working towards a much larger goal: He wants to teach 1 million people to swim. According to his website, roughly 360,000 people lost their lives to drowning in 2016, and drowning ranks fifth on the Centers for Disease Control's list of the causes of unintentional injury or death. He founded Swim Up Hill in an attempt to lower the global drowning rate. "My mission is to have an impact. I want to be more than just an athlete," the 24-year-old says. "In order to build something that will outlast

me, I have to get out there and help people. I want to be more in the teaching lane instead of just the entertaining lane."

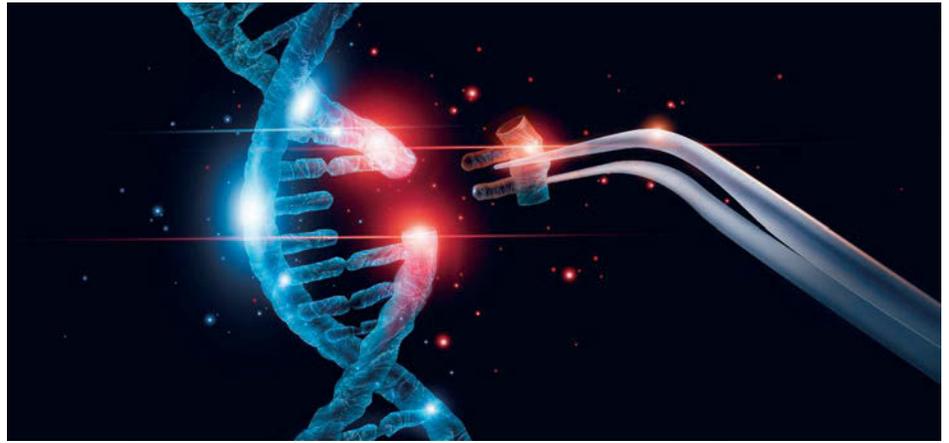
Jamal estimates that he has already taught hundreds of people in his community to swim.

"You'd be amazed at how many people have this distinct fear of the water," Hill said. "I would even count a success as someone growing comfortable enough to take a swim lesson with me."

By filming video tutorials, conducting outreach with Boys & Girls Clubs and growing his personal brand on social media, Hill has already begun to reach people outside of his hometown. "Chances are, I may not personally teach that many people to swim," Hill said. "But the beauty of the time that we live in is that this technology gives us the ability to reach the whole world." ★

"When we confront the things that challenge us, we find opportunities to grow."

CMTA Funds Cutting-Edge Gene Editing Studies for Type 2 CMT



Drs. Bruce Conklin and Luke Judge of the Gladstone Institutes and UCSF Departments of Medicine and Pediatrics will use a \$653,000 grant from the CMTA in 2020 to develop the gene-editing technique known as CRISPR for application to CMT2A, 2E and 2F.

Gene editing is a group of technologies that give researchers and scientists the ability to modify DNA. Genetic material can be added, removed or altered at certain locations in the genome, much like cutting and pasting information in a Word document. Methods to modify DNA in the genome have been around for more than 30 years, but CRISPR technology has brought major improvements in the speed, cost, accuracy and efficiency of gene editing.

Drs. Conklin and Judge, leaders in genome editing, and their team (www.ConklinLab.org) have already made substantial progress in testing CRISPR in stem cell models of CMT2A and CMT2E.

The San Francisco Bay area is at the forefront of technology development in the field: In 2012, Dr. Jennifer Doudna and her team at Berkeley discovered how CRISPR could be used for DNA editing. Dr. Doudna now directs the Innovative Genomics Institute (IGI), where Dr. Conklin serves as deputy director (<https://innovativegenomics.org/>). The newly funded project will focus on developing the pre-clinical tools and preliminary data needed to take the next steps for

eventually testing CRISPR therapies in human clinical trials.

ment of research tools and potential disease treatments. Editing the genomes of rodents and other organisms that scientists commonly study has brought forward numerous discoveries about how

the genome is connected to physical traits like eye color and diseases like CMT.

CRISPR can make deletions in the genome and/or be engineered to insert new DNA sequences. The CRISPR system was adapted from a naturally occurring gene editing systems

in bacteria. The bacteria capture snippets of DNA from invading viruses and use them to create DNA segments known as CRISPR arrays. The CRISPR arrays allow the bacteria to “remember” the viruses so that if they attack again, the bacteria can target their DNA. Remarkably, this bacterial defense system works in human cells to edit DNA and perhaps treat genetic diseases.

While many leading scientists believe that the use of CRISPR in clinical trials is still years away, the studies have the potential to advance this technology for CMT and set a new standard for therapeutic engineering for motor neuron disease that can be extended to other neurological diseases. ★

The studies have the potential to advance gene editing technology for CMT.



The Conklin Lab Team

NIH AWARDS INHERITED NEUROPATHIES CONSORTIUM \$7.2 MILLION IN RENEWED FUNDING FOR WORK ON CMT

The National Institutes of Health granted the Inherited Neuropathies Consortium (INC) \$7.2 million in renewed funding in October 2019 for continued clinical research on different forms of inherited peripheral neuropathies and improving the care of patients.

Led by University of Iowa neurologist and CMTA Board Member Michael Shy, MD, the INC is part of the Rare Diseases Clinical Research Network (RDCRN), a group of 20 teams of scientists, clinicians, patients, families and patient advocates that study a wide range of rare diseases. The RDCRN is supported by multiple NIH Institutes and Centers



CMT Board Member Dr. Michael Shy leads the Inherited Neuropathies Consortium (INC), which is part of the Rare Diseases Clinical Research Network

and led by NIH's National Center for Advancing Translational Sciences (NCATS) and the NCATS Office of Rare Diseases Research. The RDCRN grants aim to foster collaborative research among scientists to better understand how rare diseases progress and to develop improved approaches for diagnosis and treatment.

According to Shy, professor of neurology and director of the CMT Clinic at the University of Iowa Hospitals & Clinics, the funding means that Iowa will remain at the forefront of research in the field of inherited peripheral neuropathies. "We will continue to pioneer natural history studies and develop clinical and biomarker outcome instruments for patients with multiple genetic



forms of inherited peripheral neuropathies. We will also be able to continue our efforts to identify novel genetic causes of CMT and continue to train the next generation of young investigators in our field."

In addition to seeking new and better treatments for patients with inherited neuropathies, the consortium also provides up-to-date information to help patients manage their diseases and assists in connecting patients with support groups, expert doctors and clinical research opportunities.

While INC is primarily funded by NIH, the CMTA and the Muscular Dystrophy Association also provide support. ★

CMTA WELCOMES NEW BOARD MEMBER

Banker **David Coldiron** joined the CMTA Board of Directors in 2019, bringing almost two decades of business experience with him. Currently the director of mortgage lending at Legends Bank, Dave and his wife, Christina, live in Nashville with their two daughters.

"Our daughter, Hazel, was diagnosed with CMT in November 2015, Coldiron said, adding, "Christina and I were so fortunate to find the CMTA, which has provided us with medical contacts and welcomed us into their caring community. It is an honor to join a team that is doing so much to support those impacted by CMT as well as leading the charge for development of treatments."

CMTA Board Chair Gilles Bouchard said of Coldiron's appointment, "We are all looking forward to working with Dave on behalf of all CMT patients during these exciting times for our organization."



GAME-CHANGER PROFILE: Drug Therapy Expert Mark Scheideler

BY CLARK SEMMES

As an endurance athlete, Mark Scheideler—the chairman of the CMTA’s Therapy Expert Board (TEB)—sees the race for a cure for CMT not as a sprint but as a marathon. And as Mark guides the CMTA toward the finish line at a record-setting pace, one thing is certain: This is a race that Mark intends not just to finish but to win.

As head of the TEB, Mark guides the formation of therapy development partnerships that bridge efforts by the CMTA’s STAR (Strategy to Accelerate Research) network, pharmaceutical companies and specialty contract

research organizations. The TEB also evaluates the “translational” merit of proposed and ongoing research projects for how directly they advance CMT therapies.

Based on his decades of drug discovery and development experience, Mark believes there is a high probability of finding an approach to treat or solve CMT in the next few years. He says the CMTA is currently in the “sweet spot” between the identification of a disease cause and the discovery of an effective treatment or cure. This is due, he says, to the CMTA’s strategic alliances with both large and small pharmaceutical companies, which allow it to marry the leading-edge scientific know-how of STAR with the multi-disciplinary expertise and approach those companies bring to drug development. According to Mark, it’s a “unique approach among disease-oriented non-profits that we have effectively implemented, and one that will hopefully pay off big in the years to come.”

