

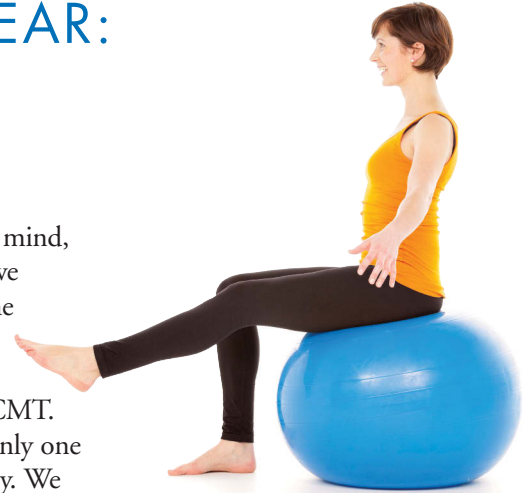
THE CMTA Report

JANUARY/FEBRUARY
2016

Information on Charcot-Marie-Tooth Disorders for Patients, Families, and the Scientific Community ★ www.cmtausa.org

ADVICE FOR THE NEW YEAR: LET'S MOVE!

BY SABRINA PAGANONI, MD, PHD



OUR MISSION:

To support the development of new drugs to treat CMT, to improve the quality of life for people with CMT and, ultimately, to find a cure.

OUR VISION:

A world without CMT.

CMTA LEADERSHIP

Patrick A. Livney
Chief Executive Officer

BOARD OF DIRECTORS

Herb Beron

Chairman and President

Elizabeth Ouellette

Vice Chair

Gary Gasper

Treasurer

Stephen Blevit

Laura Fava

Alan Korowitz

Steve O'Donnell

Chris Ouellette

Phyllis Sanders

Steven Scherer, MD, PhD

Michael Shy, MD

John Svaren, PhD

Vasi Vangelos

Peter I. Warfield, MD, MPH

Steve Weiss

Lawrence Wrabetz, MD

ADVISORY BOARD

Jonah Berger

Gregory Carter, MD

Bob DeRosa

Katy Eichinger

Tim Estilow

Shawna Feely

Sarah Kesty

Sean McKale

Bethany Noelle Meloche

David Misener

Elizabeth Misener, PhD

James Nussbaum

Sabrina Paganoni, MD, PhD

Glenn Pfeffer, MD

Susan Salzberg

Carly Siskind

Greg Stilwell, DPM

David Tannenbaum

Amy Warfield

STAR ADVISORY BOARD

John Svaren, PhD

Chair, Scientific Expert Board

Mark Scheideler, PhD

Chair, Therapy Expert Board

EMAIL CMTA AT:

info@cmtausa.org

To exercise or NOT to exercise?

If you have received conflicting advice about the role of exercise in CMT, you are not alone! Not a CMT clinic day goes by without my being asked:

"Will exercise help me get stronger?"

"Can exercise hurt people with CMT?"

"What types of exercise are best for me?"

"How much and how often should I exercise?"

The reason you may have received confusing and often contradictory answers to your questions stems from the relative lack of rigorous research on the topic. A recent systematic review of exercise and CMT identified just nine studies on the topic.* Most of the existing studies are small and use varied exercise protocols and design, preventing the possibility of generalizing results to all people with CMT. But as a whole, the available studies suggest that certain forms of exercise may be an effective tool for improving elements of health and fitness.

With this premise in mind, and drawing from what we know about exercise in the general population, let's sketch a framework for exercise for people with CMT. We are not focusing on only one muscle or area of the body. We are looking at the person as a whole with the goal of making gains in mobility, independence and overall well-being.

Several medical societies have declared that "Exercise is medicine." But if exercise is a medication, one needs to get the right type and dose. One would not use the same dose of a medication in an adult and a child, or for people who have different symptoms. Likewise, the exercise program and its methods and objectives must be tailored to each individual based on the type of CMT and several other clinical factors.

Exercise for CMT revolves around four modalities: balance, stretching, endurance and strength. Each modality can be considered a piece of a larger puzzle and needs to be combined with the other modalities for optimal results. Expert guidance from a physical therapist who has experience with CMT is invaluable and should be considered before engaging in any exercise program.

1. BALANCE

Balance problems are nearly universal with CMT. While this problem cannot be easily solved and assistive devices may still be needed, several simple balance exercises exist and can be performed at home. A chair may be used for support. Tai-chi and modified chair yoga have been shown to help with balance in other diseases.

2. STRETCHING

Stretching exercises help prevent pain and loss of range of motion at joints affected by CMT, such as the ankles. Stretching the heel cords should be a daily routine for people with CMT.

(continued on page 3)

INSIDE:

Message from Our CEO.....2

STAR Progress.....5

Rebecca Sand Award.....6

New Book for CMTers.....18

*Sman, A. D., Hackett, D., Fiatarone Singh, M., Fornusek, C., Menezes, M. P. and Burns, J. (2015), Systematic review of exercise for Charcot-Marie-Tooth disease. Journal of the Peripheral Nervous System, 20: 347-362.

THANK YOU
TO OUR
CORPORATE
SPONSORS:

allard

PHARMACEUTICAL
PARTNERS:

SANOFI/GENZYME
IONIS
PHARMACEUTICALS
ADDEX
PFIZER
NCATS (NIH)
PSYCHOGENICS
NEW YORK STEM
CELL FOUNDATION
AFFECTIS

CHEERS TO A ROBUST 2015:
MAKE YOUR 2016 RESOLUTION COUNT!

DEAR FELLOW CMTers:

As I sit and pen this letter to you, I'd like to share with you what we have accomplished together this year and what I will commit to you for 2016.

For the last three years, the CMTA has seen improvement in every meaningful metric in our march toward realizing success in our mission for a first treatment and providing help to increase the quality of life for each person with CMT!

On STAR: The CMTA is funding translational projects focused on CMT 1A, 2A, 1B, 2E, 1X, and 4 that cover more than 90 percent of CMT cases. We spent approximately \$2 million on this research, another record year. The CMTA enjoys collaborations with several pharmaceutical companies that have bought into the CMTA's STAR consortium model in our search for a first drug to treat patients. We anticipate milestones and decisions toward drug development to be a reality in the coming year.

