SPRING 2022

THE CMTA REPORT

www.cmtausa.org

OUR VOLUNTEERS ARE HELPING US REACH NEW HEIGHTS!

PICTURED LEFT, CMTACTIVE VOLUNTEER JULIE BARNETT.
READ MORE ABOUT OUR FABULOUS VOLUNTEERS ON PAGE 4

CONTENTS

SPRING 2022

RESEARCH

COMMUNITY

LIVING WITH CMT

FUNDRAISING

YOUTH

THE CMTA REPORT | SPRING 2022

Marcia Semmes, *Editor*
Virginia Vitzthum, *Contributing Editor*
Karlyn Rosen Aires, *Designer*

Charcot-Marie-Tooth Association

Charcot-Marie-Tooth Association

P.O. Box 105
Glenolden, PA 19036
1-800-606-CMTA (2682)
FAX (610) 499-9267

cmtausa.org

ISSN #1067-0181 Vol. 37, No. 1

Email the CMTA at info@cmtausa.org

CMTA
Charcot-Marie-Tooth Association

A MESSAGE FROM THE CEO

Dear Friends,

With a paid staff of just seven people, the CMTA could not survive—much less thrive as it has—without the Herculean efforts of our many front-line volunteers. They are our lifeblood, the indispensable source of our strength and vitality. That lifeblood pulses through every aspect of the CMTA's operations.

CMTA volunteers share every skill set imaginable. They hold walks and fundraisers, moderate online groups and teach campers how to take care of their feet, chair-dance and support each other. They write stories for the newsletter, staff our help desk and provide graphic design. They also provide crucial information to the researchers trying to cure CMT, taking part in clinical trials, filling out surveys and signing up for our Patients as Partners in Research initiative.

Volunteers also help the CMTA by helping each other. By sharing tips, comfort, understanding, resources and referrals, they help the CMTA fulfill its mission of improving the quality of life for everyone affected by CMT.

We salute, celebrate and thank each and every one of our volunteers. If we wrote about all of you, the Report would be 1,000 pages long, so we've selected just a few to highlight. If you're a volunteer and don't see your name here, please know that we see you and we appreciate you more than we can ever say.

With gratitude,

Amy Gray

Amy Gray
CMTA Chief Executive Officer

CMTA Gets Charity Navigator's Highest Rating For Third Year in a Row

Charity Navigator awarded the CMTA its third consecutive 4-star rating for demonstrating strong financial health and commitment to accountability and transparency on Feb. 1.

As Michael Thatcher, president and CEO of Charity Navigator, said in announcing the award, "This is our highest possible rating and indicates that your organization adheres to sector best practices and executes its mission in a financially efficient way.

"Attaining a 4-star rating verifies that Charcot-Marie-Tooth Association exceeds industry standards and outperforms most charities" in the CMTA's area of work, Thatcher said. He noted that only 27 percent of the charities that the organization evaluates have at least three consecutive 4-star evaluations, indicating that the CMTA "outperforms most other charities in America." He continued, "This exceptional designation from Charity Navigator sets Charcot-Marie-Tooth Association apart from its peers and demonstrates to the public its trustworthiness."

CHARITY NAVIGATOR
★★★★
Four Star Charity

#THANK YOU VOLUNTEERS!

HUNDREDS OF CMTA MEMBERS ACROSS THE COUNTRY LEND THEIR SKILLS AND STRENGTH TO OUR COMMUNITY

BRANCH LEADERS BUILD COMMUNITY

Martin Luther King, Jr. said, **"Life's most persistent and urgent question is, what are you doing for others?"** The CMTA's 93 branch leaders and co-leaders don't have to think about their answers to the question.

These amazing volunteers provide support, education and resource information for members in the United States, Canada and Mexico. They plan and organize meetings, walks and fundraisers, bringing in vital dollars to help support the CMTA's mission of finding a cure for CMT.

They also create a community in which people feel embraced and engaged. Members share stories, experiences and ideas for coping with CMT in an environment of acceptance and empathy and understanding. Branch leaders organize and facilitate regularly scheduled meetings where individuals share their experiences face to face, creating a haven of understanding and camaraderie.

In these structured forums, individuals can help and be helped by others, lifting the burden of isolation. Speakers often include CMT-savvy experts willing to donate their time and knowledge to educate members about their specialties. Branch members, whose lived experience gives them their own expertise, often end up enlightening presenters about their symptoms and challenges of living with CMT.

Raleigh, NC (RTP) Branch Leader Jeanne Boehlecke, a retired rehabilitation counselor with Type 2, says she volunteers as a branch leader because the meetings "offer sound, helpful suggestions for CMT management and reassurance to people newly diagnosed."

Jeanne's work taught her that groups show members that they are not alone in living with CMT challenges, a real stress reducer. In addition, she says, the CMTA's Strategy to Accelerate Research (STAR) gives her confidence that serious efforts are being made to find a cure for CMT. She shares that hope for the future by updating branch members with the latest research findings through branch meetings and CMTA webinars.

Jeanne says volunteering "expands your horizons and helps you realize talent and interests you may not know you have. It's also good for your health and for your soul. What you put your time and energy into is an indication of your values."

CMTA Outreach Director Laurel Richardson said Jeanne "consistently provides her group with meeting content meaningful to those living with CMT. She has not skipped a single meeting since she started, even when the pandemic forced meetings online. She works hard to find guest speakers who can share information about living well today with CMT. She is unflappable and does all of this with a smile on her face."

Las Vegas Branch Leader Martha Boadt, who turns 80 in April, is one of the CMTA's oldest branch leaders, but she has the energy and enthusiasm of someone decades younger. Her motto is "Keep on keeping on." Martha, like her mother, siblings and children, has 1B.

When Martha and her husband retired to Las Vegas, she looked for the local CMTA branch and found that it had folded. She decided to revive it. It took some time to build, but it's now up and running again. Her main goal, she says, is "to bring quality healthcare to people living with CMT in the Las Vegas Valley" by identifying local neurologists, orthotists, physical therapists and other CMT experts.

Today, Martha says, "I'm loving my experience volunteering with the CMTA. Every day I learn something new. I am not an expert, just a patient myself. I love when our group meets virtually or in person. If I help one person find an answer to help them cope I am excited."

Laurel Richardson praised Martha's desire to help others with CMT, adding: "Martha plans engaging meetings, researches the best neuromuscular experts in the area, and makes sure to share all CMTA updates with her branch members. She is a force to be reckoned with."

Toronto, Ontario, Branch Leader Mike Driedger said that before joining his local branch, he could "count on one hand" the number of people with CMT he'd met in his life. He says the branch "gives the opportunity to connect, learn from and share with so many others with similar yet unique experiences. It's really wonderful to have that connection point."

Mike, who has CMTX, became a branch co-leader in 2017 and then eventually the leader of the Toronto branch. What was initially a fairly localized group of members across the Toronto area "has grown into a more national group during the COVID-19 pandemic. Shifting from an in-person to an online format has provided the opportunity to connect with people from coast to coast across Canada." Asked what he would tell someone thinking about volunteering for the CMTA, Mike replied: "Do it! Please."

Laurel said, "What's special about Mike is he works full-time and is incredibly busy, but he still finds the time to serve others living with CMT. His dedication and energy are admirable and two of the qualities that make him a wonderful branch leader."

If you want to join the CMTA's amazing army of volunteer branch leaders, please reach out to laurel@cmtausa.org.

continued on page 6

WALK LEADERS GATHER SUPPORTERS

The CMTA is one of the few national nonprofit organizations with a walk campaign organized and led solely by volunteers. Those volunteers hosted 28 Walks 4 CMT in 2021, raising more than $190,000 for STAR research despite the disruptions and restrictions caused by the pandemic.

Adapting to the constant changes, leaders stayed flexible. Some hosted in-person walks at a set time and place. Others had virtual walks on a set day, with participants walking on their own time at various locations. There were also a number of statewide walks, with walkers participating from their own neighborhoods statewide on a prescribed date, then reporting their results online.

The **DC Metro Walk,** typically held on the National Mall, had to find another location due to National Park COVID restrictions. Walk Leaders Steve Weiss and Kim Hughes adapted quickly and switched the location to a park near their home. According to Steve and Kim, "When you attend a Walk 4 CMT, you experience the vibrant spirit of the people and families living with this disease. Whether in-person or virtual, in a metropolitan area or a neighborhood park, Walk 4 CMT sends the strong message that together, the CMT community can overcome any challenge the disease throws at us."

The CMTA thanks ALL walk leaders for their flexibility and creativity in raising research funds:

Birmingham, AL: Donna Webb
California: Alani Price and John Ramos
Washington, DC: Steve Weiss and Kim Hughes
Florida: Sarah Gentry, Lara Rustici and Mae Greenburg
Atlanta: Jeannie Zibrida
Maine: Mary Louie
Boston: Jill Ricci
Long Island, NY: Alessandro Cacciani
Syracuse, NY: Mike Casey
Ohio: Jill Stuhlmueller, Jo Koenig and Jessica Diamond
Bucks Co/Valley Forge, PA: Julie FitzGerald Schell and Craig Kesack
Pittsburgh: Debbie Czarnecki and Julie Tarle


DC Metro Walk

In **Florida**, walk leaders decided to go virtual and work together as a state. Tampa Walk Leader Sarah Gentry coordinated efforts for the all-state walk and hosted a Zoom event around the theme of thankfulness. "I was thrilled to collaborate with fellow Florida 4 CMT leaders to host an informative and engaging virtual gathering focusing on research and community achievements with panelists including the CMTA's CEO Amy Gray, University of Miami COE Director Mario Saporta, MD, and CMTA Youth Council Member Emmily Stufflet.

In Ohio, walk leaders Jill Stuhlmueller and Jo Koenig from Cincinnati and Jessica Diamond of Columbus worked together for an in-person walk that took place across the state. According to Jessica, "**The All-Ohio Walk** was such a success because we were able to combine all our branch resources together and doing so, we really created such a fun event for everyone. Bringing together different members from each Ohio CMTA branch and advertising to all of Ohio, we were able to meet so many new people with CMT in our state that we wouldn't have met if it weren't for this walk! We will continue to do our walks as a statewide event and we hope to grow more each year and continue to meet new faces of CMT!"


Ohio Walk

2021 WALK 4 CMT HIGHLIGHTS

$192,451 raised for CMT Research

28 Walk 4 CMT events

526 participants

9 in-person walks

6 virtual area/city walks

13 virtual all-state walks

4 sites raised more than $15,000

7 sites raised more than $10,000

DC walk raised $38,711

Top DC team "The Mighty Quinn" raised $20,655

Any city, town or state can host a Walk 4 CMT—all it takes is one leader to step forward. If you want to participate, look for a 2022 walk at www.cmtausa.org/news/walk-4-cmt/. Or, if you're ready to host a Walk 4 CMT, contact Jeana Sweeney at jeana@cmtausa.org.

PARTNERS RAISE FUNDS

While the walks raise both awareness and funds, they aren't the only volunteer-run fundraisers. Some volunteers hold wine and chocolate tastings, kids construct lemonade stands and hold bake sales and others cycle and swim for CMT.


Gary Lee Tournament

The late Gary Lee's family, for example, rallied together last year to host a golf tournament in his honor and to raise awareness of CMT. The event, held at the Meadowink Golf Course in Murrysville, PA, raised $6,400 for the CMTA. The 2nd Annual **Gary Lee Tournament** will be held on June 26.

In another example of creative fundraising, Jeff Lefkowitz decided it was time to **GoBlue for CMT** once more in 2021. In honor of his wife and children living with CMT, Jeff promised his circle of friends that if he met his $1,000 goal, he would dye his hair blue during CMT Awareness Month. Jeff raised $1,500, surpassing his goal, and sported luxurious blue locks for the full month of September.

One of the most touching 2021 fundraisers had an Olympic theme. Wendy Jensen and her family raised $300 with their own very special **"Summer Games for CMT"** at their home in Thornton, CO. After kicking off the day with a photoshoot, athlete interviews and opening ceremonies, the athletes dived into the main events, which included table tennis, gymnastics, track and field and archery.


Summer Games for CMT

Wendy pronounced the Summer Games for CMT a great success, and said, "My heart is full of gratitude that we were able to raise funds for an organization close to our hearts."