Mark earned a BA in biochemistry and molecular biology from Northwestern University in Chicago and a PhD in biochemistry from the University of Chicago. He was then a biochemistry research fellow at the Duke University School of Medicine, and later went back to school to earn a certificate in finance and accounting from The Wharton School at the University of Pennsylvania.

Leaving Duke, Mark began a five-year stint as a research assistant professor at the Albert Einstein College of Medicine in New York, working on studies sponsored by the National Institutes of Health (NIH) aimed at

explaining the structural requirements for receptor and ion channel function. These are hugely important classes of drug targets in the nervous system, and this work reflected his developing interest in learning how to directly manipulate them when it appeared they were involved in a disease process.

He then spent more than a dozen years out of the country. After receiving a career and life-changing offer to join a world-class drug discovery organization, he moved to Copenhagen, Denmark, to work for Novo Nordisk. He headed its cellular and biochemical pharmacology team, working with projects uniquely partnered in alliances with other pharma companies to design new drugs for nervous system disorders. At this point, the former high school athlete had to give up running due to wear and tear on his knees. He pivoted to bicycling and enjoyed exploring the country on two wheels, a sports interest he maintains to this day, along with sculling regularly on the Potomac.

Mark later received another dream job offer, this time as the head of neurobiology research at SmithKline Beecham (now GSK), directing its unit based in Milan, Italy. Leveraging internal company alliances across Europe and the United States, he started the company’s first clinical development project in neuropathic pain and its entry into pain therapy as a major company effort. He also guided drug discovery and pre-clinical research across four therapy areas, leading to multiple drug candidates entering clinical development. He also learned to love Italian food, particularly dishes incorporating the local deli-



Mark Scheideler leads the CMTA's Therapy Expert Board

NEW SCIENTIFIC ADVISORY BOARD MEMBER

The CMTA welcomed **Robert Burgess, PhD**, to its Scientific Advisory Board in 2019. Burgess received his BS in biochemistry from Michigan State University (1990) and his PhD in neuroscience from Stanford University (1996). After a post-doctoral fellowship at Washington University in St. Louis, Dr. Burgess took a faculty position at The Jackson Laboratory in Bar Harbor in 2001 and is now a full professor there.

The Burgess Lab's research seeks to understand the molecular mechanisms of synapse formation and maintenance at two sites in the nervous system: the peripheral neuromuscular junction and the retina. Its studies address basic molecular mechanisms relevant to human neuromuscular and neurodevelopmental disorders. The lab is increasingly using the mouse models it generated to test therapeutic strategies in preclinical studies, especially related to gene therapy approaches, with the goal of translating these findings to patients.

Dr. Burgess is on the scientific advisory boards of the Hereditary Neuropathy Foundation and the Talia Duff Foundation, and he is the chair of the NIH study section for Cellular and Molecular Biology of Neurodegeneration. He is also the director of the NIH-funded Center for Precision Genetics, the director of the post-doctoral training program in Bar Harbor, and the director for the cooperating PhD program in neuroscience with Tufts University.



cies of Piemonte, his favorite part of Italy.

Mark returned to Denmark in the early 2000s to serve as managing director-Europe and senior vice president of MDS

Proteomics, a company headquartered in Toronto, Canada. He was

responsible for biological, analytical and informatic operations at the Danish site of this

leading proteomic company, and for its alliance with Abgenix aimed at co-developing therapeutic antibodies.

Returning to the United States, Mark accepted a position as senior scientific officer at the NIH, where he developed and directed trans-NIH roadmap initiatives. These included an effort to create technologies and projects for use by the Molecular Libraries Consortium of High-Throughput

STAR is "a unique approach among disease-oriented nonprofits ... and one that will hopefully pay off big in the years to come."

Screening Centers and the founding of a public-private partnering effort across its Clinical and Translational Science Awards (CTSA)

consortium of 60 clinical research centers, which succeeded in aggregating and marketing their intellectual property via the web tool CTSA-IP.

Both efforts garnered NIH Director's Awards for Mark. While at

the NIH, he was asked to advise visiting CMTA board members on a project to screen large libraries of small molecules. He introduced them to Jim Inglese at the NIH screening center, who eventually became a collaborator on a large-scale CMTA screening project with Sanofi-Genzyme.

Mark founded HumanFirst Therapeutics LLC in 2011 with the goal of identifying therapy development projects and then

assembling the teams, funding and management needed to advance them to the clinic. On learning that Mark had left the NIH to set up a consultancy, the CMTA invited him to present a strategic plan to the board to expand its STAR consortium into alliance work aimed at therapy development. The Sanofi-Genzyme and Ionis Pharmaceutical relationships were the first to come out of that planning.

The CMTA, Mark's biggest client, has partnered with more than 20 of the world's best pharmaceutical companies and contract service organizations in search of treatments and ultimately a cure for CMT. Clinical trials are already underway on several treatments, and efforts now span a host of approaches, including the use of small molecule drugs, biologicals and gene therapy. Mark's marathon quest for a treatment or cure for CMT continues unabated and the finish line gets closer every day. ★

ADAPT, THEN SKI

HOW SARAH KESTY TOOK ON THE SLOPES



Adaptive skiers on slopes in Durango

No one ever told me I couldn't ski, but it was an unspoken understanding, a composite of other phrases that limited my concept of my body's abilities. My doctor said, "Just limit all of your activities if you don't want pain." Teachers counted me out of games without even asking if I felt up for playing. Even friends' well-intended accommodations supported my boxed-in view of myself—being invited to go on a ski trip and be the "stuff watcher" because skiing, like lots of other activities, seemed to be out of the question for someone with CMT.

A few years ago, I tried to challenge my self-concept. I attached long, slippery planks to my feet and stepped out onto slushy spring snow. It was not a pretty experience. I realized that the trickiest part of skiing for me—besides admitting that I needed two different-sized boots for my different-sized feet, besides asking for extra help during the lesson and besides getting out of my head to ignore the inner critic—was figuring out how to stop. I could slide down the smallest of bunny hills, but stopping

Editor's Note: CMTA Advisory Board members Sarah Kesty and Greg Stillwell got together in Durango, Colorado, last winter for what turned out to be a life-changing experience. Sarah not only learned to ride a ski bike, but Greg and Missy Thompson analyzed her gait, showing her that it is possible to change the way one walks, even with CMT.

was incredibly hard. It took a lower leg strength that I just didn't have. And the other form of braking, falling on my butt, was embarrassing and painful. The experience was exactly what I'd feared, and as hard as I tried, it reinforced my self-doubt and shrank my world.

The experience left me flip-pant and resistant, so when I heard about the adaptive ski program at Adaptive Sports Association (ASA) Durango, at first I didn't want to risk a rerun. I was worried I would be talked down to as the recipient of help through an adaptive program. I usually position myself on the helping end of things, and I dreaded being on the receiving end of a clinical or demeaning tone.

My worries were unfounded. ASA's approach supported my physical and emotional needs with respect and dignity. I tried a ski bike, which allowed me to sit on a bike with very short skis on my feet. The stopping part that had eluded my first skiing attempts was much more accessible on the bike: you turn uphill or dig in the side of your front ski, and you slow down. Having the right equipment and some amazing instructors was really all I needed. The few times I fell, spectacularly, into the snow were the times when I was inside my own head, judging and doubting. When I went with it, when I listened to my body and the movement of the mountain, I hit my flow. I'd

always seen skiing as a representation of freedom. It was that and so much more.

No one ever said to me, "Sarah, you can do anything," but it doesn't always need to be said. Sometimes the right help and just another chance are all a person needs.

—Sarah Kesty

THE RIGHT HELP

In March 2019, the Adaptive Sports Association-Durango and I welcomed Sarah Kesty and Jennifer Rushall to Durango for three days of learning to ski-bike and testing in our gait lab at Fort Lewis College.

The snow conditions were ideal, and allowed the students to learn grace and ease on their ski bikes, overcoming their fears and feeling more confident that winter sports CAN be done by people with CMT.

Sarah and Jennifer also helped Dr. Missy Thompson and I to add to our data on gait and pressure changes with CMT. Our hope is to create a more uniform and objective measurement methodology for centers worldwide that evaluate and treat CMT, allowing us to truly assess the effectiveness of various treatments. This could also be used in conjunction with the MRI evaluation of calf muscles, which is becoming more common in some Centers of Excellence.

We would love to work with the CMTA to continue and expand this program in the future! As a side note, Sarah also advises that the ulceration on the bottom of her right foot healed after several weeks of wearing the Stand Strong® adhesive arch support to relieve pressure under the foot.