On CMT Community: The CMTA added resources this year to continue the exploding numbers of CMTA members. All of our STAR efforts go for naught if we are not successful in identifying as many CMT patients as possible, as well as the type of CMT that affects them. The CMTA branch system has grown to more than 80 branches in 40 states, plus Canada. Together, we raised more than \$4 million in 2015, another record for the third year straight!

CMT Centers of Excellence, subsidized by the CMTA, NIH, and MDA, now number 22 globally, providing multidisciplinary touch points for CMT patients and their families under one roof.

My gratitude goes to all donors, the seven-person staff of the CMTA, all branch leaders, the invested and voluntary Board of Directors, active Advisory Board members, and dedicated STAR Scientific Board and Therapy Board members for your valuable and treasured participation and contributions for a remarkable and record 2015! I am humbled to be part of this team and community.

For 2016: I'd like to announce my renewed commitment to our mission and challenge all of you to make your New Year's Resolution count! My 2016 resolution is to challenge myself further by running a 3.5 mile Spartan race in April 2016. This will be the first time I've "run" since the year I was diagnosed at age 17, scary to say the least. My second fundraiser of the year will be my annual CMT Golf Challenge in June 2016. So you can see, I am all-in for raising as much as possible for what is shaping up to be a critical and exciting year!

I hope each and every one of you takes my challenge. Don't make some trite resolution: Make one that can make a difference.

My best to you and your family for a healthy and prosperous 2016!

Patrick A. Livney
CEO – CMTA

EXERCISE

(continued from page 1)

3. ENDURANCE
(AEROBIC) EXERCISE

Endurance (aerobic) exercise refers to activities that utilize large muscle groups such as walking, cycling and swimming. This type of exercise helps maintain cardiovascular fitness and has been shown to have several positive health effects in the general population, including improved mood and sleep. Swimming, or aqua therapy, is a great way to get some aerobic exercise as it does not subject the joints to undue stress. Nordic walking poles are



a good way to enhance the exercise that you get while walking and they help improve posture at the same time. A stationary recumbent bike is also a great way to get aerobic exercise, especially when the weather prevents outdoor activities. Portable mini-cycles can be used by people in wheelchairs to exercise the arms and/or the legs. You should aim to practice aerobic exercise two to five times per week.

4. STRENGTH TRAINING

You cannot build up muscles that are heavily affected by CMT because the nerve-muscle connection is not working well there. Still, you can strengthen those muscles that are not

affected by CMT. Some muscles may become weak simply because they are not being used enough and become "deconditioned." It is those muscles that we want to strengthen by doing gentle toning exercises two to three times a week. The goal is to use those muscles to help compensate for CMT-related weakness. The core and hip girdle muscles are areas that can be targeted for gentle toning in most people with CMT. Heavyweight, high-intensity training should be avoided and there should be no pain during or after performing these exercises. ★



Sabrina Paganoni is a member of the CMTA Advisory Board and is an Assistant Professor at Harvard Medical School.

A PLAN TO BECOME MORE ACTIVE

Start low, go slow (especially if you have not exercised recently). But aim high! The aim is to make exercise a fun component of your daily routine. If exercise is something you look forward to, you will keep doing it. Keep it fun!

One suggested sequence for getting started is:

1 Pick one balance exercise and a couple of stretching exercises for the lower body. All you need is five minutes a day, maybe while listening to your favorite song or to the news. This will soon become your favorite part of your daily routine.

2 Add moderate-intensity aerobic training two to five times a week (swimming, walking with Nordic poles, stationary bicycle) and a few core and hip girdle gentle toning exercise two to three times a week. The latter can be done standing, in the chair or in bed.



The exact length and type of exercise may vary. (Some people may be able to exercise for five to 10 minutes at a time, while others can do an entire 30 to 45 minute session.) But there should be no muscle pain and no excessive fatigue after the exercises. The idea is to gain without the pain. Muscle pain and excessive post-exercise fatigue (meaning that you need to rest for 30 minutes or more after exercise before resuming your normal activities) are clinical indicators of over-exertion, something to avoid in neuromuscular disease.

If you are looking for more ideas, you are only a click away! The CMTA is committed to empowering people with CMT by providing up-to-date educational material. Please refer to the CMTA website (www.cmtausa.org) for additional articles, tips, and videos on exercise prepared by other members of the Advisory Board. The CMTA exercise videos are a great starting point and allow for modifications depending on the type of CMT. They provide a practical guide on how to perform balance, gentle strengthening and stretching exercises.

A plan to become more active begins now!

Music City Patient/Family Conference Draws Crowds from Across South

BY BETHANY TONGATE

More than 100 people turned out to hear doctors, researchers and therapists from the Vanderbilt University Medical Center at the CMTA's Patient/Family Conference on November 14, 2015. The event was the first held in Nashville, home of the Grand Ole Opry and the other CMT—Country Music Television.

Three people from the Vanderbilt CMT Clinic spoke. Noting that more than 80 gene mutations cause CMT, Clinic Director Dr. Jun Li told the audience that, "Saying you have CMT is like saying you have a car. You could have a Toyota or Mercedes." Dr. Li informed participants that they need genetic testing in order to register for his clinical trials.

Robin Yawn, RN, the case manager for the clinic, outlined the steps necessary to get an appointment at the clinic: Have a neurologist fax a referral to the clinic at (615)-936-1263 and indicate "Dr. Li and CMT" on

the fax. She also suggested that patients bring pictures of other family members who have CMT to help Dr. Li determine how the patient's CMT will progress and to aid him with his research.

Penny Powers, RN, BSN, lead physical therapist at the clinic, exhorted the audience that "Movement is life!" This does not necessarily mean going to the gym to exercise, she said. Exercise can be done while seated on the couch or in the course of everyday activities. "The point is to move," Powers said.