Anyone who'd like to hold a fundraiser for CMT should contact Jeana Sweeney at jeana@cmtausa.org.

ONLINE VOLUNTEERS AMPLIFY OUR VOICE

In addition to the dozens of people who volunteer as CMTA branch leaders, the CMTA is very fortunate to have another group helping us motivate and inspire community members—our Facebook moderators. These superstars provide information, resources and support to community members, and we are incredibly grateful for their time and dedication.

All but three of the CMTA's 71 branches have Facebook pages. The CMTA also hosts five Facebook groups with an international reach: The CMTA Discussion Group is the largest with almost 19,000 members; CMTActive has 1,523 members; CMTA Parents has 1,359 members; the CMTA Youth Group has 253 members; and the brand-new Cycle 4 CMT Group has 54 members.

The CMTA's Emotional Support Group, which has 1,206 members, lives on the CMTA website (https://www.cmtausa.org/emotional-support-group/). Moderated by CMTA Advisory Board member and psychotherapist David Tannenbaum, the group provides a place for members to vent the feelings that come with a chronic condition and share coping techniques.

All of the groups provide community. **Jane Bauer**, for example, found the CMTA Discussion Group even before she was diagnosed. Knowing that her mother and nephew had CMT, Jane thought she probably did, too. She sat down at her computer to see if crowdsourcing could help her find a knowledgeable neurologist. "That's how I found Dr. Clifton Gooch at the University of South Florida in Tampa," Jane explained.

After receiving a confirmed diagnosis at age 47, Jane has continued to find the tips and advice of the CMTA Discussion Group invaluable. "People talk a lot about the things that help them—CBD oil, magnesium, etc. I'm hoping I've helped people, too," she said. Even more important, the CMTA Discussion Group was where she found two things sorely missing in her life: some CMT humor and understanding. "My feet are so freezing cold at night that I put on two pairs of socks and tuck in my pajama pants, even in summer. The CMTA Discussion Group was the first time I was able to laugh about it."

Mark Easter, 48, has also found the CMTA Discussion Group's support and advice life-changing. Recently diagnosed with CMT Type 2, Mark knows how it feels to go most of your life thinking you might be the only one with your disease. "They call it a rare disease, and I keep seeing that one in every 2,500 people has CMT. But it feels rarer than that when you don't know what's going on, no one

continued on page 8

ONLINE VOLUNTEERS

continued from page 7

else can help you, and you don't know anyone with it." Mark says the information he gets from the CMTA Discussion Group is better than what he received from most doctors. Mark has appreciated the opportunity to share and learn from others' experiences with CMT. "Just knowing you're not crazy" makes a world of difference, Mark said.

Three of the volunteers who moderate the CMTActive Facebook Group are young women: **Lily Sander, Julie Barnett** and **Katerina Ballsmith**. Lily, one of the youngest moderators at 14, was asked to add new energy to the group last November. She says she immediately fell in love with the low-pressure and encouraging atmosphere previous moderators created. "My CMT makes every day a bit different, therefore altering what activity I can do without becoming fatigued or in pain." Lily shares tips and videos on everything from weight lifting to chopping vegetables to dancing.


Lily

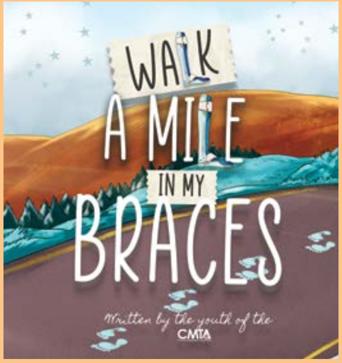
Lily says her most meaningful encounters happen every time she logs onto Facebook. "I am met with encouragement on my posts, and inspiration from others. I have been deeply and truly touched by this page and our community." She continued, "As one of (or the) youngest members of CMTActive, I find myself drawing on the wisdom of those who have lived with CMT longer than I. The older members are living proof that I cannot only live but thrive as an individual with CMT. I find myself often dwelling on the progressive aspect of this disease, but am reminded of the possibilities of life with CMT as I scroll through everyone's unique stories."

Moderator **Katerina Ballsmith**, who wasn't diagnosed until 2018, says "What I learned right away about this disease is the importance of exercise and stretching. Without it, the muscles will continue to weaken and atrophy. That is part of the reason why I still dance. Dance has been my passion


Katerina

BOOK REVIEW: "Walk a Mile in My Braces"

Reviewed by Abby Thompson, 16, from Latrobe, PA.



"Walk a Mile in My Braces" is a book written by 75 kids with CMT to welcome newly diagnosed kids to the Tribe of the Funky Feet. I am proud to be a member of that tribe as well as one of the contributors.

This book is not about the disease and its symptoms, but about the people who live with it. "Walk a Mile" is colorful and full of friendly faces and practical information for living with CMT. While the writers share many experiences, they also have their own unique stories, showing how much this disease varies. The book is even interactive, with blank pages for readers to document their own experiences.

I believe that this book will be an asset to anyone who has been diagnosed with CMT and is feeling overwhelmed, confused or isolated. The book compares the diagnosis to being in a jungle that is completely overwhelming, but also amazing. "Walk a Mile" is a map to the jungle, written by those who've already been there, making it all feel a bit less scary.

The book also includes information on how to connect and get involved with other members of the tribe. Getting involved in the CMTA Youth Association and participating in Camp Footprint has been a huge blessing and really helped me feel better about my diagnosis. The book is full of inspiration and positivity that will leave the reader knowing that while life with CMT can be challenging, it can still be a great life.

Erin Black, 18, wrote one of my favorite passages in "Walk a Mile": "[E]vents in which you get face-to-face contact with your tribe leave you speechless. Camp Footprint, dozens of CMTA conferences, youth outings, and our beloved council retreat have all contributed to my growth on this CMT journey." Everyone she's met on that journey has taught Erinsomething new "or spoke life into me and our mission to live in a world without CMT."

Erin wrote, "I truly can't find words to describe what the youth movement has done for my life. These people make me fight a little harder, be a little kinder and embrace the funk. I don't feel alone, and our goal is to spread that feeling to the thousands of youth living with CMT in this world."

Erin and the other authors share their struggles and frustrations along with their wisdom and advice on accepting their disease and living their best lives. The book is also a valuable tool for parents, friends, family and others who love and care for someone with CMT and want to understand what we go through.

"Walk a Mile" can be found online at Amazon or Barnes and Noble.

for several years before the constant pain and fatigue. I've grown to love dancing, even while sitting in a chair."

Katerina shares exercises in short weekly videos that run the gamut from stretching to chair dancing. "This community is a reminder that even though we all struggle with limitations, we can encourage each other to keep moving and stretching. I believe through groups like these, we all can be inspired not to give up."


Julie

Julie Barnett's background as a fitness instructor and personal trainer qualifies her to serve as a moderator of the CMTActive Group. On and off the CMTActive page, she tries to encourage others to move a little more than they did the day before and strengthen what they still have use of to keep mobile and flexible.

"When people with a common thread come together in a positive way, we can learn from each other," Julie said, explaining, "If a person with CMT thinks they can't do something anymore like ride a bike, but someone shares that they safely ride a three-wheel bike or a different type of two-wheel bike and the person who thinks they can't tries it, then it's usually a positive experience."

If you are interested in volunteering for our online programs, contact Sarah Kaider at sarahk@cmtausa.org.

YOUTH PROGRAM VOLUNTEERS INSPIRE

While the CMTA's Youth Program is just shy of three years old, it has grown exponentially in that time. In 2021, young volunteers raised more than $11,000 with the Second Annual Fund the Starts Venmo Bingo; hosted four Youth Zoom hangouts; launched a Discord Server for gaming; welcomed more than 100 campers to the Sixth Annual Camp Footprint; raised more than $28,000 with the first-ever Dance 4 CMT and published "Walk a Mile in My Braces," by and about young people living with CMT. Volunteers played key roles in each accomplishment.

CMTA Camp Footprint is a free camp experience for kids ages 10-18 with CMT. Part of what makes camp so special are the inspiring, kind, silly and committed camp counselors, the vast majority of whom have CMT, making them the perfect role models for campers.

The counselors clearly get something in return. Counselor **Cara Leith**, the only person in her family with the disease, thrilled to the feeling of finally getting to be around people who understood what life with CMT is like. Before Camp Footprint 2020, she said, her six-year CMT journey was something she had to do alone: "In some ways, it was like everyone's angsty teenage belief was true—quite literally, no one around me understands." At virtual Camp Footprint, by contrast, "Day after day, the screen lit up with over 100 beautiful faces who know exactly what it's like and can understand those pieces of me that I've carried alone without having to say a word. The joy in that togetherness, and the sense of peace that came with seeing other happy, thriving people with CMT across all age groups, was such a gift. I cannot wait to experience it in person one day!"

David Sellers, a middle school science teacher, and his daughter Audrey, who has CMT, were both counselors at Camp Footprint 2021, David for the fourth time. He is perhaps best known for his science experiments, particularly a spectacular one involving Mentos and Coca-Cola. "I feel so very thankful to be a part of camp; each year it fills my soul with joy to be able to help and to witness the campers enjoying each other's company, sharing their stories, coming out of their shells, and finding their camp family."

David knows how hard it is to get students involved over Zoom and was "amazed by how engaged the campers were: Many campers stayed on Zoom all day, fully engaged and excited about what was going on. It is a testament to just how much the campers need this time with others who have CMT."

Karen Brown was also a counselor in 2021. Karen is a triple threat volunteer—branch leader, Facebook group administrator and the CMTA's de facto Camp Footprint goodwill ambassador. When Ibrahim Nwar Al-Mari, father of Camp Footprint's only Egyptian camper, posted on Facebook that he needed help for his 11-year old son Reda, Karen stepped in to handle logistics. Because Reda doesn't speak English, his dad, brother and aunt all translate for him. Karen was there every step of the way, making sure he was fully engaged. He even gave the other campers a virtual tour of the pyramids.

The Youth Council represents another block of volunteers. A dozen members are tasked with fulfilling their mission to connect the youth of the CMT community, to educate, encourage and involve CMT youth in fundraising and to give the youth of the CMT community a voice.

Volunteers work in committees that include social media, special projects, meeting planning and fundraising. Council members are just the tip of the spear, though, and the committees call on hundreds of youth volunteers to implement their goals. For example, 75 members of the youth community contributed their stories to "Walk a Mile in My Braces."

If you'd like to get involved with any of the Youth Program's efforts, contact Jonah Berger at jonah@cmtausa.org.

CMTA | STAR Awards $354,826 For HSN1 Clinical Trial

On Dec. 7, 2021, the Charcot-Marie-Tooth Association – Strategy to Accelerate Research (CMTA–STAR) awarded Dr. Mary M. Reilly and her team at the UCL Queen Square Institute of Neurology in London $354,826 for a clinical trial in patients who have Hereditary Sensory Neuropathy 1 (HSN1).

HSN1, a type of CMT, is a rare autosomal dominant neuropathy that leads to severe sensory and motor impairment. The study will test the efficacy of L-serine in slowing or stopping disease progression in HSN1 secondary to SPTLC1/2 mutations.

Reilly's preliminary studies—together with previous preclinical trials in animals and an earlier pilot study in patients with HSN1—set the stage for a clinical trial of L-serine in patients with HSN1 secondary to SPTCL1/2 mutations.

This will be the first clinical trial to use and validate MRI muscle fat fraction as a primary outcome measure in an inherited neuropathy. The findings will provide direct confirmation of its utility in phase II and phase III clinical trials for CMT and related neuropathies.


Dr. Mary Reilly

CMTA Seed Money Draws NIH Support For Type 2 Gene Editing, Neurofilament Projects

The Charcot-Marie-Tooth Association – Strategy to Accelerate Research (CMTA–STAR) announced Dec. 14, 2021, that two CMTA-funded projects have been awarded additional funding from the National Institutes of Health (NIH), leveraging CMTA funding to secure additional support.

In 2020, the CMTA awarded Drs. Bruce Conklin and Luke Judge of the Gladstone Institutes and UCSF Departments of Medicine and Pediatrics $653,000 to develop the gene-editing technique known as CRISPR for CMT2A, 2E and 2F. The NIH awarded them a research grant to further the development of gene therapy treatments for CMT.