—Greg Stilwell

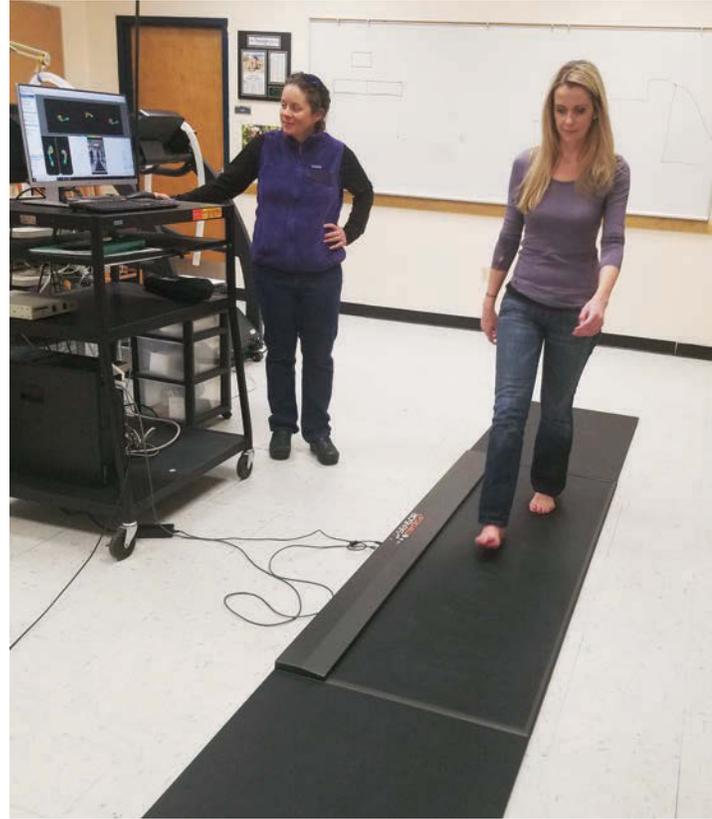
GAIT ANALYSIS REVEALS ISSUES, ANSWERS

A plantar pressure analysis was conducted with Mrs. Sarah Kesty at the Durango Performance Center located at Fort Lewis College. The pressure analysis showed that when walking barefoot Mrs. Kesty's left foot exhibited a good distribution of pressure between the forefoot and rear foot, but that there was little engagement of the toes. The right foot exhibited high rear foot pressure that extended along the lateral column of the right foot, very high pressures on the right first metatarsal head (ball of the foot)

and no engagement of the toes. The patient was healing from an ulcer at the head of the first metatarsal at the time of this analysis and the high pressure observed in this area was likely the cause.

We also evaluated plantar pressure with Mrs. Kesty walking barefoot while wearing the Hozhoni Balance Rail, which is an orthotic device that adheres to the bottom of the foot and hence can be worn both while barefoot and wearing shoes. With use of the Hozhoni Balance Rail, Mrs. Kesty exhibited substantially reduced plantar pressure under the first metatarsal head and decreased pressure along the lateral column of the right foot. This indicated that the Hozhoni Balance Rail was successful at offloading the first metatarsal head.

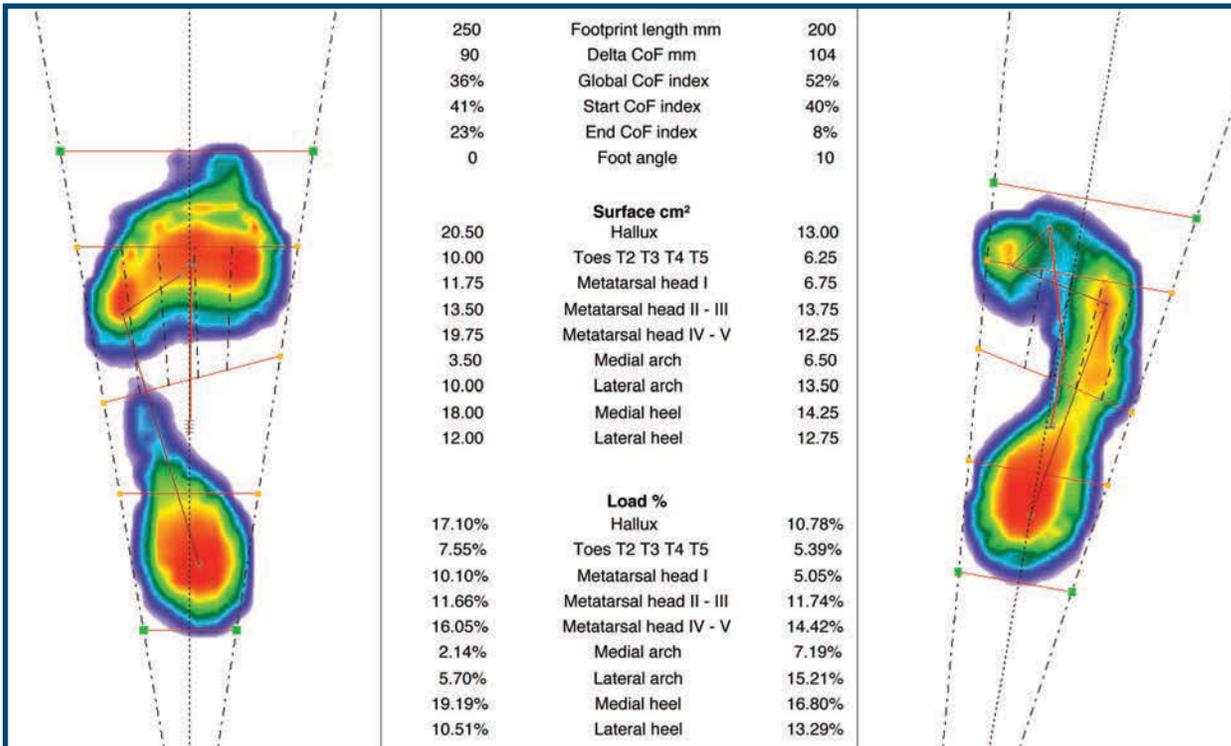
—Missy Thompson, PhD



Testing and results of gait analysis

ABOUT THE CONTRIBUTORS:

CMTA Advisory Board member Sarah Kesty is an educator, speaker and author who helps families navigate school support systems. As detailed in the Fall 2019 issue of The CMTA Report, she received a grant from the Challenged Athletes Foundation to purchase a ski-bike. Greg Stilwell, DPM, is a board certified podiatrist diagnosed with CMT1A. An inventor and a patent holder of the Hozhoni Balance Rail®, he lectures internationally on foot and ankle topics, including CMT, and is a member of the CMTA Advisory Board. Missy Thompson is an assistant professor in the Health Sciences Department at Fort Lewis College in Durango.





WALK THIS WAY:

Volunteers Step Up to Make a Difference



The CMTA's Walk 4 CMT campaign has grown exponentially since the first walk took place 11 years ago.

In 2019, the CMTA's amazing volunteers stepped up to organize walks in 32 cities in 22 states and two countries, raising \$332,500 for STAR research.

The CMTA is one of the few national non-profit organizations with a walk campaign organized and led solely by volunteers. These leaders are not only personally making a difference, they are giving others the opportunity to join a community and reach out to their networks to share their CMT stories, spread awareness about CMT and raise funds for research.

Any city or town can host a Walk 4 CMT—all it takes is one



leader to step forward. The CMTA makes it simple to host a walk with a Walk4CMT.org website, turnkey templates for sponsorship and marketing and

support from our dedicated staff. Boston Branch Leader Jill Ricci, who also organized the Boston Walk 4 CMT, said, "The CMTA makes it very easy... providing t-shirts and goodies for everyone to enjoy."

Walk 4 CMT events are not just fundraisers, they empower local CMT communities to be a part of something bigger—moving CMT research forward. Jill affirms, "A Walk 4 CMT gives everyone a chance to be part of a bigger picture—to have a hand in helping in the fight to END CMT." ★

2019 WALK 4 CMT HIGHLIGHTS

HIGHEST ATTENDANCE AT A WALK 4 CMT: 311 people at the Parkland, Florida Walk 4 CMT

MOST RAISED BY A SINGLE WALK 4 CMT: \$41,061 by Washington, D.C. Walk 4 CMT

MOST RAISED BY A WALK 4 CMT TEAM: \$11,536 by Team Rustici

TOP FUNDRAISER: Molly Fernandes (\$9,023)

8 NEW WALK 4 CMT EVENTS: San Diego, CA; Denver, CO; Miami, FL; Long Island, NY; Syracuse, NY; Columbus, OH; Greenville, SC; Houston, TX

STATES WITH MOST WALKS: Florida and California, with three each

1,836 PARTICIPANTS NATIONWIDE ★ 22 WALKS RAISED OVER \$5,000 ★ 14 WALKS RAISED OVER \$10,000



Parkland, FL
Walk 4 CMT

THE CMTA THANKS ALL OF OUR TRULY AMAZING WALK 4 CMT LEADERS!