CMTA CEO Patrick Livney updated attendees on the CMTA's Strategy to Accelerate Research (STAR). Perhaps most excitingly, the CMTA's partnership with Genzyme, a Sanofi company, has identified a handful of compound series for treating

CMT1A, and five other pharmaceutical companies are in early testing of compounds believed to have positive effects on CMT1A. Livney also stressed the importance of signing up with the Rare Disease Registry (www.rarediseasesnetwork.org/INC/register) to receive notification once a potential treatment is identified and studies are active.

The day also included lots of practical advice. Dr. Li advised audience members to let doctors know they have CMT, especially in emergency rooms, because CMT

can often mimic stroke-like symptoms.

A panel on assistive devices covered tools for helping those with CMT to function better. Kimberly Lilley from the Technology Access Center, and Valery Hanks from the Vander-

"Saying you have CMT is like saying you have a car. You could have a Toyota or Mercedes."

—Dr. Jun Li



Q&A in Nashville with CMTA Director of Development Susan Ruediger. Seated, left to right: Bruce Carter, PhD; Jun Li, MD, PhD; Penny Powers MS, PT, ATP; Robin Yawn, RN, BSN; and CMTA CEO Patrick Livney

bilt Pi Beta Phi Rehabilitation Institute, discussed one such device called the LiveScribe 3 Pen, which transfers written notes to a tablet or phone and plays audio back.

For hobbyists, the panelists suggested assistive devices like large-handled paintbrushes and electronic fishing rods. They also recommended that anyone with difficulty driving get a doctor's referral for a driving evaluation to assess the need for tools like hand controls, a left foot accelerator or a spinner knob.

Two members of the CMTA's Advisory Board spoke. Bethany Meloche, the CMTA's Director of Social Media, gave a moving presentation on how she has been affected by CMT. Stressing the importance of taking control of one's health, Bethany listed a number of action items that remain in the control of anyone with CMT, such as getting a nutritional panel to make sure CMT-like symptoms aren't being caused by vitamin or mineral deficiencies and visiting an orthotist to see if orthotics or braces are needed.

Advisory Board Member Jonah Berger instructed the audience on how to do a "mindset makeover" using the letters CMT. "C" stands for courage, he said, urging listeners to have the courage to share their stories with others and blow past self-imposed limits. "M" stands for mindset, he said, noting that it's not what happens to you in this life but how you deal with what happens to you that counts. Finally, Jonah said, "T" stands for treasure because there is always a gift. "You just have to dig for it" he concluded. ★

STAR IS WORKING!

Thanks to our generous members and donors, we were able to invest nearly \$2 million in STAR in 2015. That investment returned important research dividends on a number of different disease states, including:

CMT1A Our partnership with Genzyme, a Sanofi company, has identified a handful of compound series for treating CMT1A. Additionally, five other pharmaceutical companies are in early testing of compounds believed to have positive effects on CMT1A. We have improved the process for transforming adult human stem cells into Schwann cells (the cells that make myelin), preparing for a human cell assay. Laboratory models continue to reproduce robustly and the clinical data base is expanding. Together, the STAR team will forge ahead into preparing for clinical trials for CMT1A.

CMT1B Through the work of Dr. Brittany Wright at the NIH, three cellular assays and laboratory models are under development, and adult stem cell development is underway.

CMT2A The STAR team has developed two viable, robust laboratory models for two mutations. Characterization of the models is underway and when complete will be poised to test potential compounds. Cellular assays are underway in preparation for a high-throughput screen.

CMT2E A team of 2E experts has met to discuss the pathway toward drug delivery. Laboratory models are established and are under validation, preparing for drug candidate screening.

CMTX Early positive results from anti-inflammatory compounds have been demonstrated in laboratory models. Further investigation is underway.

CMT4 A team of type 4 experts is being assembled to develop a pathway toward drug discovery. STAR's first Type 4 project has been approved and is being documented for takeoff.

TYPE
1A

TYPE
1B

TYPE
2A

TYPE
2E

TYPE
X

TYPE
4

Beverly Wurzel Receives Rebecca Sand Volunteer of the Year Award

On November 7, 2015, CMTA Community Services Director Jeana Sweeney and Finance Director Kim Magee presented the Rebecca Sand Volunteer of the Year Award to a surprised Beverly Wurzel in honor of her 15 years as leader of the Westchester County CMTA Branch. The presentation took place at the Westchester County CMTA Holiday Luncheon for CMT. People who know Bev won't be surprised that Jeana hand-carried the award everywhere until the presentation, even the bathroom, knowing that Bev might just be tempted to take a peek into a box of unknown origin.

"Fiery" is a word people use to describe Beverly. Jeana calls Beverly a "firecracker," a one-of-a-kind individual whose faith and devotion to the CMTA are on display every day of the year. Elizabeth Ouellette, vice chair of the CMTA Board of Directors, calls Beverly "an unstoppable ball of fire, sparked into action by her sheer determination to leave this earth a better place, knowing that her hard work and dedication make a treatment for CMT not only a possibility, but a reality." According to Elizabeth, Beverly is "vivacious, optimistic, loving, unstoppable, nice, tenacious, energetic, exuberant, and resolute—in short an amazing volunteer."

Born to immigrants in Brooklyn, Beverly remembers being "dragged to every hospital in New York" when she was 10 so that doctors could examine

her gait. The doctors just shrugged. Beverly went on to attend Brooklyn College until she met and married her husband Frank when she was just 19. Together they bought their first house in Brooklyn for \$7,000. They later bought a \$24,000 house in Monroe, New York, where they raised their three children. Today,



*CMTA Director of Community Services
Jeana Sweeney and Volunteer of the Year
Beverly Wurzel*

Beverly has college-age and marriage-age grandchildren.

Before the CMTA existed, Beverly attended Muscular Dystrophy Association meetings. One day she was at an event at the Helen Hayes Hospital when a doctor told her she had Charcot-Marie-Tooth disease. Beverly had no idea what he was talking about but went in to get things checked out. On the day she was finally diagnosed and discharged, a nurse brought her a chair to wheel her out. When Bev asked why, the nurse said that her results dictated

that she leave the hospital in a wheelchair. Beverly's response was, "Maybe I'll wheel you out!"

When the CMTA came into existence, Beverly switched her allegiance. She began attending CMTA meetings and was eventually asked to lead the group. Amazingly, Beverly was initially reluctant to become a branch leader, feeling that she was too shy for the job.