Conklin, Judge and their team aim to develop and validate a therapeutic gene editing platform for dominant CMT2E mutations using human iPSC-based models. They will test mutation-specific editing for two different NEFL gene mutations and develop rigorous phenotypic assays for therapeutic effect in human iPSC-derived motor neurons. Additionally, they will identify sites of common human genetic variation that can be targeted to excise protein coding or critical regulatory regions and inactivate the disease allele (the genetic variant that causes CMT2) in the majority of patients, regardless of their individual mutations. These studies will also provide proof of concept for a strategic approach that can be applied to other forms of dominant CMT2.

Also in 2020, the CMTA awarded The Ohio State University labs of Anthony Brown, PhD, Arthur Burghes, PhD, Kathrin Meyer, PhD, and W. David Arnold, MD, $265,000 to evaluate gene therapy strategies for restoring neurofilaments to diseased neurons in Type 2 mice. Anthony Brown, professor of neuroscience at The Ohio State University, was able to leverage pilot data obtained with a grant from the CMTA to secure an NIH research grant entitled "Restoring neurofilaments to axons in a mouse model of CMT2E" (R21 NS125468). This award will permit Brown and his co-principal investigator, W. David Arnold, associate professor of Neurology at The Ohio State University, to establish "proof of principle" of a gene therapy strategy in NFL-/- knock-out mice, which is a mouse model of recessive CMT2E. The work will inform a more general therapeutic strategy for dominant CMT2E.

All the CMTA's research efforts are consolidated under the banner of STAR—which brings together the world's largest network of biotech research partners, research scientists, clinicians, and patients—and funds more CMT grants than any other philanthropic organization to increase the likelihood of finding a cure. Since 2008, the CMTA has invested more than $17.5 million in STAR, with plans to invest another $10 million in the next few years.

Registrational Phase 2/3 Study of AT-007 In SORD Deficiency Initiated

CMTA partner Applied Therapeutics Inc. announced on Dec. 16, 2021, the initiation of a registrational phase 2/3 study of AT-007 in Sorbitol Dehydrogenase (SORD) Deficiency. The study, called INSPIRE (inhibition of sorbitol production through inhibition of the aldose reductase enzyme), will investigate biomarker efficacy, clinical outcomes and safety in people living with SORD Deficiency treated with AT-007 versus a placebo.

SORD Deficiency is a rare, progressive, debilitating hereditary neuropathy that affects peripheral nerves and motor neurons. It is one of the most common forms of recessive hereditary neuropathy, affecting approximately 3,300 patients in the U.S. and 4,000 patients in Europe. The disease is caused by a lack of the enzyme sorbitol dehydrogenase, responsible for metabolizing sorbitol, which causes sorbitol to accumulate at high levels and become toxic to the body. Sorbitol accumulation results in significant disability, loss of sensory function and neuromuscular dysfunction.

Inhibition of aldose reductase addresses the underlying cause of SORD Deficiency by preventing the conversion of glucose into sorbitol, which can be up to 100 times higher in the blood of patients with SORD Deficiency than in unaffected individuals. In a pilot open-label study in eight SORD Deficiency patients, AT-007 reduced blood sorbitol levels by approximately 66 percent from baseline through 30 days of treatment. The range of reduction from baseline in patients was 54 percent to 75 percent. AT-007 was safe and well-tolerated in all treated patients.

The global registrational phase 2/3, placebo-controlled trial is designed to evaluate both biomarker efficacy (reduction in sorbitol) and clinical outcomes in patients with SORD Deficiency. The primary biomarker efficacy endpoint will measure the reduction in sorbitol after three months of treatment compared to baseline. The primary clinical outcome measure will assess changes in the Charcot-Marie-Tooth Functional Outcome Measures (CMT-FOM) lower-limb function domain, a key measure of mobility and motility in SORD patients that is greatly affected by the disease. Key secondary clinical outcomes include CMT-FOM domain sub-scales, CMT Health Index (CMT-HI) patient-reported outcomes, fatigue assessment and muscle MRI.

APPLIED THERAPEUTICS

Applied Therapeutics is a clinical-stage biopharmaceutical company developing a pipeline of novel drug candidates against validated molecular targets in indications of high unmet medical need. Applied's chief medical officer, Riccardo Perfetti, MD, PhD, said, "The INSPIRE registrational trial in SORD Deficiency represents an important milestone for patients and is the second late-stage program initiated with AT-007." According to Perfetti, "The science demonstrating the role of aldose reductase, and the toxic impact of excess sorbitol, is well-established. We are excited to have achieved proof of concept in the pilot trial and look forward to continuing to advance AT-007 for SORD Deficiency."

CMTA CEO Amy Gray commented, "People living with SORD Deficiency are in urgent need of a therapeutic option. The initiation of the first registrational trial in SORD Deficiency marks an important milestone for the patient community."

Gray added, "Developing partnerships with companies like Applied Therapeutics is central to the mission of the CMTA and important to the CMT community."

If you or someone you know is interested in a free screening for SORD Deficiency, please email: sord@appliedtherapeutics.com

YES, PLEASE PUT MY CONTRIBUTION TO USE IN THE FIGHT AGAINST THE PROGRESSIVE AND DEVASTATING EFFECTS OF CMT.

Please earmark my gift for STAR Research towards:

○ Type 1A Dollar-for-Dollar Match ○ Type 2 Dollar-for-Dollar Match ○ STAR Research (All CMT Types)

YES! I want to make 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

Name____Card #___________________________________Exp. Date___________

Signature______________________________________Address____

City____________________________________State____________________Zip_________Phone______________________________

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

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

7 NEW CENTERS OF EXCELLENCE BRING TOTAL TO 48

The CMTA added seven new Centers of Excellence (COEs) in 2021, expanding the network's reach and giving more CMT patients access to a center. COEs are patient-centric, multidisciplinary CMT clinics where children, adults and families affected by CMT receive comprehensive care from a team of CMT experts. In addition to neuromuscular clinicians with clinical and research experience in CMT, teams can include nurse specialists, social workers, genetic counselors, physical therapists, podiatrists and orthotists. **Here are the newest centers:**

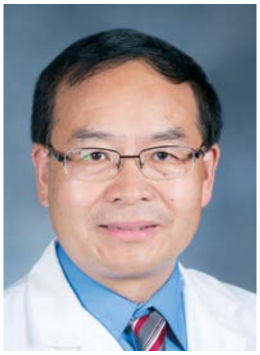
University of Louisville

Dr. Zeng Wang

401 E. Chestnut St., Ste 510
Louisville, KY 40202

Clinic website: https://uoflphysicians.com/service-specialty/neurology/

Appointments: 502-588-4800



Dr. Wang is an associate professor of clinical neurology, chief of the Neuromuscular Division, and program director of the Clinical Neurophysiology Fellowship at University of Louisville. After receiving his MD in China and PhD in Sweden, Dr. Wang completed his neurology residency at Southern Illinois University and fellowship in neuromuscular disease and clinical neurophysiology at Washington University in St. Louis. Dr. Wang's clinical interests are diagnosis and treatments of neuromuscular diseases, including CMT. He also has strong research interests in immunological and molecular mechanisms, and clinical trials for neuromuscular diseases including CMT. He has co-authored over 40 peer-reviewed publications.

Atrium Health Neuroscience Institute

Dr. Urvi Desai

1010 Edgehill Road North
Charlotte, NC 28207

Clinic website: https://atriumhealth.org/medical-services/specialty-care/neurosciences

Appointments: 704-446-1900

Dr. Desai is a board-certified neurologist with added board certification in neuromuscular disorders and EMG. She sees patients across age groups with varied neuromuscular disorders—genetic, autoimmune, inflammatory and degenerative. She leads a large comprehensive multidisciplinary CMT clinic at Atrium Health and an EMG lab accredited by the American Association of Neuromuscular & Electrodiagnostic Medicine. The clinic comprises neurologists, physiatrists, residents, medical students, a physical therapist, an occupational therapist, a respiratory therapist, a speech therapist, a nutritionist and a social worker. The clinic also helps coordinate services of equipment specialists for repair and delivery of assistive devices and works closely with other specialties like cardiology, pulmonary and genetics.

Austin Neuromuscular Center

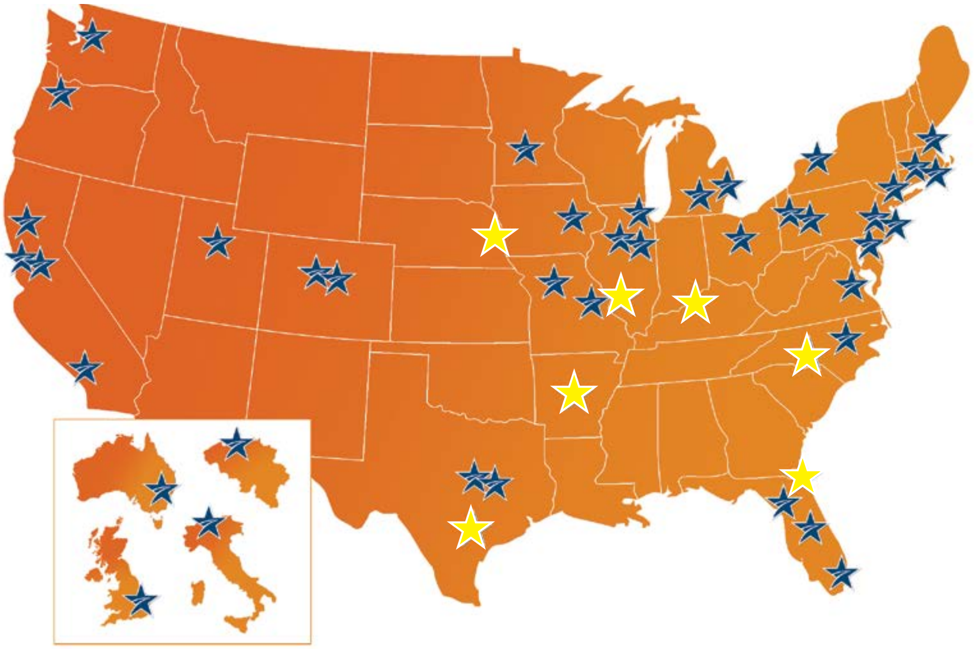
Dr. Yessar Hussain

3901 Medical Parkway, Ste. 300
Austin, TX 78756

Clinic website: https://austinneuromuscle.com/contact/

Appointments: 512-920-0140

Dr. Hussain is board-certified in neuromuscular medicine, neurology, clinical neuromuscular pathology and electrodiagnostic medicine. He completed his fellowship in neuromuscular medicine and electromyography at Washington University School of Medicine/Barnes-Jewish Hospital in St. Louis after his neurology residency at State University of New York's University Hospital in Syracuse. He received his MD from the University of Baghdad College of Medicine in Baghdad, Iraq. Dr. Hussain is an assistant professor in both the neurology and physical medicine and rehabilitation residency programs at Dell Medical School at the University of Texas at Austin. He is also the director of Austin Neuromuscular Center, which provides comprehensive neuromuscular services and educational and teaching opportunities for medical residents and students.



THE NEW CENTERS ARE LOCATED AT:

- **University of Louisville (KY)**
- **Atrium Health (NC)**
- **Austin Neuromuscular Center (TX)**
- **Arkansas Children's Hospital**
- **Mayo Clinic (FL)**
- **St. Elizabeth's Hospital (IL)**
- **Creighton University (NE)**—*details forthcoming*

Arkansas Children's Hospital

Dr. Aravindhan Veerapandiyan

1 Children's Way, Little Rock, AR 72202

Clinic website: https://bit.ly/3HSxAla

Appointments: 501-364-1850

Dr. Veerapandiyan (Dr. Panda) is a child neurologist and assistant professor with specialized interest and training in neuromuscular disorders in children. He is the director of the Comprehensive Neuromuscular Program, director of the CMTA Center of Excellence, and co-director of the Muscular Dystrophy Association Care Center at ACH. He brings his training and expertise to perform electromyography and nerve conduction studies in children. Dr. Veerapandiyan earned his medical degree from the K.A.P. Vishwanatham Government Medical College in India. He completed a residency in Child Neurology at Rutgers University–New Jersey Medical School in Newark, NJ and a fellowship in Neuromuscular Medicine at University of Rochester–Strong Memorial Hospital in Rochester, NY. Dr. Veerapandiyan has published more than 40 peer-reviewed articles and has presented his work at national meetings. He is actively involved in industry-sponsored clinical trials focusing on neuromuscular disorders in children.