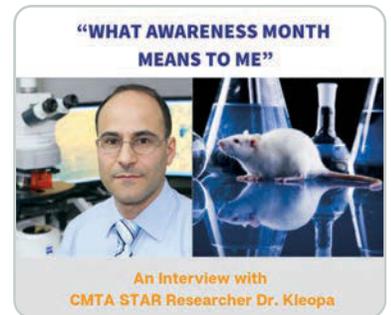
- ARIZONA.....*Tucson:* Kristen Oaxaca
- CALIFORNIA.....*Los Angeles:* Alani Price
Palo Alto: Ori Bash & Tau O’Sullivan
San Diego: Kendall Trout
- COLORADO*Denver:* Carol Ris
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AWARENESS MONTH OPENS EYES WITH “SEE CMT!” CAMPAIGN

CMT Awareness Month was an exciting and memorable celebration of community in 2019, as well as a successful educational event that made a big impact on the CMTA’s social networks and beyond.

The 2019 theme was “See CMT!” illustrated with a pair of orange CMTA glasses symbolizing that many of the symptoms and challenges that come with having CMT are invisible from the outside.

The glasses were featured in the Facebook frame supporters used to deck out their profile pictures and get in the Awareness Month spirit. During the month, the CMTA issued weekly challenges, including uploading the Facebook frame, sharing a “What is CMT?” infographic, participating in a Walk 4 CMT and supporting CMT research. Videos accompanying the challenges starred community members, and the CMTA website featured two interviews with community members who are actively raising awareness: Jess Diamond shared her experience as a walk leader and Dr. Kleopas Kleopa revealed what Awareness Month is like for a CMTA researcher.



Community members participated in all of the challenges and made a huge wave on social media. On Facebook alone, CMT Awareness Month posts received 37,686 hits and 3,448 shares. On average, each Awareness Month Facebook post of 2019 reached 9,123 people. The “What is CMT?” infographic posted on the CMTA Facebook page was shared 2,723 times!

Community members also started the “CMT Lego Challenge.” Like the ALS Ice Bucket Challenge, the CMT Lego Challenge involved nominating friends to walk across a floor covered with Lego pieces, simulating the neuropathic pain and balance difficulties that people with CMT experience. ★

The Surgical Correction of the CMT Foot: IS SURGERY RIGHT FOR YOU?

THE FIRST ARTICLE IN A FOUR-PART SERIES

BY GLENN B. PFEFFER, MD

My father was a general surgeon who performed huge and difficult operations. One day he had a simple mole removed from his face. “I get it now,” he explained to me when I was 13. “I know the difference between a big operation and a small one. It’s big when it’s on you.”

Decades later I too get it, having had an operation on my own foot 15 years ago. Surgery is scary, especially if it is elective and you are getting by okay. But okay should not be good enough if you can do better. No one should accept impairment as the new normal and that’s why I am writing this four-part column.

To start, we need to discuss what causes the CMT foot deformity—and I think it is fair to call it that in the more advanced cases—and how surgery can correct it.

Surgery is definitely not for everyone with CMT. Many people have little to no muscle function below the knee. For them, one of the many excellent ground-reaction braces is probably the best solution. An ankle fusion is rarely indicated.

Seventy percent of people with CMT have a cavovarus foot—a very high arch with an in-turned heel. The toes are often drawn up, and the inside of the foot is twisted downward. This

twisted shape makes walking very difficult, unbalanced and painful. It takes a lot of energy. These are the CMTers who will greatly benefit from surgery.

The underlying problem with the cavovarus foot is an imbalance of the muscles: Some are strong while others are weak; some of the nerves still work, while others are compromised. The strong muscles overpower the weaker ones, which causes the foot and toes to take an abnormal posture. If the problem



starts during childhood, the bones can develop with an abnormal shape. The longer the imbalance is in place, the worse the deformity becomes, which is why surgery should ideally be done early in life although it’s never too late—I once operated on a 78-year-old woman.

The goal of surgery is to balance the muscle pull, straighten the bones and bring the foot into a flat position. Even with people who need a brace, the foot has to be flat on the ground to function maximally.

The surgery is done on an out-patient basis with general anesthesia and a nerve block. I have operated on hundreds of people with CMT using a nerve block. The block provides pain relief for 48 hours or more after surgery. After that, pain meds may be needed for up to a week, sometimes two. A non-weight bearing cast is used for a total of six weeks. In the majority of cases, weight-bearing and physical therapy then begin in a removable cast boot.

Only one foot can be operated on at a time and complete recovery can take up to a year. Most people fix one foot and then the other a few months later. There is no question that it takes commitment and a strong support group.

There are four parts to surgical correction: The calcaneus (heel bone), the metatarsal bones in the middle of the foot, the soft tissues and muscles and the toes. They are all

addressed during the same surgery.

This series begins with a discussion of the heel, which has to be brought out of its in-turned position (varus) to one that is out-turned (valgus). The valgus position maximizes motion, creates a heel that is flat on the ground and balances weight-bearing. Everyone with a cavovarus foot knows the feeling of toppling over when walking. I imagine it feels like an impending ankle sprain with every step.

(continued on page 16)

Atlanta Patient/Family Conference— Standing Room Only

The CMTA's latest Patient/Family Conference, held in Atlanta on November 2, 2019, was virtually standing room only and featured a full agenda with CMT experts from across the country. CMTA CEO Amy Gray kicked off the program by introducing the Youth Council, whose members serve as the voice of youth within the CMTA community. The aim of the Youth Council is to connect as many CMT youth as possible. As one member stated, "Making connections with others the same age is a game changer."

CMTA Board Member Michael Shy, MD, head of the CMTA Center of Excellence at the University of Iowa, presented exciting news about clinical trials. New approaches to research, use of biomarkers and MRIs are getting us closer to finding success in changing the course of CMT, he told the audience. Shawna Feely, CGC, also from the CMTA Center of Excellence at the University of Iowa, educated the attendees about genetics. She referred to CMT as "one name, many diseases" and covered testing options as well as how CMT is diagnosed and passed down in families.

CMTA Board Chairman Gilles Bouchard gave a STAR research update that included drug development, gene therapy and biomarkers. He reviewed specific plans for CMT1A, CMT1X, CMT2A, CMT2E, CMT4C and undiagnosed CMT 2s. CMTA Board Member Elizabeth Ouellette presented "Staying Active: Move, Groove & Improve." Noting that CMT patients often feel differently than they appear, she suggested dealing with chronic

pain and fatigue through exercise, laughter and activity.

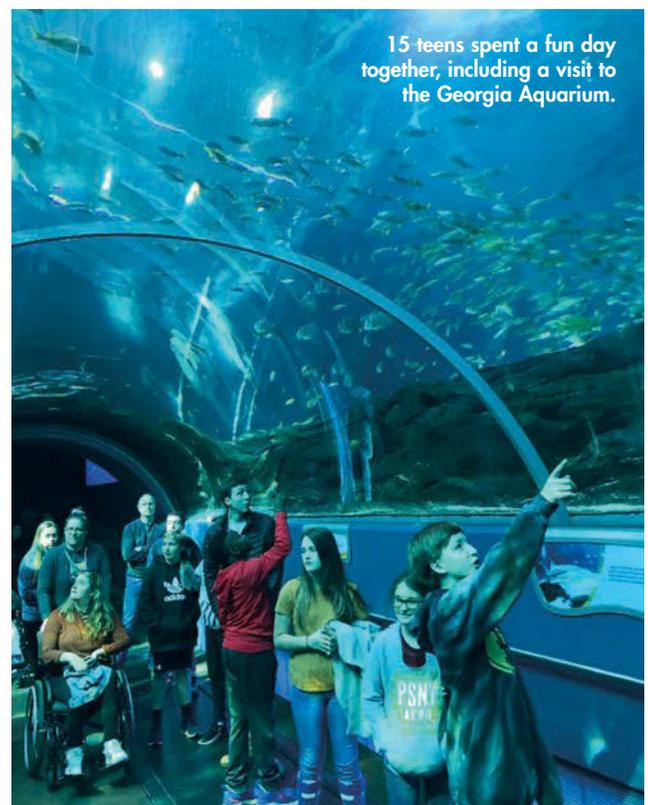
There were also presentations by several members of the CMTA Advisory Board. Pulmonary specialist Ashraf Elsayegh, MD, FCCP, from Cedars-Sinai Medical Center addressed pulmonary complications and management in CMT, including diaphragm dysfunction, scoliosis, vocal cord paralysis and sleep disorders. Glenn Pfeffer, MD, also from Cedars-Sinai Medical Center, reviewed foot surgery options. Observing that there are 20 muscles, 26 bones and 33 joints in the foot, Dr. Pfeffer said that walking a mile puts 100,000 pounds of stress on the feet of an individual without CMT. For someone with CMT, that same mile puts 1 million pounds of stress on the foot, he said, adding that surgery can eliminate bracing and future damage to the feet. Elizabeth Misener, PhD, LMSW, the co-founder of Vitality at Work, spoke about the challenges of coping with CMT, which affects three members of her immediate family. She emphasizes breathing, acknowledging and embracing imperfections and working on a growth mindset.