In the 15 years since then, Beverly has become a role model and an inspiration for all CMTA branch leaders. She says that one of the secrets of becoming a great branch leader is getting to know all branch members on a personal level. Beverly is a fearless fundraiser. She believes in taking advantage of matching donations and when she hears about a match, she contacts all of her members and lets them know about the opportunity to double the impact of their donation. She also believes in doubling down on her asks.

In her own words: "When someone asks me if \$25 would be an okay donation, I tell them \$50 would be better."

Beverly attributes the tremendous growth in the CMTA to its leadership—people like CEO Pat Livney, Elizabeth and Jeana. The growth motivates her, she says, and the CMTA's work gives her group hope. Beverly firmly believes there will be a treatment, and ultimately a cure, in her lifetime. Until then, the CMTA is grateful to have leaders like Beverly raising funds and awareness for CMT! ★

NEW CENTER OF EXCELLENCE AT UNIVERSITY OF UTAH

The Charcot-Marie-Tooth Association has recognized a new Center of Excellence at the University of Utah. The new CMT clinic is a multidisciplinary clinic committed to improving the quality of life for children and adults with CMT. Initial visits typically take two or more hours to see all specialists, which include an RN clinical coordinator, a physical therapist, an orthotist, a genetic counselor, a dietician, a social worker, and orthopedic surgeons as needed. Helmed by Dr. Nicholas Johnson, the clinic is committed to research in CMT and can enroll patients in clinical studies. Its



University of Utah CMT Lab

research coordinator is also in clinic to address any questions about ongoing research at the University of Utah. Consultations can be coordinated by contacting Abby Smart, RN, Clinical Coordinator at 801-587-9824. The clinic will need a referral, demographic information and all pertinent medical records, including consultation notes, any genetic testing results and any EMG/NCV study results. Fax to 801-585-9179, Attention Angie O'Campo.



*University of Utah
CMT Clinic Director
Dr. Nicholas Johnson*

The CMTA Gratefully Acknowledges Gifts:

IN MEMORY OF:

ANNA MAE C. BERLIN
Mr. and Mrs. J. F. Becker, Jr
FREIDA BRIDGES
Mr. and Mrs. Frank Gunnison
BETTY CHOW
Mr. and Mrs. Eugene Lew
JAMES DAKIN
Ms. Judith Therrien
COREY ELLIOTT
Mr. and Mrs. Greg Barrow
Mr. Bill Shatto
SARA C. FUHRMAN
Mr. and Mrs. J. F. Becker, Jr
DOLORES GASPER
Mr. and Mrs. Keith Bockwoldt
Ms. Mary Marcowka
Mr. and Mrs. Patrick Rohan
ROSE GILDENHORN
Mr. and Mrs. Jeff Manchester
MARK E. HORAN
Mrs. Betty Horan
IRMA JAFFE
Mr. and Mrs. Samuel Jaffe
STEVE JUMPER
Mr. and Mrs. Gary Everhart
DICK KUTZ
Mr. and Mrs. Mike Westmoreland
BETH LABONTE
Ms. Chevelle E. Bazo
SHARON L. LaFONTAINE
Mr. Robert LaFontaine

THEODORE LOULOUDES
Ms. Joann Mahoney
AUDREY MacDONALD
Mr. Douglas MacDonald
REV. JIM McCracken
Mr. Steve Daniels
Mr. and Mrs. Terrence Tighe
CLEMENS PETSCHER
Mr. Collin McCartney
RON PLAGEMAN
Mr. and Mrs. Mike Westmoreland
JAMES POLITE
Mrs. Dolores Polite
SARAH SWAIN
Mr. Lawrence Senear
MIKE AND ELIZABETH
Mr. and Mrs. Norman Grant

IN HONOR OF:

TRENTON ANGELL
Mr. Jason Angell
HEATHER AND CORY BARKSDALE
Ms. Karen Ruberti
ROY BEHLKE
Mrs. Mary Kate Donais
LAUREN BLANCHETTE
Ms. MaryBeth DeVecchia
BARBARA CASTLE
Mr. and Mrs. Tom Bird
Mr. and Mrs. Jim Merrill

JACOB FERRIS
Mr. and Mrs. Stan Ferris
JAMES FREEMAN
Mr. Joseph H. Ficarrotta
STEPHEN GASPER
Dr. Deborah E. Jessiman
KYLE GILLIAM
Ms. Mary Sweitzer-Doyle
LORI GORDON
Ms. Merrie Hawley
ELLA HELFRICH
Ms. Christine Perrelli
BYARS HOLLAND
Ms. Teresa Hicks
HERBERT HOLLY
Ms. Betty Holly
PEGGIE JUMPER
Mr. and Mrs. Gary Everhart
ZACH KOROWITZ
Mr. Larry Shapiro
PETE MCHUGH – Happy 70th Birthday!
Ms. Vicki Brown
Ms. Ellen Chaimov
Mr. Joseph Curelo
Mr. Robert Cyders
Mr. James Davidson
Ms. Anne Duschka
Ms. Shirley Foey
Mrs. Brittany J. Gardner
Ms. Lynne Gary
Ms. Virginia Geehan

Mr. George Hafeman
Ms. Darlene Hess
Ms. Katherine Hinds
Mr. Mark Hoffmann
Mr. Calvin Katterman
Mr. Andrew Kercher
Mr. and Mrs. Bill Krohn and Family
Ms. Marion Langton
Mr. and Mrs. Dick Magnuson
Mr. and Mrs. Peter McHugh
Mr. Ted Moon
Ms. Enriqueta Ortiz
Mr. Dean Rinard
The Samuel S. Johnson Foundation
Mr. David Sorenson
Ms. MaryAnn Stanton
Ms. Reba Sundberg
Mr. Martin Voge
Mr. John Vogel
Mr. Steve Woods
THE MOLLNER FAMILY
Mr. and Mrs. Lowell Holsinger
DYLAN NEUFELD
Ms. Anna Stefos
CHRIS O'DONNELL
Mr. Kenneth E. Goodman
VINCENT PROVENZANO
Ms. Phyllis Nardella
GWEN REDICK
Mr. J. Michael Campbell
WILLIAM RING
Mr. Theodore Palmer