Mayo Clinic Florida

Dr. Christopher Lamb

4500 Mellish Road
Jacksonville, FL 32224

Clinic website: https://mayocl.in/3JxbpBL

Appointments: 904-953-0853

The purpose of the multidisciplinary CMT clinic at Mayo Clinic Florida is to identify and care for patients with inherited neuropathies who may benefit from subspecialized care and supportive strategies. Those strategies include the use of orthotic devices, gait aids, physical and occupational therapy and medications. Patients may also be candidates to participate research studies. The clinic consists of a morning multidisciplinary clinic with physical therapy, occupational therapy, genetics counseling, and neurological assessments for returning patients with a confirmed genetic diagnosis of CMT, or other inherited neuropathies. An afternoon clinic serves new or returning patients who have a confirmed genetic diagnosis and want a comprehensive neurologic evaluation for inherited neuropathies, but who may not require multidisciplinary assistance.

HSHS St. Elizabeth's Hospital

Dr. Raghav Govindarajan

3 St. Elizabeth, Suite 5000,
O'Fallon, IL 62269

Clinic website: https://www.hshs.org/stelizabeths.

Appointments: 618-641-5803

The CMTA Center of Excellence at St. Elizabeth's provides comprehensive care focusing on all aspects of CMT. In addition to providing a holistic approach to the management of CMT, it is actively engaged in CMT research. Dr. Govindarajan is board-certified in neuromuscular medicine and in clinical neuromuscular pathology, with more than 100 publications and numerous research grants and teaching awards. He is actively involved with CMT patient care and in clinical research to find new treatments for CMT.

Visit the CMTA website for more information about the CMTA Centers of Excellence. A complete listing of our current COEs appears on page 25.

Pulmonary Complications of CMT Include Diaphragm Dysfunction, Sleep Disorders

Pulmonary complaints are not very common in CMT patients. When problems do occur, they are usually slow in both onset and progression, CMTA Advisory Board Member Dr. Ashraf Elsayegh told attendees at the Patient/Family Conference Nov. 6, 2021.

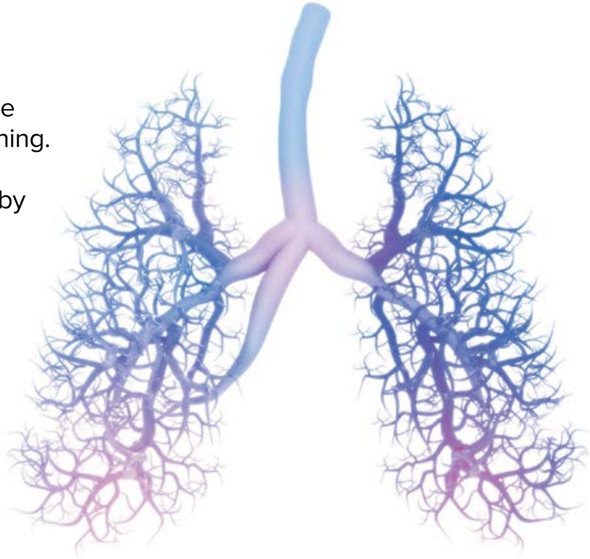
Dr. Elsayegh, who currently practices at Cedars-Sinai Medical Center and is an associate clinical professor at UCLA School of Medicine, explained four different pulmonary complications of CMT–diaphragm dysfunction, scoliosis, vocal cord paralysis and sleep disorders. He also shared the best treatments for each.

Dr. Elsayegh distinguished between diseases of lung tissue and diseases involving the respiratory muscles, which both cause dyspnea. CMT-induced neuromuscular respiratory muscle weakness causes shortness of breath by impairing the chest cavity's full expansion.

Although respiratory dysfunction is not very common in CMT patients, it can occur. An early sign is dyspnea while lying flat. Patients will have obvious shortness of breath or difficulty catching their breath when lying flat that is usually relieved by sitting upright or sleeping at an angled position.

CMTA Advisory Board Member Dr. Ashraf Elsayegh

Diaphragm Dysfunction



As Dr. Elsayegh explained, the diaphragm is the most significant of the multiple muscles that control breathing. Located at the bottom of the chest cavity, it is controlled on each side by the right and left phrenic nerves. If the phrenic nerve is damaged by CMT, the diaphragm will not be able to contract properly, resulting in shortness of breath (dyspnea).

Diaphragm dysfunction can be diagnosed with a pulmonary function test (PFT), which tests the strength and efficiency of breathing. A full PFT is not typically recommended in CMT patients unless there are breathing problems. If abnormalities are detected on initial testing, the spirometry portion (which measures the volume of air that one breathes in and out) can be repeated twice a year. Given that respiratory decline is rare and slow to occur in CMT, more frequent spirometry is usually not indicated.

Another relatively easy way to evaluate diaphragm dysfunction is a sniff test, which requires the patient to sniff while undergoing fluoroscopic imaging so that the movement of the diaphragm can be measured. In general, this does not need to be performed unless surgical intervention on the diaphragm is required, Elsayegh explained.

Finally, phrenic nerve conduction and/or diaphragm electromyography (EMG) can also evaluate diaphragm function. The phrenic nerve study is similar to the nerve conduction study done for the diagnosis of CMT, except the phrenic nerve is stimulated in the neck and the recording is done with sticky pads on the chest wall and lateral rib. It is safe and no more uncomfortable than routine nerve tests.

The diaphragm EMG requires a needle in the area of the diaphragm and carries some small risks. More recently, ultrasound to evaluate the diaphragm has been utilized, though neither of these is necessary unless surgical intervention is needed.

The mainstay of therapy for respiratory decline is noninvasive ventilation (NIV), which delivers pressure to the wearer via a face mask, eliminating the need for an endotracheal airway. Continuous positive airway pressure (CPAP) therapy is not recommended in neuromuscular disease. Although bilevel positive airway pressure (BiPAP) therapy is better than CPAP, newer, more sophisticated forms of NIV are much more comfortable for and much better tolerated by patients.

As the respiratory function declines further, the newer forms of NIV are far superior in maintaining the respiratory system and a proper carbon dioxide (CO2) level. If the patient has reached the point of retaining CO2, they should already be on noninvasive ventilation.

Monitoring blood gases to follow the CO2 level is usually not indicated: It is a painful invasive test that will not add much information or contribute to changes in management. The exception is if the patient has signs of hypercapnia (high CO2), such as altered mental status, headaches or difficulty waking up, at which point measuring CO2 levels with a blood gas test will help in adjusting the NIV settings. Dr. Elsayegh recommended wearing the NIV at night to start so that it doesn't interfere with activities of daily living. If dyspnea occurs, NIV may also be used during the day.

In CMT, as with other neuromuscular disorders, the problem is with weak respiratory muscles—the actual lungs are usually not affected. CMT generally does not lower oxygen levels, though CO2 may go up because of the inability to blow it out of the body. Supplemental oxygen is rarely needed in CMT and may actually be harmful in some patients.

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

Diaphragm pacing—essentially a pacemaker for the diaphragm—has equivocal results at best in neuromuscular patients and has never been tested specifically in CMT patients, Elsayegh said. He recommended discussing the subject with a center experienced in the procedure if diaphragm dysfunction becomes a significant problem.

A weakened diaphragm can also diminish the ability to cough, leading to secretion buildup in the chest cavity, and potentially pneumonia. If coughing is affected, two main forms of airway clearance devices can help: a high-frequency chest wall oscillator, which helps break down secretions so the patient can expectorate them, and a cough assist that can help expectorate the secretions for the patient. The newest device is the Volara System for home use, which helps break down secretions in the lungs, helps the user to expectorate secretions, helps expand the lungs and can be used as a nebulizer to deliver medications that can help loosen impacted secretions.

The most important key to proper pulmonary care is finding a durable medical equipment company and respiratory therapist that specialize in neuromuscular patients. They will help with day-to-day management and adjustment of the noninvasive ventilator and will be an excellent resource for the patient on simple questions involving the equipment.

Scoliosis and Vocal Cord Involvement

Scoliosis may also affect breathing as the curvature of the spine compresses one or both lungs, shrinking and hardening them in an abnormal contour. Lung volumes shrink and the movement of the ribs is impeded with progressive worsening of dyspnea.

Some studies have shown that surgical correction of the vertebral column improves pulmonary function in severely decreased lung volumes, though it usually does not improve symptomatic dyspnea.

Although relatively rare, some patients with CMT can have unilateral or bilateral vocal cord paralysis. Unilateral vocal cord paralysis rarely presents with symptoms, though chronic aspiration of gastric content may occur, resulting in repeated cases of pneumonia. Bilateral vocal cord paralysis can result in stridor (a harsh vibrating noise when breathing) and difficulty breathing.

If a laryngoscopy reveals one-sided paralysis, aspiration precautions should be taken, but no significant intervention is usually warranted. If both vocal cords are paralyzed, resulting in stridor (a harsh, vibrating noise while breathing) and difficulty breathing, a tracheostomy is indicated. These situations are rare and more common in children than in adults.

Sleep Disorders

Sleep disorders are the most common breathing problem Dr. Elsayegh sees in his CMT patients. Both obstructive (OSA) and central (CSA) sleep apnea may present in CMT patients, along with restless leg syndrome (RLS) and periodic limb movements (PLMs).

Sleep apnea is the cessation of breathing (or reduction of respiratory effort) during sleep. Obstructive sleep apnea is secondary to an obstruction of the upper airways, whereas central sleep apnea is caused by receptors in the brain. Symptoms of sleep apnea include poor sleep, frequent nighttime awakenings, daytime fatigue and headaches.

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

OSA, CSA and PLMs may all be diagnosed with an overnight sleep study, but a screening sleep study is not indicated. However, if patients are symptomatic, an overnight sleep study (Polysomnogram/PSG) would be indicated. Because CPAP therapy is generally sufficient to treat obstructive sleep apnea but not central sleep apnea, Dr. Elsayegh usually recommends NIV for all patients with neuromuscular disease and sleep apnea.

The only caveat to this recommendation is for patients who have already received NIV for respiratory dysfunction. If the patient has NIV already ordered, they generally do not also need a sleep study since the NIV will treat the sleep apnea automatically. In addition, most PLMs improve with treatment of the sleep apnea, and NIV is usually all that is needed. Restless leg syndrome is a diagnosis that is made clinically and no testing is needed: Multiple pharmacological treatments are available.