The program was completed by DeLana Finney, CPO, from Hanger Clinic and Rodney Vaden, Mid-South District Manager for Allard USA, who talked about braces and "finding the right tool for the job."

While the experts opined and parents mingled, a group of 15 teens embarked on a day of fun and friendship. Their first stop was the acclaimed Georgia Aquarium,



where the group spent two hours exploring. Over a pizza lunch, the group chatted about the best and worst parts of having CMT, connecting over similar struggles and emotions. Post-lunch, the teens headed to the Coca-Cola Museum, where they learned the history of Coke products from all over the world and had the opportunity to taste-test 57 of them. It was a wonderful day, proving again that when the CMTA brings together youth who understand CMT, fun and friendship result. ★



15 teens spent a fun day together, including a visit to the Georgia Aquarium.



NEW VIDEO SERIES SPOTLIGHTS EXERCISES FOR CMTERS

After attending the CMTA Physical Therapy Workshop in late 2017, CMTA Board Member Steve O'Donnell realized the tremendous need for a series of CMT physical therapy-centered exercise videos. An avid and committed workout enthusiast, Steve has seen first-hand the crucial role that fitness plays in slowing the progression of CMT symptoms.

Steve partnered with renowned physical therapist/neurologic clinical specialist Mike Studer to create a comprehensive fitness program for all levels and abilities, focusing on flexibility, balance, strength, endurance and function.

The videos are full of tips and information and can be done at different levels as one progresses. The balance video, for example, demonstrates how the brain can be retrained so that sensory cues can be picked up from some source other than the feet, which don't give CMT patients good information.

According to Steve, the most important thing is to start and keep going. "If you're tired, rest, but don't stop because if you continue to work at this, your life will change." The program "will enable you to do more than you think you can do," he added.



The videos focus on increasing flexibility, balance, strength, endurance and function. Watch the videos at www.cmtausa.org/pt-exercise.

FOOT SURGERY

(continued from page 14)

Correction of the heel deformity can be formidable. The classic way to correct the abnormal position is by cutting through the heel with a power saw (I know it sounds horrible but that is what foot and ankle orthopedics is all about) and shifting the weight-bearing part of the heel laterally (away from the other foot). The goal is to create a more balanced stance. With more severe in-turning we also take a wedge of bone out of the heel (shaped like a pie slice). This is called a Dwyer osteotomy and allows a further shift of the heel into a valgus position. The cut bone is held with two screws inside the bone that can't be felt and rarely have to be removed.

The problem is that the classic correction is often not enough. Several years ago, the CMTA gave me a research grant to address this difficult problem. We won a national award from the American Academy of Orthopedic Surgeons for research "most likely to change orthopedic care in the future." Using 3D modeling from a CMT patient, we showed that a bone cut, with a Dwyer osteotomy *and* a complex rotation of the heel achieved the best correction of significant varus. With this technique, a fusion is rarely required. It is important to ask your surgeon about what approach he or she uses to correct heel varus.

In the next installment of this series, I'll discuss muscle imbalance and tendon transfers. In the meantime, you can follow me on Instagram at @Charcotmarietoothsurgery. ★

Dr. Pfeffer is an orthopedic foot and ankle surgeon and co-director of the CMTA's Center of Excellence at Cedars-Sinai Medical Center, Los Angeles. He is also a member of the CMTA's Advisory Board.

From CMT Denier to Defier in One Patient/Family Conference

BY JULIE STONE

My relationship with CMT has always been really complicated. My symptoms started when I was 6 and I developed foot drop. My mom had foot drop and muscle weakness so we always thought this could be a possibility for me, but we never talked about it and I never really knew what was wrong. When people asked I always said, “It’s just this weird disease that makes my muscles suck.” I kept all my feelings buried and pretended like it didn’t exist. Although I had this “thing” I remained very active.

When I was 27, I was forced to confront my condition after back-to-back injuries caused by my foot drop. I decided it was time to find out what was going on and learned I had CMT! My doctor passed along the info for the CMTA Patient/Family Conference in Seattle in October 2018 and I decided to attend. I wasn’t quite

sure what I was getting myself into, so I went super incognito!

The conference changed my world. I learned so much about CMT and found my amazing network of doctors, who have worked really hard to make my day-to-day life easier. I also met CMTA

“If anyone had told me a year ago that I’d be out and talking about my disability I wouldn’t have believed it.”

Development Director Jeana Sweeney, who really inspired me with her amazing style, positive outlook and bubbly personality. Jeana broke down a lot of my stereotypes about what people with disabilities are like.

This mindset had prevented me from accepting my CMT. I thought of myself as a fitness girl who worked in fashion—I have a fashion degree and currently work on the women’s design team at Zumiez, and I’ve always been really active with diving and competitive gymnastics. In my mind, I wasn’t disabled because a disabled person wouldn’t do these things. What ignorant thoughts! I’m so



JULIE, now 30, lives, works and spreads the word about CMT in Seattle.

embarrassed to admit that I was once one of those people whose minds I’m now working so hard to change.

If anyone had told me a year ago that I’d be out and talking about my disability I wouldn’t have believed it. But talking about its ups and downs has been so liberating. It has opened up a whole new world to me. I have a fitness channel on YouTube where I showcase fitness possibilities for CMTers in my “One Class a Week Challenge.” Thus far I’ve done boxing, aerial yoga, Zumba, cycling and pole dancing. I hope to inspire other people to be more open about their CMT and help to break down the negative stereotypes that are associated with it. I’ve learned that by focusing on what I have instead of what I don’t have, I can be a fitness girl with CMT who works in fashion.

Please check out my website and start defying stereotypes with me at www.cmtdefy.com. Stay fit with me on YouTube by subscribing to my channel: CMTdefy. Follow me on Instagram @CMTdefy. ★

CMT SURVIVOR SHIRT!

The Baltimore Biker Trading Company is teaming up with the CMTA and CMTA Board Member Steve O'Donnell to raise critical dollars for research.

For every Plane Survivor shirt sold, the Baltimore Biker Trading Company will donate \$5.00 to the CMTA!

Order yours today at planes.social/apparel5.

CMTA
Charcot-Marie-Tooth Association

Youth of Tomorrow Shine at 10th Annual STAR Gala

BY YVETTE GREEVES

The CMTA honored the “Youth of Tomorrow” at the 10th Annual STAR Gala in New York on October 29, 2019. Hosted by Phyllis Sanders and Alan and Ruth Korowitz at the Essex House hotel, the evening officially began with a VIP reception where five

exceptionally well-spoken and mature-beyond-their-years youth explained what a treatment for CMT would mean to each of them.

The five honorees—Vittorio Ricci, 21; Krystyn Finelli, 19; Evan Zeltsar, 14; James Morgan, 10, and my 10-year-old daughter Ava—shared their experiences living with CMT. Vittorio expressed gratitude that he’s had the CMTA as a constant resource throughout his journey while Krystyn talked about writing poetry both as therapy and a way for others to understand her struggle in a very personal way.

Many of the honorees mentioned the transformative power of Camp Footprint. Ava spoke about the immediate sense of belonging she and her fellow campers

experienced. She didn’t feel “different,” as she sometimes does at school or when playing with neighborhood kids. She didn’t have to explain to anyone what this strange-sounding disease means and why she can’t run and jump like they can. Her fellow campers knew her before they even met and she now counts every one of them as family. The reception was a truly heartwarming event, and each of the honorees was presented with a Star Naming Certificate to represent their bright futures.

The entire evening was filled with laughter and tears, catching up with old CMTA friends and meeting new ones, hearing about research updates, and most importantly, shopping to aid the CMTA’s fundraising efforts. Dr. Steven Scherer delivered the keynote address with an update on the promise of gene therapy treatments, particularly for CMT Type 2. He also emphasized how important the introduction of biomarkers will be in speeding the pace of discovery and treatments, as they will allow researchers to know whether certain treatments are feasible before launching a full study.