MR. AND MRS. JACK ROSE AND FAMILY
Ms. Nancy Cyanovich
PAYTON RULE – Happy Birthday!
Mr. Jason Rule
RACHEL RYAN
Mr. J. Michael Campbell
DON AND MARY ELLEN SHARP
Mrs. Emily Sharp
KAREN SMITH
Cedar Chapel United Methodist Women
DENISE SNOW
Ms. Judith Romeo
KATHY STEES
Mr. Richard Stees
VASI VANGELOS – Happy Birthday!
The Rome Family
L. RAY VEACH
Mrs. Sherry Veach
LOUISE VELLA
Mr. Simon Farrugia
MISSY WARFIELD
Mr. and Mrs. Burtis Dougherty
HARRIET GREENBERG WEISS AND FAMILY
Mr. David Horowitz
Mr. John McGrath

A NEW YEAR'S WISH FROM **HANGER CLINIC**, NEW CMTA PARTNER*Happy New Year, CMTA Members!*

ON BEHALF OF EVERYONE AT HANGER CLINIC, I am delighted to announce a new partnership with the CMTA. Both of our organizations work to improve the quality of life for those affected by CMT, while still clearly envisioning a world without it. Our combined efforts will allow greater collaboration, communication and success for those in need of our support and services.

Hanger Clinic is dedicated to fostering human potential and empowering everyone to live a healthier, happier and more active life. Your stories are incredibly inspiring, and Hanger Clinic is proud to partner with the CMTA's amazing, proactive leadership.

Everyone wants to live a life without limitations. We are here to help you do just that! Hanger Clinic is the largest provider of orthotic and prosthetic services in the country. We are committed to:

- Quality patient care programs to achieve the highest outcomes possible,
- Exceedingly skilled and educated clinicians (2016 will be busy with a variety of clinical and educational events),
- Convenient access to orthotic and prosthetic clinical care with over 750 locations across the U.S.
- The latest products and technologies, including the innovative low-profile Silicone AFO (SiAFO), available exclusively from Hanger, and,
- Recognizing that each individual's challenges are unique.

We are excited about the potential to help the CMTA make a difference, and we hope to hear from you about the obstacles you're facing and how Hanger Clinic can help you overcome them. Many of our clinicians have been involved in CMT clinics and with CMTA activities for years, and they will continue to share their experiences and expertise with all of our clinicians and other healthcare professionals.

Hanger Clinic believes in this partnership because we share the same optimism, passion for your independence and mobility, and belief in the power of your potential. Most importantly, we want to support your goals and add our voice to yours. To contact us or to learn more, visit www.HangerClinic.com or call 1-877-4Hanger.

Here's to an empowered 2016!

My very best regards,

Deanna Fish, MS, CPO, Hanger Clinic Vice President, Orthotics

HERE ARE SOME OF THE THINGS HANGER CLINIC CLINICIANS HAVE TO SAY ABOUT THE CMTA:

"This partnership offers so many exciting opportunities. CMT runs through my veins ... so I have a passion for finding the best intervention for myself, my family members and my other patients."

—Branch Hunsaker, CPO,
CMTA 2015 "I'm a Star" Winner

"I have been privileged to help many people with CMT over the past three years. I listen to each individual's goals and then create customized, flexible clinical solutions that meet their needs."

—Tim Leist, CO, who works
with Dr. Michael Shy at the
University of Iowa CMT
Research Clinic

Youth Will Out: LEAVING PATIENT/FAMILY CONFERENCES FOR NIGHTS ON THE TOWN

BY JONAH BERGER

A few years ago, the folks at the CMTA called me with an idea. They had noticed that many young people were attending the CMTA's Patient/Family Conferences. They had also noticed that the conferences didn't provide the right environment for those young people to connect or to share their experiences with CMT. Youth Outings were born, and Bethany Meloche, the CMTA's director of social media, and I were asked to lead them.

The concept was simple: After the science and speakers and table sitting of the conference were over, teens would gather, jump (or walk slowly) into a van and head out into the city for an evening of activity and fun. The concept was simple, but the results have been complex and wonderful.

In the last few years, we have taken Youth Outings in Chicago, Los Angeles, San Francisco, Orlando, Boston and Nashville! We have eaten together, bowled, gone to Disney World, quacked through a duck boat tour, escaped from "The Escape Game," and so much more. We're always aware of physicality when choosing the activities and we keep an eye on each other while participating in them. It is interesting to see a group of 15 people walking in the same

unusual way, with the same unusual but undeniably similar hands. On one trip, we took a group picture and instead of the traditional pose, we all put our hands in the middle and made a circle. Moments like these where we are all celebrating our strangeness and laughing at our struggle are precious beyond my ability to put into words.

Bethany and I soon realized that these young folks were really open to the others in the group.

There was a common tie among these strangers. They all knew what it was like to deal with differences, to face challenges every day and to manage the array of reactions they got from their peers. We began to use the dinner portion of the outings to facilitate a discussion about our experiences with CMT. Our staple starter questions are: What is the worst part of having CMT and what is the best part of having CMT? The answers we have received over the years are impressive and wonderful. Once the kids get talking, we usually sit back and watch the magic happen. We listen as they complain about

braces, tripping, being mocked, and just wanting to be normal. We listen as they discuss the wisdom they have acquired from

their path, the ability to see the struggles in others and the appreciation of being different in a world that so values conformity.

Among all the activities that I participate in, the Youth Out-

ings are among my favorites. There is something so special about being surrounded by people who understand some of your greatest challenges without any need of explanation. There is something magical about sharing the company and the laughter of such wise and special young people. While the outings were created to bring understanding and healing to the youth, I think that the leaders derive the very same things. ★



Youth Outing circle of hands

The concept was simple, but the results have been complex and wonderful.

Editor's Note: Beginning in 2016, the Youth Outings will fall under the new umbrella for all things having to do with CMters aged 13 to 19—CMTeen!