THE CMTA GRATEFULLY ACKNOWLEDGES GIFTS…

In Memory of:

Cheyene Alfino
Mr. and Mrs. Ken Hurst

Dan L. Anderson
Ms. Joan F. Anderson

David Bank
Jerry and Debra Bank

Paul and Mona Barkley
Richard and Peg Hodel

Greg Beeman
Alex, Chris, and Eric Smith
Mrs. Katherine McDaniel
Mr. Will Weisiger

Anna Mae C. Berlin
Colleen Becker

Harris Blauer
Mr. Charles L. Blauer

Edgar Earl Bostick
Mr. Russell Bostick

Lenora Buck
The Family

Caitlin Burkhardt
Nancy Burkhardt

Virginia Rose Carneval
Dr. Mary Carneval

Nadine Cirka
Mr. George Cirka

Cecilia Clemente
F. Richard Clemente
Roseann C. Lanyon

Esther R. Cohen
Mr. and Mrs. Marc Rotenberg

Elwood Cole
Mr. Robert Cole

Avia Daigle
Harold and Susan Shafer

Daniel Crafton
Mrs. Diana Knox

John Dalgliesh
Dr. and Mrs. Nicholas E. Mihelic

Connie Deller
Tony Deller

Charlie Dezern
Gary and Dianne Everhart

Pauline Dinges
Mr. Peter Grassi

Lee Doyle
Mr. and Mrs. Stephen Doyle

Michael B. Dupont
Mr. and Mrs. Theodore Alfond
Mr. and Mrs. George Conant
Onolee C. Dupont
Andrew and Maura Edmonds, Jr.
Mr. and Mrs. Jonathan Estoff
Molly and Clark Lea
Patricia Lucy
Frank Lyon
Jane R. McCaffrey
Mr. Robert Melvin
Ms. Sandra O'Reilly
Mr. Dennis O'Shea
Pinnaclecare International
H. Michael and Margaret Stevens

Donald Dwyer
Glenn and Sarah Dwyer
Richard and Betty Harvey

Mr. and Mrs. Ford Farabow
Ms. Jean Fordis

Henry Friedmann
Esther Friedmann

Sara C. Fuhrman
Colleen Becker

Billie Jean Gabbard
Mr. Julian G. Gabbard

Shirley Garrigan
Mrs. Laurel Brunell
Mr. Michael Garrigan, Jr.
Mr. James Guenther
Ms. Christine Hickey
Mrs. Lola Mitchell
Mr. and Mrs. Ronald Scott
Ms. Linda Walton

Erico Andres Gomez
Berta Gomez

Kathy Greer
Ms. Rebecca S. Floch

Robert C. Harris
Ms. Pamela Lett

Bob and Deb Haas
Mr. Anthony Haas

Joan Heitner
Joan M. Greenbaum

Robyn Henley
George and Sandra Carter
Douglas and Lynne Cook
Mr. and Mrs. John Evans, Sr.
John and Nancy Freeze
Mallick Mechanical Contractors, Inc
Straub Construction Co., Inc.
Mr. Gerald Therrien
Ms. Michelle Wyrick

Gayle Hibbard
Ms. Julie Cooper

Edward Hughes
Pamela Tice

Don Johnson
Mrs. Linda Johnson

Peter L. Johnson
Mr. Kurt Johnson

Flora Jones
Cindy Chesteen

James Ervin Jones
Curtis and Karen Crick

Steve Jumper
Gary and Dianne Everhart

James Keenan
Mrs. Ann M. Gonzales

Lois Kesack
Virginia Benner

Kenneth Konstalid
Friends at the Thruway

Sharon LaFontaine
Mr. Robert LaFontaine

Drucilla Lake
Ms. Linda C. Whipp

Beverly Lash
Mr. Robert Fine
Mr. Dan Honig
Mr. James Malloy
Mr. Michael Trivers
Mr. and Mrs. Bruce Weinstein

Elliott Levitch
Felice Viggers

Jane Ludwig
Mr. Bill Mertens

Audrey MacDonald
Mr. Douglas W. MacDonald

Edward Madeja
Linda Madeja

Kathleen Mannion
Mary Hunt

Michael McClellan
Ms. Jeanne O'Connor

Velwood McDaniel
Linda McDaniel

George Meyerson
Madeleine Policzer

Richard Mikesell
Ms. Elizabeth Brown

John Paul Morris
Mr. and Mrs. John Snow

Colleen Murphey
Mr. and Mrs. Donald Murphey

Mary Neeper
Rev. and Mrs. Earl Brooks

Kelly J. O'Neil
Marvin D. and Jeanne F. O'Neil Family Fund
Mark Sorensen-Rabo AgiFinance

Julie Ols
Polly Maziasz

Barbara Palmer
Kevin and Jodi Rose

Melvin Palmer
Ronald and Linda Simpson

Rocco Louis Pignone
Mrs. Rosanne Schloss

Suzzanne Mary Poore
Mr. Stephen Humberstone

Joseph and Nida Raffaelli
John Raffaelli

Harvey Rappaport
Mrs. Glenda Chernoff
Mrs. Jill Rosa

Greg Reuther
Connie Griner
Sue Reuther
Tony Reuther's Friends at DCFS

Gladys Richardson
Mr. Larry Richardson

Charles Rotenberg
Sheryl R. Cohen
Mr. and Mrs. Marc Rotenberg

James "Greg" Reuther
Mr. Robert Burrus
Mrs. Sara Reuther

Rebecca Sand
Stephen Sand

Joyce Schmitz
The Browning and Belli Families
Mrs. Ruth Wallin

Pat Simonetti
Julia Ann Volpe

Herbert Sperry
Kuebler Family Foundation

Nancy Sponaugle
Mr. Ronald Sponaugle

Duffy A. Stelly
Mr. Mark Stelly

Charlotte Stilwell
Mr. Joseph Stilwell

Bernard Strittmatter, Jr.
Mary Strittmatter

Dorothy Susman
Mrs. Karen Scacco

Mary K. Taylor
Ms. Jan Miller

Norman Taylor
Larry and Karen Prada

Dr. James A. Thomas
Drs. Richard and Rhonda Soricelli

Dolly Thompson
Robin Thompson

Jeannette Wegener
Brad and Lynn Herman

Betty Weigle
Mrs. Beverly Buhl

Tom Weseli
Ms. Elizabeth Weseli

Lois Williard
Mr. Steve M. Williard

Sweetpea Worden
Mr. and Mrs. David Kamp

Melissa Yeager-Pellinger
Mr. F. Richard Clemente

Anthony Zahn
Emily Maitin

In Honor of:

Kate and Maya
Mrs. Meghan Kollas

Kelsey
Mr. Dean Jarman

Luna
Mr. Larry Kane

Taylor
Elmer and Vivian Herman (aka Grammy and Papa)

Arianna Addis
Mr. and Mrs. Rodney Bright

Austin Anderson
Mrs. Rita Anderson

Iris Anderson
Ms. Rochelle Morrison

Edward Arnold
Lannon Twomey

Richard M. Bales, Jr.
Susan Richardson

Scott, Courtney, and Brooke Bennett
Mrs. Nene Simmons

Roy F. Belhke
Mrs. Mary Kate Donais
Nancy and Craig Tanner

Jonah and Amelia Berger
Mr. Mitch Brandt

Erin Black
Ms. Nancy Douthwaite
Patricia Harrell
Mrs. Kathy Rigdon
Ms. Peggy Twohig

Danny Block and Gina Edick
Ms. Julie Hubbard

Jeanne Boehlecke
Ms. Elena Vizvary

Rev. Earl Brooks
Dr. Nick Landavere
Dan and Cindy Mohney

Karen Brown
Dr. Kenneth Humphreys

Madeleine Brown
Mel and Mary Ross
Robyn and Bernard Ross-Squirrell, Jr.

Grace and MaryBeth Calderone
Katherine M. Gibson

Linda Campbell
James Campbell, Sr.

Michael Casey
Mary Jean Piraino

Barbara Castle
Dr. and Mrs. Thomas Bird
Louise and Earl Dudley

CMTA Staff
Mr. Bob DeRosa

Hazel Coldiron
Mr. Matthew Coldiron
Mr. Justin Floyd
Mr. Bart Jones
Mr. Jeremy Smith
Mr. Eric Watson

Barry Cooper
Sheila Deannuntis

Sheila Deannuntis
Dr. Patrica Loudis

Dr. Frank Dingwerth
Sherry Brown

Roy Diton
Martin and Catha Abrahams

Linda Dobosh
Eric Brink

Beth Dorin
Mrs. Denise Maryse Dorin

Larry Dow
Richard and Dorothy Stoll

Kate and Harrison Drews
Dr. and Mrs. Tim and Ann Fisher

Michael Driedger
Jane and John King

Renee Dudovitz
Helene and Jay Eichler

Kayden Emma Dunkin
Mr. Kevin Dunkin

continued on page 17

Newest Member Joins Legacy Society And Another Angel Gets Her Wings

Even before Jeannie Zibirda joined the Legacy Society, the Atlanta branch leader was one of the CMTA's guardian angels, aka volunteers.

Jeannie had followed *The CMTA Report* since the early 1990s, and quickly signed up when a local branch formed in 2007. "It was fun to meet so many people from all over Atlanta," she says of those early meetings. "We sat around and took off our shoes and socks and compared feet. What an awakening to see so many people with my feet!" Jeannie stepped up to lead the branch when the former leader left.

Like the other branch leaders, Jeannie had to pivot when the pandemic came along. But, she says, "The CMTA stepped up to give us so many resources on Zoom! I've learned so much because of their dedication."

Jeannie says the CMTA's staff, volunteers, board of directors and researchers all "seem to be on fire to keep working for all of us to find a cure." The CMTA is also the heart of helping patients with resources, online conferences and newsletters, she noted. "I support all of what the CMTA does and the Strategy to Accelerate Research (STAR) is getting us closer to a cure."

Jeannie graduated from Florida State University in 1977, then moved to Atlanta, where she earned a master's in nutrition. She spent the next 10 years in nutrition support, then started a career educating surgical and medical residents about enteral nutrition. She went on to gastrointestinal diagnostics, overseeing pharmacology, rheumatology and pain management with three different startup companies. Her final position was in specialty oncology and neurology. She retired five years ago.

Jeannie was diagnosed with CMT1A after a neurologist friend shook hands with her father, then 60, and immediately told him, "You have CMT." He then looked at Jeannie's feet and said "So do you." Her younger brother and aunt have it as well.

Post-diagnosis, Jeannie continued water skiing, snow skiing and jogging. She ran a 10K, the Peachtree Road Race, in 1998. She had a number of orthopedic surgeries, including having the arches of both feet lowered.

Jeannie is leaving a bequest to the CMTA because she's "honored to be a part of this group" and wants to "leave some money that will help raise more money to continue research for a cure and support all the aspects of CMTA to help the community." She adds that everyone who can should consider putting the CMTA in their will—whether it's a fund or a certain percentage of an account, which is what she did. Like an angel, she'll be watching over the CMT community for years to come.

Plant the seeds of change and watch the chances of a cure for CMT grow.

By investing in the CMTA's Legacy Society, you can ensure that children with CMT will grow up with the hope of a world without CMT.

A bequest can be made in a number of ways:

1. You can donate a specific dollar amount or asset to the CMTA.
2. You can donate a percentage of your estate to the CMTA.
3. You can donate to the CMTA from the balance—or residue—of your estate.
4. You can designate the CMTA to receive specific assets.

Making a gift to the CMTA's Legacy Society helps fund research that is already beginning to yield a harvest of promising results, with more on the horizon. With your kindness and generosity, the promise of a brighter future is close at hand. What will your legacy be?

With your support, the promise of a brighter future for many is close at hand.

cmtausa.org/legacy

To learn more or to have a confidential conversation, please call Director of Development Jeana Sweeney at 800.606.2682

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

Tracy Adamson
Scott Allen
Christian Ayoub
Aaron Baker
David Balbi
Mr. & Mrs. Stanley G. Banner, Jr.
James Barker
Chavelle Bazo
Brendan Bennett
Robert Bourn
H. Lorna Brand
Deborah D. Breuer
Dr. & Mrs. William Buchanan
William Buchanan
Mr. & Mrs. Walter Carroll
William Chandler
Peter & Cynthia Cherpes
James D. Clark
Mr. & Mrs. David Coldiron
Peter T. Cole
Cheryl E. Collins
James Copeland
John H. Cordonnier
Diane M. Covington
Kelly Demonte
Laura Dillon
Marilynn A. Dodge
Jacky Donahue
Elaine K. Donovan
Teri Drapeau
Mr. & Mrs. Bruce Egnew
Mr. & Mrs. Rick Fansler
Mitchell Fedde
Tera D. Fey
Jim Fiorentino
Mr. & Mrs. Efthimios Foundakos
Vanessa Franco Lopez
Arya Fritz
Mr. & Mrs. Stephen Giles
Amy Gray
Rob Greenstine
Mr. & Mrs. Jay S. Grife
Mark Groenink
Diane Gross
Randy Gunn
Mr. & Mrs. David Haines
Kathryn Harshaw
Rev. Pamela C. Hawkins
Mr. & Mrs. William Helmke
Diann Jackson
Mark Johnsen
Doug Kampe
Drs. Craig & Andrea Kesack
William J. Kesack
Frank S. Kramer
Jane & Joe Krukar
Scott Kuhl
Ella D. LaFara
Doug Lane
Nancy L. Leets
Mr. & Mrs. Matt Lindsay
Ronnie Mendoza
Mr. & Mrs. Jacob Mersing
Mr. & Mrs. William W. Millar
Alba Moratinos
Fred A. Mueller
Cara Natale
Diane Navarrete
Cody Nelson
David A. Nelson
Michael Newell
Gary S. Oelze
Jeannie Palmero
Cristina Penas
Angelina Pennington
Suzanne M. Powers
Jonathan Preston
Susan Rems
John Rizzo
Chelsea Rose
Raymond Roth
Mary Russell
Mr. & Mrs. Travis Russell
Mr. & Mrs. Michael Sayward
Karen Scacco
Mario Scacco
Mr. & Mrs. Richard N. Schaeffer, Jr.
Donald Schlosser
Margarita D Shackelford
Debbie & Steve Sinclair
Bruce K. Spackman
Raymond & Ann Stanhope
Joyce A. Steinkamp
Jim Stetor
Richele Stroop
Barbara Stuck
Mr. & Mrs. Brett Taylor
Patricia L. Tedford
Virginia Thiel
Dr. & Mrs. Thomas Twaddell
Lynn C. Upton
Alexander Van Riper
Angela Vandersteen
Bruce Vieira
Judy Weinsheimer
Robert J. Weis
Gary E. Whitney
Mr. & Mrs. Mark R. Willis
Rebecca Willis
Zachary Willis
Dr. Robert C. Wills
Daniel J. Woltjer
Chansik Yang

"When we found out about Kaelyn's diagnosis, her mother and I researched everything we could about CMT. The CMTA was the best organization we found for information and support. We wanted to support the CMTA community and research any way we could. We know we have a lot more to learn and look forward to supporting the community any way we can."