The event raised more than \$235,000 for STAR research, much of it from a silent auction that included jewelry, sporting memorabilia, art, travel, sporting events and concerts. Even though Ava loved the idea of a ticket and backstage pass to a Jonas Brothers concert, we agreed that our favorite auction item was the opportunity to sponsor a child to attend Camp Footprint.

It was the first time our family had the pleasure of attending a CMTA Gala, and we were very impressed, not only by the efforts of the sponsors and the CMTA but also by the inspiring words of our youth. They remind us time and again that we are family and that we are all in this journey together. On behalf of all CMT parents, we thank the CMTA for continuing to support and connect the children through Camp Footprint and the launch of the new CMTA youth programs. By giving them a voice, we empower them to lead us into the future. ★



Drs. John Svaren and Steve Scherer bookending honorees Evan Zeltsar and Vittorio Ricci (rear) and Yvette Greeves and James Morgan (front)



Krystyn Finelli, also honored at the event, spoke about writing poetry as therapy. Her poem “So What?” appeared in the Winter 2018/19 issue of *The CMTA Report*.



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

Dear David,

For the last 10 years, I have had my dream job working as a physical education teacher in an elementary school in a great school district. Unfortunately, I was diagnosed with CMT1A at 32 years old. That was three years ago. I am only mildly affected at this point and my CMT is not affecting my ability to do my job, but my boyfriend insists that I quit immediately and find a different career before I make a fool of myself. One of the aspects that attracted me to teaching physical education was the opportunity to be sensitive to the children who were less coordinated and had a more difficult time with sports. I need some guidance around my career choice.

David replies:

My first response is that you should keep the job and ditch the boyfriend. Just kidding, but seriously, this is your decision, not his. It seems foolish to leave a profession you love before your CMT interferes with your ability to do your job—you could still continue teaching in an area you love for many years. Good teachers are

invaluable in our society, and I am sure your skills are transferable if the need to change areas of specialty arises. Your passion and sensitivity to kids who might be different will contribute to your being a top-notch educator, regardless of the subject you teach.

I have received many letters from members of our CMT community who are in similar situations. Just when we think we have a part of our life figured out, we are thrown a curve. In many occupations, apart from doing construction on skyscrapers, there are usually workarounds. If it means taking things a bit more slowly and giving ourselves breaks to rest, then so be it. It's the same with trying to keep up with active kids. Don't for one second think that you are a less effective worker if you need to take things at a slower pace. Your head and your heart are just fine and won't interfere with your job performance. What will interfere is your anxiety about what others think.

A larger issue that concerns many of us is how to find meaning in life when physical limitations become more pro-

nounced. Finding opportunities to help others is a great way to get out of our heads and see that CMT does not have to stop us from having a life of meaning and purpose. Our suffering is real, but when we focus on it we lose perspective and forget that all of us have so much to give in our own way. We have an opportunity to find meaning in life just by the way we handle our attitude towards our own suffering. We can find meaning just by the way we handle our challenges. Picking ourselves up and showing others that a limitation does not have to keep us down is inspirational to others who let the slightest problem in life become overwhelming. Trying to have compassion for ourselves is the key, which is not the same as feeling sorry for ourselves, although that is okay on occasion as long as it's temporary. Compassion for ourselves reminds us that loving ourselves is not in spite of our CMT, but just maybe because of it. Seeing beauty and accepting our imperfections gives us a depth of being that only those who face challenges in life can truly understand. ★



Write to David at info@cmtausa.org.

David Tannenbaum has an LCSW degree and has been a psychotherapist in New York City for the past 30 years, specializing in helping others with the task of growing emotionally and spiritually through physical challenges. "My CMT has been my greatest challenge and my best teacher in life," says David.

Donate and support the CMTA in the fight against the progressive and devastating effects of CMT

Complete and return to: CMTA • PO Box 105 • Glenolden, PA 19036

\$25 \$50 \$100 \$250 \$500 \$1,000 Other: \$ _____

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

Please make this amount a One-time or Monthly gift and charge to my Visa MasterCard American Express

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City _____ State _____ Zip _____ Phone _____

I am making this donation in honor or in memory of (name): _____

Please send me CMTA updates via email at: _____





CMTA BRANCH NEWS

CONNECTICUT BRANCHES

The Hartford and Southern Connecticut Branches held a joint meeting on October 24, 2019, at the Hospital for Special Care, a CMT Center of Excellence. Dr. Kevin Felice and his team gave the 35 attendees a tour of the neuromuscular clinic at the Hospital for Special Care and facilitated a discussion about CMT



and what's happening in research. Kudos to branch leaders Roy Behlke (Hartford, CT/Naples, FL) and Lynne Krupa (Southern Connecticut) for helping plan the meeting.

★ CHICAGO, IL

Jim King of Allard USA spoke to nine members of the Chicago branch on August 31,

2019. He explained how braces help people walk and shared information about Allard's dynamic carbon-fiber braces. The braces got the thumbs up from all the members who tried them.

★ SPRINGFIELD, MO

Nine people attended the Springfield branch meeting on September 21, 2019. They reviewed the slides (and received copies of the presentations) from the St. Louis CMTA Patient/Family Conference. Members discussed challenges in functioning, their doctors and the day-to-day issues of living with CMT. Group members decided to hold a Walk 4 CMT on September 19, 2020.

★ LAS VEGAS, NV

Nineteen people attended the Las Vegas Branch meeting on October 19, 2019. Branch Leader Martha Boadt shared highlights and presentations from the CMTA Patient/Family Conference in St. Louis. The group also talked about easy ways to fundraise, such as writing family and friends asking them to contribute to STAR research. Martha raised \$873 simply by emailing a fundraising letter to friends and family.

★ NEWBURY, NH

The Newbury Branch welcomed 12 attendees to its meeting on November 9, 2019, to hear orthotist and CMTA Advisory Board Member Ken Cornell talk about the benefits of therapeutic interventions such as AFOs, which can improve gait and balance issues for CMT



patients. One of Ken's recommended bracing options was the Dynamic Carbon Ground Reaction AFO, and a few members were so impressed with the brace that they made follow-up appointments with Ken to learn more.

★ NEW MEXICO

Three new members came to the New Mexico branch meeting on November 2, 2019, for a total of 18 attendees. Two representatives from GeneDX talked about genetic testing for CMT and the practical aspects of getting testing done, such as getting a request "through the system" and who can order it.

★ WESTCHESTER, NY

Eighty-three people came out for the Westchester Branch's 7th annual luncheon at the Tarrytown Hilton on September 14, 2019.

(continued on page 22)

CMTA CENTERS OF EXCELLENCE

www.cmtausa.org/coe

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



CMTA CENTER OF EXCELLENCE

Cedars-Sinai Medical Center (Los Angeles).....	Drs. Robert Baloh and Richard Lewis
Children's Hospital of Philadelphia (Philadelphia).....	Dr. Sabrina Yum
Children's Hospital of Pittsburgh (Pittsburgh)*	Dr. Hodas Abdel-Hamid
Connecticut Children's Medical Center (Farmington).....	Dr. Gyula Acsadi
Hospital for Special Care* (New Britain, CT)	Dr. Kevin J. Felice
Johns Hopkins University (Baltimore)	Dr. Thomas Lloyd
Kane Hall Barry Neurology (Dallas/Fort Worth)*	Dr. Sharique Ansari
Lucile Packard Children's Hospital at Stanford (Palo Alto)	Drs. John Day and Ana Tesi Rocha
Massachusetts General Hospital (Boston)	Dr. Reza Seyedsadjadi
Nationwide Children's Hospital (Columbus)*	Dr. Zarife Sahenk
Nemours Children's Hospital (Orlando)	Dr. Richard Finkel
Northwestern Memorial Hospital (Chicago)*	Dr. Daniela Maria Menichella
Ohio State University (Columbus)	Dr. Amro Stino
Oregon Health & Science University (Portland)*	Dr. Chafic Karam
Rush University (Chicago)*	Dr. Ryan D. Jacobson
Stanford Neuroscience Health Center (Palo Alto)	Dr. John Day
University of Florida (Gainesville)*	Dr. James Wymer
University of Illinois at Chicago (Chicago)*	Dr. Charles K. Abrams
University of Iowa (Iowa City)	Dr. Michael Shy
University of Miami (Miami)	Dr. Mario Saporta
University of Michigan (Ann Arbor)*	Dr. Dustin Nowacek
University of Minnesota (Maple Grove)	Dr. David Walk
University of Missouri (Columbia)	Dr. Raghav Govindarajan
University of North Carolina (Chapel Hill)*	Dr. Rebecca Traub
University of Pennsylvania (Philadelphia)	Dr. Steven Scherer
University of Pittsburgh Medical Center (Pittsburgh)*	Dr. Sasha Zivkovic
University of Rochester (Rochester, NY)	Dr. David Herrmann
University of Texas Southwestern (Dallas)*	Drs. Susan Iannaccone and Diana Castro
University of Utah (Salt Lake City)	Dr. Russell Butterfield
University of Washington (Seattle)	Dr. Michael Weiss
Wayne State University (Detroit)	Dr. Jun Li

*These Centers of Excellence are not part of the INC.