Disney Youth Outing

CMTA REMEMBRANCES

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

Honorary Gift:

In honor of (person you wish to honor)

Send acknowledgment to:

Name: _____

Address: _____

Occasion (if desired):

- ☐ Birthday ☐ Holiday ☐ Wedding
☐ Thank You ☐ Anniversary ☐ Other

Memorial Gift:

In memory of (name of deceased)

Send acknowledgment to:

Name: _____

Address: _____

Amount Enclosed: _____ ☐ Check Enclosed

☐ VISA ☐ MasterCard ☐ American Express

Card # _____

Exp. Date _____

Signature _____

Gift Given By:

Name: _____

Address: _____

CMT1A RESEARCH: A View from the Bench

BY JOHN SVAREN, PHD

Last year was a big one for the CMTA, and I want to give my perspective on how the Strategy to Accelerate Research (STAR) program is reaching the goals that we set for new therapy development for CMT.

As a faculty member at the University of Wisconsin-Madison, I was invited several years ago to participate in the STAR research program dedicated to CMT1A. At that time, my laboratory was interested in trying to do studies relevant to CMT1A but needed help to gather the necessary resources and to develop a plan of attack.

CMT1A was of great interest to me because it is caused by having an extra copy of the

PMP22 gene, and we thought that our investigation into genetic control of Schwann cells would give us the tools to identify ways to reduce PMP22 levels and thereby come up with an effective therapy for CMT1A.

Although relatively little was known about regulation of PMP22 at the time, resources provided by the

CMTA enabled us to identify regulatory pathways that can be used to downregulate PMP22. Along the way, our collaborations with the laboratory of Dr. James Inglese at the National Institutes of Health gave rise to a groundbreaking approach in which we developed and screened a new cell-based assay for CMT1A that utilized recent developments in genome editing.

Genome editing refers to newly developed technology that allows us to “re-write” a cell’s DNA sequence, much like editing a document with a word processor. In our case, we took Schwann cells, which make myelin (the coating around the nerves) in the peripheral nervous system, and inserted a new gene that would allow us to measure the output of the PMP22 gene. Like fiber optic cables that simultaneously transmit many types of data, our cells use light generated by a firefly gene, which emits light indicating the level of the PMP22 gene. Our goal was to use these genetically modified Schwann cells in order to simultaneously test large

numbers of compounds to find those that would reduce PMP22 levels and thereby identify candidate compounds for treatment of CMT1A.

In our own lab, we could test hundreds of compounds, but the Inglese lab at NIH had the capability to test multitudes more and we initially used these

“The ultimate success of these efforts begins and ends with the support and involvement of the CMTA community...”

cells to screen a collection of 3,000 compounds. The success of our approach led to using the same modified Schwann cells with partners at NIH and Genzyme, a Sanofi company, to test approximately 450,000 and almost 2 million compounds, respectively, for their ability to reduce high levels of the PMP22 gene.

Those tests were completed last year and we are now in the midst of testing hundreds of promising compounds using independent types of Schwann cells to try to identify those that can reduce PMP22 levels in a safe manner. These efforts have involved collaborations with scientists at other institutions, including Dr. Laura Feltri at the University at Buffalo and Dr. Gabsang Lee at Johns Hopkins University. In addition, our efforts involve many excellent clinician scientists like Dr. Michael Shy at the University of Iowa and Dr. Steven Scherer at the University of Pennsylvania, who were part of the early meetings that envisioned the goals of the STAR program.

Finally, these efforts have involved six people in my lab who have worked on various aspects of the project in a CMTA-sponsored collaborative network with other individuals at NIH and Genzyme-Sanofi who are skilled at drug screening and drug development. While we have come a long way, a lot of intense effort remains in order to refine and test our list candidate drugs using multiple testing platforms. Also, since we do not wish to put all of our eggs in one basket, we work on multiple parallel tracks to identify new ways to treat CMT1A.

This project has been immensely satisfying, not only because of the new scientific insights into the basis of CMT1A, but also because of the support and collaboration we have received from NIH and our pharmaceutical partners. The potential of this project to provide new treatments for CMT1A is a major motivator for everyone involved.

Along the way, the CMTA has fostered all of our initiatives and has supported similar approaches to develop therapy pathways for other major CMT subtypes like CMT2E, CMT1B, and CMT2A.

The ultimate success of these efforts begins and ends with the support and involvement of the CMTA community, which has been a major factor in attracting investment from NIH and pharmaceutical partners. Ultimately, we hope that our efforts will lead to clinical trials that can be used to increase the quality of life for individuals with CMT. ★



John Svaren is a member of the CMTA Board of Directors and the chair of the CMTA's Scientific Expert Board.

Sam Houston State Bearkats Raise \$21K for Research

Sam Houston State University’s women’s volleyball team hosted its first-ever CMT Awareness Night September 15 as the Bearkats took on Texas State. The Bearkats lost the game, but the CMTA won big: The event raised almost \$21,000 for CMT research.

The event was conceived in August 2015, just a few short weeks before its birth. Because there was no CMTA branch in Houston, Director of Community Services Jeana Sweeney and Midwest Regional Branch Manager Michelle Hayes organized what they called a “pop-up meeting” in an effort to kick-start interest in this major American city. They sent out e-mails, located a meeting room and waited to see who would show up. Happily for the CMTA, Devon Gray did. Her son Boston was recently diagnosed with CMT Type 2A, and her mother-in-law, Brenda Gray, is the Bearkats coach.

Working on a tight deadline, Devon and a host of volunteers created CMTA STARs—blue and orange cardboard cards with the CMTA Star graphic on a volleyball. The stars were sold for \$10 apiece (three for \$25, \$100 for a business). Donors were encouraged to write their names on the stars with encouraging words for Boston. Devon and her family began spreading the word, and the fundraiser took on a life of its own. The event was advertised on radio, Facebook and in the local newspaper. Volunteer Assistant Coach Tayler Gray sold tickets and stars all over town.



Boston Gray

The university graciously donated \$3 from every ticket sold to the CMTA.