—DAVID AND SARAH BALBI

"Do something.
Lead, follow or
get out of the way."

—Anonymous

We led and happily you followed. You outmatched the Warfield Family $500,000 challenge. Together, more than 400 of us raised more than $1.1 million for CMT1A research. We all did something incredible.

There were huge gifts and middle-size gifts and many wonderfully thoughtful donations. This generosity happened because someone took the lead, and so many others—friends, family and total strangers—realized the need to push funds into research for a cure for CMT1A. We cannot thank you enough.

We have always tried to "Do something." At first it was volunteering at our children's schools or as scout leaders, then serving as leaders at our church and on civic boards. As our children grew up and started their own families, Missy's CMT was slowing her down, so we focused our financial giving on helping the CMTA, the leader in driving CMT research.

We led once again when we formed the CMTA branch on Maryland's Eastern Shore. There are three generations and five people with CMT1A in our immediate family of 12. It seemed only right to help push research to the goal line of a cure.

We are asking you to lead too. Make any gift a "matching" challenge. It does not need to be a large amount, simply a challenge that invites others to help. Soon we all will be leading and encouraging others to follow.

You gave so generously. The CMT community thanks you and we do too.

—Missy and Seth Warfield


Missy and Seth Warfield

THE CMTA GRATEFULLY ACKNOWLEDGES GIFTS...

In Honor of (cont.):

Ehredt, McCarthy and Chee Clan
Lewis M. Esther Perlstein Family Fdtn

Jaden Ellman
Ms. Sydney Ellman
Mrs. Sylvia Spector

Steven Ericson
Charles and Janice Thompson

Dianne Everhart
Gary Everhart

Jason Everhart
Gary and Dianne Everhart

Gail Feeney Coyle – "Happy Birthday!"
Marian Bakken

Quinn Fernandes
Molly Fernandes
Ms. Rachel Sitta

Herb Fey
Patricia Fey

Travis Flaxman
Ms. Pamela Weiss

Jake Flores
Mr. and Mrs. James Rogers

Friends Of Dignity Health - Las Vegas
Mr. Douglas Eck

Karen Garr
Ms. Suellen Beaulieu

Kenneth Glover
Mrs. Stephanie G. Evans

Judy Galle
John Galle

Andy Geary
Mr. and Mrs. Marinus DenDulk

Sarah Gentry
Erica Haas

Lori Gordon
Michele Vezina

Neal Gossard
Judy Gossard

Sonny and Stella Granger
Mr. Chris Palmer

The Matthew and Yve Greeves Family
Mrs. Judith Kelly

Peter Gunther and Paulette Thabault
Penny Marwede

Courtney Halldorson
Susan Guarinello

Evan Hedges
Kim Misener

Christine Helmke
Bradley Silverman

Landry Heyman
Mrs. Marcia Probasco

Dawn Higginson
Caroline Lydon

Nicholas Houghtaling
Mr. and Mrs. David Houghtaling
Ms. Geralyn Kilcoyne

Joshua Hubbard
Ms. Judy Tung

Lori and Stephen Joyce
Mr. Jeffrey Joyce

Peggy Jumper
Gary and Dianne Everhart

Nancy Kennerly
William Kennerly

Miriam Kleiman
Ms. Ladeene Freimuth

Mr. and Mrs. Robert Kleinman
Mr. Thomas Killeen, PC

Andrea Kodsi
John Kodsi

Jo Koenig
Mrs. Phyllis Stavale

Alana Kohler
Mrs. Robin Kohler

Zach Korowitz
Alan and Ruth Korowitz
Eric Wollman, Esq.

Diane Kosik
Janet Sandor

Kennedy Kraemer
Georgette Hartner

Frank Kramer
Elaine and Mel Rosenburg
Sheffield Van Buren

Jane and Joe Krukar
Ms. Mary Ruetten

Maria Laude
Ms. Kathryn Peters

Maddie Leard
David and Kristin Leard

Ariel Levitch
Felice Viggers

Hope Lindsay
Carole Carlson

The McGovern Family
Barbara Farquharson

Kaya Lakshmi Medley
Dr. and Mrs. Lachman Chablani
Ms. Raisha Lavigne

Steven Lamb
Mary Beth Lamb

Everly Langevin
Lucie Langevin

Anthony Lapinski, Sr.
Ms. Keri Lapinski

Nicholas Larkin
Mr. Louis Campbell
Mrs. Jennifer Larkin

Marci Laude
Ms. Marcia Marino

Richard Lehmann
Marcia Lehmann

Debra Martin
Linda L. Gust

Betty and Barrie Martland
Mrs. Bonnie Anderson

Aeden and Austen Mason Hayes
Ms. Anne Marie Mason

Michael McClellan
Mrs. Carol Ryerson Greeley

Urian Mejia
Nicole Smith

William Millar
Mrs. Holly Noel

Alex Miller
Mr. Patrick D. Miller

Mary Elizabeth Mirical
Cynthia McKenna

Carter Mortensen
Keith and Sharon Mortensen

Margaret Mullery
Mr. Steven Mullery

Louise Myers
Mr. Raymond Betzler
Peter and Patty Malloy

Sue Nagle
Mr. and Mrs. Marinus DenDulk

Julia Norcross
Mrs. Janet T. Gleason

Frank Northrop
John Northrop

Lia O'Sullivan
Ms. Shelby Kelela Jones

Peter Olson
Mrs. Kerrie Renter

Shannon Pace
Maria D. Murphy

Tessa Pate
Melody Ann Ross

Barbara Peisch
Edward Stimer

Katherine Pendo
Ms. Jennifer Birdsall

Orval Peterson
Dianne Martenson

John Phleger
Richard and Jane McCurdy

Bobby F. Pitts
Elizabeth Pitts Jones

Doreen and Ashley Pomykala
Ms. Virginia Thiel

Kenneth Raymond
Barbara Roberts

Julie Reynolds Walker
Mrs. Carla White

Vittorio Ricci and Family
Brett and Elizabeth Joseph

Laurel Richardson
Carolyn Johnsen

Rachel Rivlin
Mr. and Mrs. Fred Lasky

James R. Robinson
Ms. Connie Kidd

Bruce Glenn Rowe
Clay Battle

Deborah Rozanski
Andrew Volovar

Payton Rule
Mr. Matt Scott

Evohn Rutledge
Hannelore Freundlich

Prudence Ryan
Joan and Tim Porta

Lily Sander
William A. Lengyel

Richard Schaeffer Jr.
Mrs. Christine Miller

Dr. Thomas Scherer
Richard T. Brinkmann

Joseph Schlenger
Peter and Patty Malloy

Jennifer Scott
Mrs. Stephanie G. Evans

Clark Semmes
Dr. Claudia Beverly

Ava Shaffer - "Happy Bat Mitzvah!"
Mrs. Selma Aaronson
Bill and Kay Gillet
Ms. Lori Gordon
Mr. William Helmke
Mr. and Mrs. William Helmke
Mr. Keith Helmke
Mr. Steven Helmke
Joseph and Judith Kassel
Ms. Cynthia Lane
Mr. and Mrs. Burton Masnick
Mrs. Teri Ofarrell
Mrs. Betty Shaffer
Mr. and Mrs. Sol Sturm
Mr. and Mrs. Frank Wurzel

William Sharp
Don and Mary Ellen Sharp

Xavier Shaw
Cheryl Collins

Samantha Sheriff
Nancy Headrick

Amy Solomon
Mrs. Maura K. Harrington

William N. Smith
Mr. Joseph Rhodes

Bill Steele
Ms. Mary Deganhart

Kathleen Stees
Richard Stees

Edward Stimer
Ms. Diane Wittenberg

Kieran Strei
Niamh M. Strei

Arnold Sucher
Joan Charney

Douglass Sutherland
Michael Ganino

David Tannenbaum
Ms. Jennifer Scarlott

Taylor and Noah Teague
Mr. and Mrs. Travis Teague

Grayson Thomas
Ms. Deborah Brewer

Michelle Tressel
Mr. and Mrs. Robert Elliott

Kayla Tschida
Ms. Kristin J. Tschida

Herb and Barb Turner
Mr. Dustin Turner

Steven Turner
Mr. and Mrs. Elliot Turner

J. A. Valazquez
Leonor Deetjen

Peter Warfield and Family
Salliann and Burt Daughter

Dr. Peter I. Warfield and Dr. Jessica Long
Daniel and Gena Feith

Seth and Missy Warfield
Salliann and Burt Dougherty
Lynn and Bob Holtzclaw
Mr. and Mrs. John Hunter

Edie Rose Ward
Mr. and Mrs. Wes Ward

Lynne Marie Warner
Dr. Ashley Mullen

Anita Weaver
Enon Valley Church of Christ

Debi Weber
Dr. Brian Hoefer
Ms. Carole Lukens
Ms. Kathryn Peters

Lisa Weiner
Brian Rea
Michael Weiner
Susan Weiner

Maureen Weir
Catherine Klymec

Robert Weis
Joel E. Mandel MD Foundation for the Arts and Sciences

The Weiss Family
Francine and Ronald Cohen

Ryan Whitman
Charles McMurray Company

Diane Wittenberg and Family
Barbara Peisch
Edward Stimer

Patricia Wood
Mark Wood

Dennis Woodard
John and Noreen Burke

Larry and Connie Zahn
Mr. and Mrs. Robert Thorpe

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

○ $50 ○ $100 ○ $250 ○ $500 ○ $1,000 ○ Other: $____________

○ **YES, I WANT TO BE AN INNERVATOR—**Please 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/donate

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

Name____ Card #__________________________________ Exp. Date____________

Signature_____________________________________ Address____

City____________________________________ State___________________ Zip_________ Phone____________________________

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

A Tale of Two Parents

Parenting is never easy, but CMT adds even greater challenges. In this issue we bring you stories from the perspectives of a parent with a child who has CMT and of a parent with CMT.

Parenting a Child with CMT

By Abby Havermann

It was a day like any other when I hustled Jaden along the sidewalk on the way to second grade. My mothering mind ran on autopilot: Brush your teeth! Get your shoes on! Did you pack a water bottle? How hard is it to be on time? Suddenly, time slowed as I watched Jaden, now 11, fall over.

I didn't run back to hoist him up, I didn't dust him off. I stood watching him. As he climbed to his feet, I saw an image in my brain: Jaden loping next to me. Loping, loping, always loping to keep up.

That's when we began to pay attention to how he walked. I remembered how hard it was to teach him to ride a bike. It registered how often he complained of growing pains.

On bad days, when I reflect on the stress of my pregnancy, I'm sure that Jaden's CMT is my fault. On mediocre days, it's not that I know why this has happened, it's just that I know it's teaching me something. I wish he didn't have to suffer for whatever the lessons may be. On good days, I know that his soul chose me and mine him and the why doesn't matter.

No matter the emotions I wake up with, however, the choice of whether I live out a bad, mediocre or good day is entirely up to me. I decide what thoughts I allow myself to indulge.