INTERNATIONAL

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

THE CMTA GRATEFULLY ACKNOWLEDGES GIFTS...

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PATRICIA DREIBELBIS

Mr. Steven Koshrova

FRANK ECKMAN

Ms. Deborah Tamburello

HELEN EMBLETON

Mrs. Gisele Napolitano

CHARLES FOYE, JR.

Ms. Patti Condon

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Mrs. Kristin Carroll

Mr. & Mrs. Frederick Dale

Mrs. Joyce Feodoroff

Mr. & Mrs. Charles Keller

Ms. Arlene Little

Ms. Donna Quinn

Mr. & Mrs. Bennie Schiavo

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Mrs. Kimberly Carpenter

Mr. David Mazzetti

Ms. Kaitlin McCormick

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KATHY GREER

Ms. Rebecca Floch

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Ms. Andrea Mullen

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JOAN HEITNER

Ms. Joan Greenbaum

Ms. Ann Silverman

Ms. Judi Silverstein

ANTHONY JOHNSEN

Mr. William Allhusen

Mr. Jared Anderson

Ms. Sasha Arasteh

Ms. Barbara Behrmann

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Ms. Catherine Chergotis

Ms. Maura Garrity

Mrs. Anne Marie Graham

Mrs. Carol Opland

Ms. Toni Overacker

Ms. Katherine Rapp

Ms. Joyce Rozmus

Mrs. Robyn Schaefer

Mr. & Mrs. Raymon Twedell

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Mrs. Dianne Martenson

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Dr. Julie Schell

JAMES KEENAN

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Ms. Janet Fiore

Ms. Amy Goldman

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Ms. Kimberly Hinkley

Mr. Don Hossler

Mrs. Joan Kaiser

Ms. Courtney Kallas

Mr. & Dr. Jurgen Kutscher

Mrs. Jacqueline Lucas

Ms. Lynn McCleary

Mr. Christopher Roken

Mrs. Donna Tokarz

Dr. Michael Wehmeyer

Mr. & Mrs. Thomas Wiedl

Ms. Fotini Xenidis

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Ms. Audrey Holt

REV. H. J. KOLB

Ms. Sandra Thompson

IRVIN J. KOLB

Ms. Sandra Thompson

FLORENCE KOSEWICK

Mr. & Mrs. Suzanne Jones

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Mrs. Ann Weiss

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Ms. Kari Lee

Ms. Sally Lee

JIM LINDSAY

Mrs. Diane Lindsay

STEVE LOY

Mr. & Mrs. Wesley Moody

NANCY MARDEN MCLEAN

Mr. Michael Caparelli

Ms. Kathryn Lynch

SHIRLEY MCKEE

Ms. Debra Jo Alexander

MARY JEAN MENDICINO

Mrs. Amy Costello

MELANIE MESSER-SMITH

Ms. Ruthmarie Gray

McGuire

CARMEN MOSER

Mrs. Dianne Martenson

MARVIN MOSER

Mrs. Dianne Martenson

SUSANE O'BRIEN

Mrs. Leona Doyle

LOREN O'CONNOR

Ms. Rita Lalumia

MARK POPE

Mrs. Naomi Pope

THERESA FRANZETTI PUCKETT

Mrs. Nene Simmons

JEAN RADNER

Mr. Richard Kaufman

MIKE ROSS

Ms. Laura Moorman

BILL RUGG

Sunlake Social Club

Bingo

LILA B. SCAFFE

Ms. Beverly Stevens

Ms. Jeanette Widener

ALTHEA SCOTT

Mr. Ronald Scott

CHRISTINE SHUMWAY

Mr. Chad Dutcher

Ms. Linda Komarmy

Mr. & Mrs. Daryl Popowitch

Mr. & Mrs. Keith Southerton

Ms. Lillian Stehr

PASQUALE SIMONETTI

The Mercy Girls

Mr. & Mrs. David Rowan

CHARLES F. SMITH, JR.

Ms. Linda Craft

Ms. Susan McConnell

CHARLES STERLING

Ms. Patricia Hooker

Ms. Cindy Wasserman

DR. EDWARD STERN

Mr. & Mrs. Martin Sarver

SARAH SWAIN

Mr. Lawrence Seneor

ROBERT AND MARGARET SWITZER

Dr. Margie Switzer

ROBERT AND MARGARET SWITZER

Cowan

DOLLY THOMPSON

Ms. Robin Thompson

SIDNEY TOUGH

Mr. Thomas Tough

ESTHER VAGLIO

Mrs. Teri Drapeau

JENNY VONK

Ms. Annie Veenstra-Planting

RICHARD J. WATERFIELD

Mr. & Mrs. Walter Hagen

BETTY WEIGLE

Ms. Beverly Buhl

ROBY WINTERS

Ms. Katherine Carr

Touchstone

NOLE WOLF

Mrs. Alice Stout

MARY ELIZABETH YORK

Ms. Tusnelada Hodgkin

IN HONOR OF:

JULIE, BRIDGET, JOHN AND STEVE

Mr. & Mrs. Joseph Campanella

CAROL ALBRIGHT

Mr. Bryan Albright

DEEANN ALONGI

Ms. Ranae Knight

5 BRANCHES ADDED TO THE CMTA FAMILY TREE IN 2019

The CMTA added five new branches and seven new branch leaders and co-leaders in 2019. The branches—volunteer-driven support and education groups—are a vital component of the CMTA’s education and awareness mission. Guest speakers provide education about the disease and best practices for therapeutic intervention to help manage disease symptoms.

The new branches and leaders are:

- **JACKSONVILLE, FL:** Tim Nightingale and Stephanie Burkhalter
- **NEWBURY, NH:** Jacinta Gantz
- **SYRACUSE, NY:** Mike Casey
- **SOUTHWESTERN VIRGINIA:** Karen Brown
- **TULSA, OK:** Lonna Henry and Natasha Morgan

This amazing group of new branch leaders hit the ground running by building their branches, planning fun and informative meetings and taking time to support the CMT community in their areas.

Two of the new branch leaders—Tim in Florida and Jacinta in New Hampshire—expressed surprise at the number of people (aka potential members) in their vicinity who have CMT. According to Tim, they share a thirst for knowledge and information about the disease. Jacinta said her peak experience since taking on the new role has been meeting so many others with CMT who also felt alone and were excited to meet others just like themselves.

Mike from Syracuse, who held both his first meeting and his first fundraiser in 2019, said he was most surprised by the turnout for his branch’s walk, which raised almost \$4,000 and made him feel that “We are NOT alone.” One of those people was a 6-year old with purple braces who had never seen another human being wearing AFOs before Mike.

Karen from Southwestern Virginia said that while she was nervous about leading a new branch all by herself and imagined she’d be begging people to help her, the complete opposite was true, a “welcome and wonderful surprise.” Out of a total of three CMTers at her first two meetings, two were “all-in” with helping grow the branch and planning a fundraising event. “I did not anticipate such willing help and great ideas right away and from such small numbers, but my people are as excited as I am to have a regional presence, and I have a real team already,” she says.

**SEARCH OVER 70+ BRANCH LOCATIONS
NATIONWIDE TO FIND ONE NEAR YOU:**

www.cmtausa.org/branches/.

**If you don’t find one, consider starting one!
For more information, contact CMTA Director
of Community Outreach Laurel Richardson
at laurel@cmtausa.org.**

BRANCH NEWS

(continued from page 20)

Volunteers and supporters of this active group raised close to \$22,000 for STAR research. After a delicious buffet, the group held a 40/20/20/20 raffle, and two out of three winners donated their winnings back to the CMTA. Funds also came from



donated gift cards from various vendors and restaurants and a silent auction that included a pre-owned mink coat. The afternoon also included a “Big Hat Contest” with the winner receiving a donated gift card.

On November 9, 2019, CMTA Board Member Elizabeth Ouellette spoke to the Westchester group via Skype. She stressed the benefits of keeping active and suggested that members check out board member Steve O’Donnell’s new exercise video series on the CMTA website. She also gave a STAR research update.