On the day of the event, Michelle arrived at the Coliseum and broke into tears. More than 20 volunteers were hard at work filling helium balloons and decorating the Coliseum in CMTA blue and orange. All four walls around the court of the Coliseum were covered in signed CMTA STARs. The Sam Houston volleyball players warmed up in CMTA T-shirts. At halftime, the CMTA PSA was played on the Coliseum’s big screen, and Michelle spoke to the crowd. The response was tremendous and plans are in place to make it an annual event.

The CMTA gives a big Texas-sized thank you to Devon Gray, Brenda Gray, the entire Gray family, the Sam Houston State University, and the city of Huntsville, Texas! Go Bearkats! ★

Editor’s Note: We inadvertently left one very important story out of our Awareness Month coverage. It’s too good to be forgotten, so here it is, a little late, but still amazing!

CMTA RECEIVES CHARITY NAVIGATOR’S 4-STAR RATING FOR SECOND YEAR IN A ROW

The CMTA has earned a second consecutive 4-star rating from Charity Navigator. Only 21 percent of the nonprofits rated by Charity Navigator get two consecutive 4-star evaluations, indicating that the CMTA outperforms most other charities in America.

According to Charity Navigator President and CEO Michael Thatcher, receiving four out of four stars indicates that an organization adheres to good governance and other best practices that minimize the chance of unethical activities and consistently executes its mission in a fiscally responsible way.

“This ‘exceptional’ designation from Charity Navigator differentiates the Charcot-Marie-Tooth Association from its peers and demonstrates to the public it is worthy of their trust,” Thatcher said in announcing the award.

Charity Navigator evaluates 10 times more charities than its nearest competitor and currently attracts more visitors to its website than all other charity rating groups combined.





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

Dear David,

I was diagnosed about 10 years ago with CMT (while in my mid-thirties), and this last year it feels like it has gotten a little worse. As far as I know I am the only one in my family with CMT. I am married with three children in their late teens who show no symptoms. My question has to do with the daily struggle I have with living with all the uncertainty. My husband and I are ambivalent about getting the kids tested at this time. They are smart kids but show little interest in knowing. Do you have some tips for handling uncertainty in general? I am anxious and depressed much of the time even though there is much to be grateful for in my life.

David replies:

Although having CMT shoves uncertainty in our faces, uncertainty is a good challenge to master. Focusing on what-if-this-happens or what-if-that-happens can keep you in a state of anxiety that will paralyze you and lead to depression. I know because I did it myself for years. I used every little change in my body to justify all my worst fears. Ninety-nine percent of this worry was my imagination. I knew I had to find a way to manage my fears because of the very real connection between my anxiety and feeling physically weaker. I wish I had those years back because if I knew then what I know now, I would not have suffered so and maybe could have just enjoyed my young adulthood.

Allison Carmen wrote a great little book called "The Gift of Maybe." The concept of "maybe" helps you stand in the moment and also gives you an opening to recognize that things can always change. In your moment of feeling hopeless, negative or frightened, identify what you are feeling and say it out loud. Take a deep breath and wait a few minutes until you begin to notice a small shift. Allow these feelings to slowly pass away, notice what's left and then ask yourself: OK, what's next? Be mindful that life is filled with possibilities, even if you can't feel it in the moment. The idea of "maybe" allows you to have these feelings but will not let you get stuck in this place of negativity. "Maybe" offers you an opening of hope that you can move into naturally when you are ready. We get so stuck in the way we think our life should be that we can't see what is really in front of us. That is called linear thinking.



Write to David at info@cmtausa.org.

Linear thinking makes us believe that there is a right way and wrong way for our lives to unfold. Joseph Campbell said, "We must be willing to let go of the life we have planned, so to accept the life that is waiting for us." Arguing that things should be different from what they are is like banging your head against a closed door.

As we embrace the idea of "maybe," our thoughts dissipate and all the negative stories we tell ourselves about the future dissolve into the past. The goal is to try to sit comfortably in the present without our stories, knowing that the future can unfold in many ways and just maybe not turn out as badly as we have imagined. Be curious and open to all possibilities! ★

David Tannenbaum has an LCSW degree and has been a psychotherapist in New York City for the past 30 years. He has specialized 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.

NO, NO, THANK YOU!

Now it's our turn to be grateful! You responded to our Thanksgiving Match by raising \$100,000 in just 40 days. In total, the CMT community raised almost \$2 million for CMT research in 2015, more than \$1.2 million of it for CMT1A research. You're the reason why STAR is working! To read more about 1A research, see page 10. For a recap of progress on all disease states, see page 5.

MEMBER SPOTLIGHT: Deaf and Blind, Artist with CMT Creates, Inspires

Artist Christian Markovic named his company Fuzzy Wuzzy Design because that's how everything looked to him as he gradually lost his eyesight—fuzzy.

Deaf from the age of 2, Christian was diagnosed with Charcot-Marie-Tooth disease at the age of 13. At 20, he was declared legally blind. Despite his seemingly insurmountable health issues, Christian persevered and thrived. The first deaf graduate of Savannah College of Art and Design, Christian holds a BA in fine arts. Unable to find work after graduating from college due to his disabilities, Christian decided to start his own business, Fuzzy Wuzzy Design, a graphic design company that specializes in handcrafted greeting cards and clothes.

Christian inherited CMT from his mother. When his parents divorced, Christian was raised by his father, Bob Markovic. After Christian came down with the flu at the age of 13, Bob began noticing that he was experiencing walking issues, including occasional collapses. The deterioration of his eyesight soon followed.

Despite his health issues, Christian maintains a sunny disposition. According to his father he is always smiling and lives by the adage "Never Give Up!" Always artistically inclined, Christian received good grades at the Savannah College of Art and Design, and his creative abilities are on clear display in his artwork and the line of greeting cards and clothing incorporating it.



Artist Christian Markovic

To create his drawings, Christian works on a Macintosh computer using a zoom program that enlarges his work 10 times. His work has been shown at the Credit Suisse Gallery in New York City and the Pierro Gallery in South Orange, New Jersey. He is also one of the primary artists at Arts Unbound in Orange, New Jersey. Some of Christian's corporate customers are Harlem Day Charter School, Bright Horizons Day Care Centers and the United Way Foundation.