First, I must identify what horrible stories I'm telling myself about Jaden's future. I must become conscious of all the ways I'm beating myself up for not being better, not doing better, for not knowing what to do.

Then I must pour different information into my brain as if guzzling orange juice and a raw egg to treat a hangover. Maybe it's an uplifting podcast, maybe it's an article on mindset, maybe it's an Al-Anon meeting, a webinar on gratitude or maybe it's just blasting dance tunes. I might have to go on a bike ride or meditate or practice a breathing tool, or all the above.

Most often these days, I can turn it around in short order if I'm paying attention. But there are times when it can take the better part of a day to turn myself right side up again. That is part of raising a child with a disability.

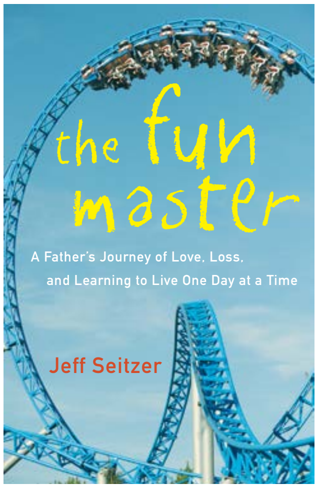
The biggest danger I face is when I go unconscious, the times I'm not paying attention to how I'm thinking and feeling and acting. That is when things turn ugly, not just for myself, but for whoever lives under my roof.


Abby and her son Jaden

I'm not ashamed to crawl into bed for a day, but even that must be done with awareness. It's an act of self-love, an attempt to rejuvenate. It is not a self-indulgent pity party, and it is certainly not an excuse to self-flagellate for being a sloth. Neither of those things are loving to me, and if there is one thing my kid doesn't need, it's a self-loathing mom whose energy is sucked by her own guilt and shame and victimhood.

My goal is no longer to understand why Jaden was dealt his hand. My goal is to be the best version of myself I can be. Otherwise, how can I ask the same of Jaden when he has so many more mountains to climb than me? It is not whether we win or lose the battle on any given day, but the willful intention we bring to each one.

Abby lives in Colorado where she is a speaker, author and transformational coach.



Jeff Seitzer was a self-admitted self-involved academic struggling to cope with CMTX and the residual effects of encephalitis when he took over the care of his son Ethan, who was born in fragile health. Ethan thrived despite multiple surgeries, hospitalizations, serious breathing and swallowing problems, hearing loss, and a challenging social environment. His instinct for fun proved the perfect complement to Jeff's determination to live life fully. Ethan drowned in August 2010 at age 9, though not before he, his sister Penelope and their mother Janet taught Jeff that the true path to happiness was putting other people's needs before his own—and living in the moment rather than trying to control it. This is an excerpt from Jeff's forthcoming memoir, The Fun Master: A Father's Journey of Love, Loss, and Learning to Live One Day at a Time *(SparkPress August 2022).*

When the Parent Has CMT

By Jeff Seitzer

Early on a cold February morning, I stirred my coffee while Ted, the owner of my local café, told me about the plumbing problems that had caused him to close for a few days. Ethan and I had spent a lot of time in his establishment, reading books and chatting with the regulars. It was kind of a community center for us. I was relieved he had reopened that morning.

"Hope to see Ethan with you again soon," Ted said, as I turned to leave.

"Later today if I have anything to say about it."

I pushed the heavy door open with my shoulder and stepped out into a school of hurried commuters. I sipped my coffee while they weaved their way around me, then quickly crossed the street and entered the pharmacy.

"Back again so soon?" the pharmacist said. "That boy needs a lot of medication. How is he?"

"He's great, thanks," I said over my shoulder, as I hustled out. As always, crackling with nervous energy, I was anxious to get a walk in before having to return home. I had learned my lesson the day before—12 hours of unrelenting baby care without stepping a foot outside. I adjusted my scarf against the strong breeze, thinking longingly of a baby-care version of the famous Marxist formula. Instead of working a lathe in the morning, fishing in the afternoon, and doing literary criticism at night, one does baby care in the morning, exercises and reads in the afternoon, and socializes at night.

I rounded the corner by the library and stopped at the alley for a standing car. It merged into traffic, and it was like a curtain in a play had been raised, revealing a different winter scene. Most of the sidewalks I had traversed that morning had been well-shoveled. The long block before me had not been cleared and appeared to be completely trampled. Small, hard-packed mounds of snow caused my ankles to buckle; deep ruts with hardened edges often caught and held the ball of my foot. Fearing a sprained ankle or perhaps a fall, I reversed course and went to the next block.

Even on the smooth pavement, my feet twisted and turned with each step, particularly my right foot, which was weaker and more deformed than its companion. I inherited CMT from my mom and my symptoms were comparatively mild, according to a long list of experts. The pain in my feet suggested otherwise and reminded me of how much I missed my exercise talisman—lap swimming—because it reduced the high-voltage electric current that coursed through me, without taxing my legs.

I hadn't been near a pool since Ethan was born. There wasn't enough time in the day. We spent every spare minute caring for him, and the complexity of his treatments and risk of infection kept us from hiring babysitters. Stretching helped counteract the effects of the disease by forcing bones, tendons and muscles into their proper position. But it was hard to find time even for that recently. My feet got worse and worse.

At the corner of our block, a black lab added some color to the feet of a snowman some neighborhood kids and I had built.

"Hey!" I yelled to the owner, hurrying toward him. "Can't you find a tree or something?"

"Oh, sorry," he said, wide-eyed before yanking on the dog's collar, too late to prevent our canine visitor from leaving a yellow trail across the snowman's feet.

Farther down the block, a neighbor tried to pull her car out of the deep snow. I pushed from behind as she rocked back and forth, spraying sludge-covered snow all over my pants and boots. Suddenly, the vehicle jumped the rut, then lurched backward, knocking me back into a parked car before it sped forward and careened into the street at an angle.

"Thanks a million," she said through the open passenger window. "I'm sorry to hear that Janet has gone back to work. We could all use more time with our kids."

"Indeed we could," I responded, waving as she drove down the street.

Walking toward our house, I recalled Uncle Maury asking me after I got my first teaching job if it bothered me that Janet made more money than I did. "Not nearly as badly than if she made less," I countered.

That was before we had Ethan. It mattered a lot more now. If I taught full-time, Janet would still have to work at least part-time or probably even full-time to make ends meet. So, we were stuck in this difficult position, with her having too much career and me having too little.

As I trudged slowly up our front steps, I saw Janet inside gathering up her things to go to work. "The countdown begins," I said under my breath.

"Good luck today," she said as she zipped her backpack. "I hope you guys will be okay."

"Like Ozzie and Harriett," I said with a sigh as I watched her drive down the alley before reading Ethan's care regimen for the day.

Jeff teaches at Roosevelt University and has published in Adoptive Families Magazine, The Omaha World Herald, Brevity Nonfiction Blog, Hippocampus, and elsewhere. Read more at **www.jeffreyseitzer.com**


Janet, Ethan, Jeff and Penelope in 2010

WHAT'S ON YOUR MIND? ASK DAVID.

David Tannenbaum answers questions from readers in his column "What's On Your Mind? Ask David" regularly in The CMTA Report*. David has an LCSW degree and has been a psychotherapist in New York City for the past 30 years, specializing in helping others with the task of growing emotionally and spiritually through physical challenges. "My CMT has been my greatest challenge and my best teacher in life," says David.*

Dear David,

I have CMT and I strongly suspect that my adult sons, aged 23 and 25, also have it. They know that I have some issues walking, but they've never really questioned my mild symptoms. I feel so guilty for passing the CMT gene on to them, and when I look at my elderly father—who is very affected by the disease—I feel sick and sad. Now that they both have girlfriends, I need to tell them about the CMT, but I don't know how to do it without them being very angry and disappointed that I didn't do it earlier. What would you advise?

David Replies:

If I had one piece of advice for CMT parents, it would be to let go of your guilt about passing the CMT gene down to your children NOW! Guilt is an emotion as old as human societies, but no parent would intentionally give their children CMT.

Even though guilt can compel us to altruistic action by allowing us to empathize with those in need, it can also be debilitating, filling us with a deep sense of shame that shuts us off from others. In psychological terms, guilt is an internal punishment process: You've done something bad so you need to be punished. Holding on to feelings of guilt is how we keep ourselves moral. Before scientists discovered the specific genes that cause CMT, we might have blamed it on an act of God. That's harder to do now that we know how it is passed on. In some ways we feel worse. Because feeling guilty about a genetic disease is an irrational feeling, the best thing to do is think about it rationally through talking with friends, family, a psychologist or a religious advisor. Keep in mind that the problem doesn't come from the initial feelings of guilt; it comes from ruminating over the same guilty thoughts over and over. We find it uncomfortable when we feel helpless and guilt can give us back some degree of control. We can eventually work through our sadness and feelings of helplessness, but guilt simply keeps us stuck.

It does surprise me that your children never really questioned your dad's illness, but denial can be a powerful mechanism to avoid feelings of discomfort. I myself did not show major symptoms in my teens and I'm glad I didn't know before I needed to. But your sons should know now to be able to make or adjust plans they may have on several fronts. Although I understand your concerns about their anger and disappointment for not telling them sooner, you have not done irreparable harm by not telling them before this. Your wanting to protect them from this knowledge is also about your love for them. Often, it is only when our guilt gets uncomfortable that we take action to free ourselves of these feelings. If your sons are angry with you, the best thing to do is simply apologize for not telling them sooner. None of us is perfect and we need to accept that. Allowing yourself to be open to whatever happens is an act of courage. Forgiving yourself is an act of love and will benefit everyone around you, especially your children.

Letting go of your guilt will help you be more present for them and give them the kind of support they might need in the future. When CMT is not spoken about it can lead to feelings of shame that can ultimately restrict our lives. What is hidden or unexpressed can manifest in depression or anxiety. Coming out of the CMT closet will ultimately bring a sense of freedom to all.

SAVE THE DATE

CMT PATIENT & RESEARCH SUMMIT

VIRTUAL • SATURDAY, OCTOBER 1, 2022

CMTA
Charcot-Marie-Tooth Association

ACCELERATING RESEARCH. EMPOWERING PATIENTS.

BRANCH NOTES

THE CMTA WELCOMED FOUR NEW BRANCHES IN 2021:

- **Northern Alabama,** led by Kimberly Parry (kmiddletonparry@gmail.com)
- **Central Michigan,** led by Megan and Jonah Berger (jonah@cmtausa.org)
- **Manitowoc, Wisconsin,** led by Barry Hett (barryhett@gmail.com)
- **Guadalajara, Mexico,** led by Tomas Luis Lopez Valenzuela and Guadalupe Valenzuela Cazares (tomaslopezv@hotmail.com)

NORTHERN ALABAMA

Eight people gathered for the first Northern Alabama branch meeting on Nov. 16, 2021. Fitness instructor and CMTActive Administrator Julie Barnett shared stretching and exercise tips for people with CMT and branch members shared feedback and their experiences.

JACKSONVILLE, FL

Several branch members gathered virtually on Dec. 4, 2021. After introducing themselves and getting to know each other, members shared the exciting news that the Mayo Clinic in Jacksonville is now a CMTA Center of Excellence led by Dr. Christopher Lamb (see related story p. 13). Members also discussed the results of their branch survey and planned their 2022 meeting dates.

CHICAGO, IL

The Chicago branch held a Zoom meeting on Dec. 7, 2021, using the opportunity to reconnect and catch up. They also talked about the resources available on the CMTA website, STAR research, the CMTA's Youth Program and Camp Footprint.

LAS VEGAS, NV

Ten people turned out for the Nevada branch's first in-person meeting in two years on Nov. 20, 2021. They were excited to be together as a group. Douglas Eck, a board-certified neurological clinical specialist joined the group and will be working with them to find qualified physicians and specialists to treat the CMT community in Vegas.


Las Vegas


Raleigh NC

RALEIGH, NC (RTP)

The RTP Branch met in-person on Nov. 13, 2021, for the first time since the pandemic began. Eight members enjoyed socializing over lunch, then put their masks back on to continue the meeting. The topic was "Managing Stress When Living with CMT." Stressors discussed included difficulty asking for help, being a caregiver for a family member and the loss of previously enjoyed activities.