★ **WILMINGTON, NC**

The Wilmington Branch welcomed foot and ankle surgeon Dr. Tyler Gonzalez to its meeting on November 9, 2019. Dr. Gonzalez, from the University of South Carolina, specializes in CMT. He shared information about therapeutic interventions that can obviate the need for surgery. He also discussed options for CMT patients when surgery becomes necessary. Twenty people attended and after the presentation Dr. Gonzalez spent time answering questions and talking with them one-on-one.

★ **CLEVELAND, OH**

Nine Cleveland Branch members came out on September 26, 2019, for the first branch meeting under the new leadership of Shelly McMahon. After Shelly thanked Heather Hawk Frank for her years of leadership, she laid out meeting dates for 2020 and members discussed prospective guest speakers and fundraising ideas and got acquainted.

★ **TULSA, OK**

The newly formed Tulsa branch had a great first meeting in 2019. Branch Leaders Lonna Henry and Natasha Morgan welcomed a couple of families and shared information about the CMTA and what’s going on in research and in the community. The branch is looking forward to meeting regularly and growing.



CMTA Branches

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

ALASKA

Anchorage Area
Lisa Hubert
907-223-4566

ARKANSAS

Little Rock
Lisa Jones
501-776-5364

ARIZONA

Phoenix Area
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

San Diego Area

Annette Van Veen
760-473-5014
Kendall Trout
760-632-5654

South Bay Area

Ori Bash
408-829-4562
Tau O'Sullivan
916-806-2173

COLORADO

Denver Area

Ron Plageman
303-929-9647
Dick Kutz
303-988-5581

CONNECTICUT

Hartford

Roy Behlke
239-682-6785

North Haven

Lynne Krupa
203-288-6673

DISTRICT OF COLUMBIA

Washington, DC

Steven Weiss
Kimberly Hughes
301-962-8885

FLORIDA

Central Florida

Linda Davis
Mitch Davis
863-875-4239

Jacksonville Area

Tim Nightingale
904-504-1953
Stephanie Burkhalter
904-710-3771

Naples

Roy Behlke
239-455-5571
Bill Millar
202-309-5685

Sarasota Area

Rachel Rivlin
941-284-0766
Manuel Goldberg
941-726-8633

Tampa Bay Area

Edward Linde
813-712-4101
Sarah Gentry
941-706-5348

West Palm Beach

Phil Lewis
561-307-0100
Eileen Martinez
561-901-5566

GEORGIA

Atlanta Area

Jeannie Zibrida
404-307-6519

IOWA

Iowa City Area

Jeffrey Megown
319-981-0171

ILLINOIS

Chicago Area

Jay Pate
630-888-4673
Doreen Pomykala
815-351-1328

INDIANA

Fort Wayne Area

Aimee Trammell
574-304-0968

KANSAS

Kansas City Area

Tammy Adkins
314-608-6889
Aron Taylor
913-744-5674

Wichita Area

Karen Smith
316-200-0453

LOUISIANA

Baton Rouge Area

Corey Johnson
704-904-2828
Danielle Dalfrey
318-294-1976

MASSACHUSETTS

Boston

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

MAINE

Portland Area

Mary Louie
207-450-5679

MICHIGAN

Grand Rapids Area

Amy Reynolds
616-916-6732

MINNESOTA

Central Minnesota

Jo Smith
612-807-4729

Minneapolis Area

Duane Hodges
612-325-5448

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

Jessica Brantner
417-468-8049
Jessica Hardy
417-434-1656

NORTH CAROLINA

Charlotte Area

Carrie Johnson
704-904-2828
Durham Area

Jeanne Boehlecke

919-942-7909
Rick Nelson
919-889-9776

Wilmington Area

Laurel Richardson
910-515-8488

NEBRASKA

Lincoln Area

Brandon Lederer
402-680-0502

NEW HAMPSHIRE

Newbury Area

Jacinta Gantz
978-596-4444

NEW JERSEY

Central New Jersey

Mark Willis
732-252-8299
Jacqueline Donahue
732-780-0857

NEW MEXICO

Albuquerque Area

Gary Shepherd
505-296-1238

NEVADA

Las Vegas Area

Martha Boadt
231-852-4251

NEW YORK

Buffalo Area

Peter Morris
716-866-3519
Maryann Ciskal
716-435-3899

Syracuse Area

Michael Casey
315-439-9325
Albany Region

Cara Leath

914-262-1108
Westchester Area

Beverly Wurzel

Frank Wurzel
201-224-5795

OHIO

Cincinnati Area

Jill Stuhlmueller
513-254-4065
Jo Koenig
513-607-2822

Cleveland Area

Shelly McMahon
440-781-8329

Columbus Area

Jessica Diamond
216-570-6432

OKLAHOMA

Tulsa Area

Lonna Henry
918-961-1418

OREGON

Grants Pass

Jessica Barton
541-218-5350 (cell)
541-846-8525

PENNSYLVANIA

Bucks County Area

Julie FitzGerald Schell
315-573-3919

Chester County

Ashley Trout
484-364-9334

Harrisburg

Erin Weierbach
717-379-7504

Johnstown Area

J.D. Griffith
814-539-2341
Jeana Sweeney
814-269-1319

Northwestern Area

Joyce Steinkamp
814-833-8495

Pittsburgh

Debra Czarnecki
412-331-6744

SOUTH CAROLINA

Columbia Area

Zack Boyd
803-622-6565
Kyle Bryant
803-378-6202

Greenville Area

Rebecca Lauriault
864-918-2437

TENNESSEE

Nashville Area

Bridget Sarver
615-390-0699
Teresa Shoaf
615-772-8810

TEXAS

Austin Area

Nate Halk
512-415-6097

Dallas/Fort Worth

Thomas Roderiguz
717-913-8477

El Paso Area

Jo Ann Longoria
915-526-0676
Olivia Longoria
915-491-0786

Houston Area

Kristin Leard
713-516-8630
Benjy Hershorn
832-731-0121

Meredith Wells

832-264-7312

UTAH

Orem Area

Melissa Arakaki
801-494-3658

VIRGINIA

Blacksburg/ Roanoke Area

Karen Brown
540-252-9453

Fredericksburg

Leigh Van Doren
540-370-1968

Suffolk Area

Holly Hall
757-477-6122

WASHINGTON

Seattle Area

Denise Snow
206-321-1261
Emily Osborne
425-220-4225

WEST VIRGINIA

Charleston Area

Karen McClure
304-548-4413

WISCONSIN

Madison Area

Debi Weber
608-712-8709

Milwaukee Area

Lois Hawkins
414-249-0390

CANADA

Southern Ontario

Kelly Hall
519-843-6119

Toronto Area

Linda Scott Barber
416-997-5084
Michael Driedger
647-680-7601

Interested in starting a branch in your area?

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



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 (Erbix)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddI, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Eribulin (Halaven)
Fluoroquinolones
Gold salts
Ipilimumab (Yervoy)
Ixabepilone (Ixempra)
Leflunomide (Arava)
Lenalidomide (Revlimid)
Metronidazole/Misonidazole
(extended use)
Nitrofurantoin (Macrochantin,
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-Fluorouracil
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)
α-Interferon

Negligible or doubtful risk:

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



The Charcot-Marie-Tooth Association

P.O. Box 105

Glenolden, PA 19036

1-800-606-CMTA (2682) FAX (610) 499-9267

www.cmtausa.org

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WHAT IS CMT?

- ▶ More than 2.8 million people worldwide have CMT, which is one of the most commonly inherited nerve disorders and affects the motor and sensory nerves.
- ▶ CMT is slowly progressive, causing the loss of muscle function and/or sensation in the lower legs and feet, as well as hands and arms.
- ▶ Men and women in all ethnic groups may be affected by CMT.
- ▶ CMT is genetic, but it can also develop as a new, spontaneous mutation.
- ▶ CMT can vary greatly in severity, even within the same family.
- ▶ CMT causes structural deformities such as high-arched or very flat feet, hammertoes, hand contractures, scoliosis (spinal curvature) and kyphosis (rounded back).
- ▶ CMT can also cause foot drop, poor balance, cold extremities, cramps, nerve, muscle and joint pain, altered reflexes, fatigue, tremor, sleep apnea, hearing loss and breathing difficulties.
- ▶ CMT rarely affects life expectancy.
- ▶ Some medications are neurotoxic and pose a high risk to people with CMT, notably Vincristine and Taxols. See full list (at left) of medications that may pose a risk.
- ▶ More than 100 different genetic causes of CMT have been identified.
- ▶ Many types of CMT can be determined by genetic testing. Please consult with a genetic counselor (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's STAR research program and extensive partnerships with pharmaceutical companies are driving remarkable progress toward delivering treatments for CMT, bringing us closer to a world without CMT.