BUCKS COUNTY, PA

The Bucks County branch welcomed 15 members to its Dec. 4, 2021, meeting, both in person and virtually. Members swapped stories, encouragement and resources with each other while decorating holiday sugar cookies. They also shared highlights from their branch Walk 4 CMT, updates from the CMTA and CMT-friendly holiday gift ideas.

SAVE THE DATE

COLDIRON DERBY

FUNDRAISER

100% of Proceeds Donated to Help Fund Research to Cure Charcot-Marie-Tooth Disease

CMTA
Charcot-Marie-Tooth Association

05/07/22

from 3pm to 8pm

SAGE CREEK FARM, TENNESSEE

ColdironDerbyCMTA.com

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

COLUMBIA (ADULT & PEDIATRIC)
University of Missouri*
Clinical Director:
Raghav Govindarajan, MD, FAAN
Appts: 573-882-1515

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
Appts: 585-275-2559

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

OREGON

PORTLAND (ADULT & PEDIATRIC)
Oregon Health & Science University
Clinical Director: Nizar Chahin, MD
Appts: 503-494-0744

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
Appts: (02) 98451325
daralyn.hodgson@health.nsw.gov.au

BELGIUM

B-2650 EDEGEM (ADULT & PEDIATRIC)
Antwerp University Hospital
Clinical Director:
Prof. Dr. Peter De Jonghe
Appts: +32 3 821 34 23
Neuromusculaire@uza.be

ENGLAND

LONDON (ADULT)
University College London Hospitals*
Clinical Director: Mary M. Reilly, MD
Appts: Mariola Skorupinska, (0044)2034488019
mariola.skorupinska@uclh.nhs.uk

ITALY

MILAN (ADULT & PEDIATRIC)
C. Besta Neurological Institute*
Clinical Director: Davide Pareyson, MD
Appts: +39-02-70631911
sara.nuzzo@istituto-besta.it

*** 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

MOST 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
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

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
Linda Davis
Mitch Davis
863-875-4239
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
Jay Pate
630-888-4673
Doreen Pomykala
815-351-1328

INDIANA
Fort Wayne Area
Aimee Trammell
574-304-0968
Indianapolis Area
Leader Needed—Call:
800-606-2682, Ext. 112

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

LOUISIANA
Baton Rouge Area
Leader Needed—Call:
800-606-2682, Ext. 112

MAINE
Portland Area
Mary Louie
207-450-5679

MARYLAND
Baltimore
Leader Needed
800-606-2682 Ext. 112

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
Leader Needed—Call:
800-606-2682, Ext. 112

NORTH CAROLINA
Charlotte Area
Mona Doshi
980-339-8560
Scott Roehrig
980-297-1449
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 DaCosta
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
Maryann Ciskal
716-435-3899
Kristen Braun
716-270-3095
Syracuse Area
Michael Casey
315-439-9325
Albany Region
Leader Needed—Call:
800-606-2682, Ext. 112
Westchester Area
Beverly Wurzel
Frank Wurzel
201-224-5795

OHIO
Cincinnati Area
Jill Stuhlmueller
513-254-4065
Jo Koenig
513-607-2822
Cleveland Area
Heather Hawk Frank
440-479-5094
Columbus Area
Jessica Diamond
216-570-6432

OKLAHOMA
Tulsa Area
Lonna Henry
918-961-1418
Natasha Karki
405-413-1831

PENNSYLVANIA
Bucks County Area
Julie FitzGerald Schell
315-573-3919
Chester County
Carol Aruffo
610-405-9291
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
Teresa Shoaf
615-772-8810

TEXAS
Austin Area
Nate Halk
512-415-6097
Dallas/Fort Worth
Keaton Butowsky
214-215-2081
El Paso Area
Jo Ann Longoria
915-526-0676
Olivia Longoria
915-491-0786
Houston Area
Miranda Burge
512-784-3086

UTAH
Orem Area
Melissa Arakaki
801-494-3658

VIRGINIA
Blacksburg/ Roanoke Area
Karen Brown
540-252-9453
Suffolk Area
Holly Hall
757-477-6122

WASHINGTON
Seattle Area
Denise Snow
206-321-1261
Emily Osborne
425-220-4225

WISCONSIN
Madison Area
Debi Weber
608-712-8709
Manitowoc Area
Barry Hett
920-388-9992

CANADA
Southern Ontario
Kelly Hall
519-843-6119
Toronto Area
Michael Driedger
647-680-7601

MEXICO
Guadalajara, Jalisco Area
Tomas Luis Lopez
Valenzuela
+52 1 33-18-28-17-07
Guadalupe Valenzuela
Cazares
+52 1 33-17-94-53-21

INTERESTED IN STARTING A BRANCH IN YOUR AREA?

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

CMTA STAR ALLIANCE PARTNERS

Acceleron Pharma
ARQ Genetics
Charles River
Genzyme, A Sanofi Company
HumanFirst Therapeutics LLC
Horizon Discovery
Ionis Pharmaceuticals
InFlectis BioScience
The Jackson Laboratory
NCATS (NIH)
New York Stem Cell Foundation
Passage Bio
PsychoGenics
Regency Pharmaceuticals
Renovo Neural, Inc.

CMTA CORPORATE PARTNERS

Aetrex Worldwide, Inc.
Allard, USA
Applied Therapeutics
Balance Walking
Foot Solutions
GeneDx
Hanger Clinic
Kinetic Research
Turbomed Orthotics

CMTA STAFF

Jonah Berger
National Youth Programs Manager
jonah@cmtausa.org

Sarah Gentry
Technology Manager
Sarah@cmtausa.org

Sarah Kaider
Digital Marketing Manager
sarahk@cmtausa.org

Kim Magee
Director of Finance and Administration
kim@cmtausa.org

Laurel Richardson
Director of Community Outreach
laurel@cmtausa.org

Jeana Sweeney
Director of Development
jeana@cmtausa.org

CMTA LEADERSHIP

Amy J. Gray, *CEO*

BOARD OF DIRECTORS

Gilles Bouchard, *Chairman*
Gary Gasper, *Treasurer*
Herb Beron, *Secretary*
Dan Chamby
David Coldiron
Thomas W. Dubensky, Jr., PhD
Laura Fava
Pete Foley
Alan Korowitz
David Norcom
Steve O'Donnell
Chris Ouellette
Elizabeth Ouellette
Kevin Sami
Phyllis Sanders, Esq.
Steven Scherer, MD, PhD
Michael Shy, MD
John Svaren, PhD
Special Advisor to the Board
Bruce Chizen

ADVISORY BOARD

Teresa Carroll, MS, PhD
Gregory Carter, MD, MS
Ken Cornell, CO
Bob DeRosa
Katy Eichinger, PT, DPT, NCS
Ashraf Elsayegh, MD, FCCP
Tim Estilow, OTR/L
Shawna Feely, MS, CGC
Valery Hanks, OTR/L, C/NDT
Sarah Kesty
Kate Lair
Bethany Noelle Meloche
Tom Meloche
David Misener, BSc (HK), CPO, MBA
Elizabeth Misener, PhD, LMSW
Christine Murray, MD
James Nussbaum, PT, PhD, SCS, EMT
Sabrina Paganoni, MD, PhD
Glenn Pfeffer, MD
Kenneth Raymond
Clark Semmes
Carly Siskind, MS, CGC
Greg Stilwell, DPM
David Tannenbaum, LCSW
Amy Warfield, PT, DPT
Evan Zeltsar

STAR ADVISORY BOARD

John Svaren, PhD, SAB Chair
University of Wisconsin

Frank Baas, MD, PhD
University of Amsterdam,
The Netherlands

Robert Burgess, PhD
The Jackson Laboratory

Maurizio D'Antonio, PhD
San Raffaele Scientific Institute,
DIBIT, Milan, Italy

M. Laura Feltri, MD
University at Buffalo

Steven Gray, PhD
University of Texas
Southwestern Medical Center

Scott Harper, PhD
The Ohio State University
School of Medicine

Kleopas Kleopa, MD
Cyprus Institute of Neurology &
Genetics

Jun Li, MD, PhD
Wayne State University

Rudolph Martini, PhD
University of Würzburg, Germany

Klaus-Armin Nave, PhD
Max Planck Institute for
Experimental Medicine
University of Göttingen, Germany

Brian Popko, MD
University of Chicago

Mario Saporta, MD, PhD
University of Miami

Steven S. Scherer, MD, PhD
University of Pennsylvania

Lawrence Wrabetz, MD
University at Buffalo

Stephan Züchner, MD, PhD
University of Miami

Mark Scheideler, PhD, TEB Chair
HumanFirst Therapeutics LLC

David Herrmann, MD
University of Rochester

Tage Honore, PhD
Aestus Therapeutics Inc.

Christopher Klein, MD
Mayo Clinic, Rochester, Minnesota

Lars J. Knutsen, PhD
Discovery Pharma Consulting LL

Claes Wahlestedt, MD, PhD
University of Miami

Michael E. Shy, MD, CEB Chair
University of Iowa

Mary Reilly, MD, CEB Co-Chair
National Hospital, London, England

Richard Finkel, MD
Nemour's Children's Hospital,
Orlando, Florida

Davide Pareyson, MD
Besta Institute, Milan, Italy

Joshua Burns, PhD
University of Sydney, Australia

Michael McDermott, PhD,
Consultant, University of
Rochester Medical Center

Email the CMTA at info@cmtausa.org

CMTA
Charcot-Marie-Tooth Association

P.O. Box 105
Glenolden, PA 19036
1-800-606-CMTA (2682)
FAX (610) 499-9267

cmtausa.org

Non-Profit Org.
U.S. Postage Paid
Tiffin, OH
Permit #52

CMT PATIENT MEDICATION ALERT:

Definite high risk (including asymptomatic CMT):

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

Moderate to significant risk:

Amiodarone (Cordarone)
Arsenic Trioxide (Trisenox)
Bortezomib (Velcade)
Brentuximab Vedotin (Adcetris)
Cetuximab (Erbitux)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddl, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Eribulin (Halaven)
Fluoroquinolones
Gold salts
Ipilimumab (Yervoy)
Ixabepilone (Ixempra)
Lefluonamide (Arava)
Lenalidomide (Revlimid)
Metronidazole/Misonidazole (extended use)
Nitrofurantoin (Macrodantin, Furadantin, Macrobid)
Nitrous oxide (inhalation abuse)
Nivolumab (Opdivo)
Pembrolizumab (Keytruda)
Perhexiline (not used in US)
Pomalidomide (Pomalyst)
Pyridoxine (mega dose of Vitamin B6)
Stavudine (d4T, Zerit)
Suramin
Thalidomide
Zalcitabine (ddC, Hivid)

Uncertain or minor risk:

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

Negligible or doubtful risk:

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

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

WHAT IS **CMT?**

More than 3 million people worldwide have CMT. It is one of the most commonly inherited nerve disorders and affects the motor and sensory nerves.

CMT is slowly progressive, causing the loss of muscle function and/or sensation in the lower legs and feet, as well as hands and arms.

People in **all ethnic groups may be affected by CMT.**

CMT is genetic, but it can also develop as a new, spontaneous mutation.

CMT can vary greatly in severity, even within the same family.

CMT causes structural deformities such as high-arched or very flat feet, hammertoes, hand contractures, scoliosis (spinal curvature) and kyphosis (rounded back).

CMT can also cause foot drop, poor balance, cold extremities, cramps, nerve, muscle and joint pain, altered reflexes, fatigue, tremor, sleep apnea, hearing loss and breathing difficulties.

CMT rarely affects life expectancy.

Some medications are neurotoxic and pose a high risk to people with CMT, notably Vincristine and Taxols. See full list (at left) of medications that may pose a risk.

More than 100 different genetic causes of CMT have been identified.

Many types of CMT can be determined by genetic testing. Please consult with a genetic counselor (www.nsgc.org) or your physician for more information.

Although there are no drug treatments for CMT, a healthy diet, moderate exercise, physical and/or occupational therapy, leg braces or orthopedic surgery may help maintain mobility and function.

The CMTA|STAR research program and extensive partnerships with pharmaceutical companies are driving remarkable progress toward delivering treatments for CMT, bringing us closer to a world without CMT.