Christian points out that regardless of his diagnosis and loss of function, he is still a contributing member of society. He fights his CMT every day by staying active with physical therapy and going to the gym. He also continues to flex his creative muscles and recently took up sculpting.

"It's important for me to share my story, especially with others who have a similar diagnosis," Christian says, adding, "I want everyone to see my accomplishments in business and use that as inspiration." ★



© CHRISTIAN MARKOVIC

Christian sells his screened cards and other products from his website, www.fuzzywuzzydesign.com, as well as his ETSY store, which is located at www.etsy.com/shop/fuzzywuzzy4u.



CMTA Branches

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

ALABAMA

Birmingham Area

Bethany Tongate
David Tongate
205-533-8558

North Florence

Tina Smith
256-366-5273
Todd Oyen
256-810-6582

ARIZONA

Little Rock

Lisa Jones
501-776-5364

ARIZONA

Phoenix Area

Pamela Palmer
ppalmeraz@gmail.com
480-236-2445
Liz Ramadhan
240-731-6938

CALIFORNIA

Los Angeles Area

Alani Price
310-710-2376
Tara George
412-607-5193

CALIFORNIA

North Bay Area

Sharon Bello
650-465-3953

Sacramento

Holly Stevens
408-203-8804

San Diego Area

Jordan Thomas
619-549-0872

South Bay Area

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

COLORADO

Denver Area

Ron Plageman
303-929-9647

Dick Kutz

303-988-5581

CONNECTICUT

Hartford

Roy Behlke
239-682-6785

North Haven

Lynne Krupa
203-288-6673

DISTRICT OF COLUMBIA

Washington, DC

Steven Weiss
Kimberly Hughes
301-962-8885

FLORIDA

Naples

Roy Behlke
239-682-6785

Sarasota Area

Rachel Rivlin
941-870-3326

Tampa Bay Area

Vicki Polyea
813-251-5512

West Palm Beach

Phil Lewis
561-307-0100
Eileen Martinez
561-777-8471

GEORGIA

Atlanta Area

Susan Ruediger
678-595-2817

Katerina Marks

404-372-3158

IOWA

Iowa City Area

Jeffrey Megown
319-981-0171

North Iowa

Michael Groesbeck
641-512-1718

ILLINOIS

Chicago Area

Dale Lopez
708-499-6274

Norridge Area

Charles Barrett
224-628-5642

Sy Ahmed

847-912-1062

INDIANA

Fort Wayne Area

Aimee Trammell
574-304-0968

Priscilla Creaven

260-925-1488

Indianapolis Area

Nancy Allen
317-459-8773

Patricia Wood

317-345-2254

KANSAS

Wichita Area

Karen Smith
316-200-0453

MASSACHUSETTS

Boston

Mimi Works
617-913-4600

Jill Ricci

978-887-1014

MARYLAND

Baltimore

Clark Semmes
410-350-4812

Easton

Missy Warfield
410-820-0576

MAINE

Portland Area

Mary Louie
207-450-5679

MICHIGAN

Kalamazoo Area

Jori Reijonen
269-341-4415

MINNESOTA

Central Minnesota

Jo Smith
612-807-4729

Minneapolis Area

Marilyn Menser
320-522-0871

NORTH CAROLINA

Asheville Area

Ruth Ann Carroll
203-981-3738

Charlotte Area

Todd Long
704-902-4700

Trisha Hirsch

404-226-1389

Durham Area

Margaret Lee
919-656-2948

Wilmington Area

Laurel Richardson
814-404-8046

NEBRASKA

Hastings Area

Levi Stanger
402-705-4358

Lincoln Area

Karri Hood
402-641-0443

NEW JERSEY

Central New Jersey

Mark Willis
732-252-8299

Jacqueline Donahue

732-780-0857

Morris County

Alanna Huber
973-933-2635

NEW MEXICO

Albuquerque Area

Gary Shepherd
505-296-1238

NEVADA

Las Vegas Area

Virgina Mamone
702-343-3572

NEW YORK

Buffalo Area

Chris Warden
716-870-4425

Manhattan Area

Amanda Imbriano
516-680-2981

Upstate New York

Elizabeth Misener
David Misener

518-527-0895

Westchester Area

Beverly Wurzel
Frank Wurzel

201-224-5795

OHIO

Cleveland Area

Heather Hawk Frank
440-479-5094

Mandy Kimpton

440-724-1731

Columbus/

Newark Area

Rebecca Knapp
740-668-2103

OREGON

Grants Pass

Jessica Barton
541-218-5350 (cell)

541-846-8525

Portland Area

Debbie McHugh
520-296-1888

PENNSYLVANIA

Bucks County Area

Linda Davis
215-943-0760

Chester County

Ashley Caspersen
484-364-9334

Harrisburg

Erin Gaul
(717) 379-7504

Johnstown Area

J.D. Griffith
814-539-2341

Jeana Sweeney

814-269-1319

Northwestern Area

Joyce Steinkamp
814-833-8495

Pittsburgh

Carolyn Roberts
412-363-2811

SOUTH CAROLINA

Columbia Area

Elise Bidwell
803-462-1788

Greenville Area

Rebecca Lauriault
864-918-2437

Kitty Hodinka

610-761-6874

TENNESSEE

Hendersonville Area

Brittney Grabiel
423-213-2336

Nashville Area

Bridget Sarver
615-390-0699

Gwen Redick

256-655-0391

TEXAS

Dallas Area

Michelle Hayes
972-539-0905

El Paso Area

Veronica Gallegos
915-203-4391

Houston Area

Kristin Leard
713-516-8630

Benjy Hershorn

832-731-0121

Meredith Wells

832-264-7312

Vidor Area

Angie Beaumont
281-229-2099

UTAH

Orem Area

Melissa Arakaki
801-494-3658

Emily Hunsaker

801-356-1830

VIRGINIA

Chesapeake Area

Donna Koch
757-650-2417

Fredericksburg

Leigh Van Doren
540-370-1968

Harrisonburg Area

Jeanette Thompson
540-383-6195

Anne Long

540-568-8328

Richmond Area

David Collins
804-730-2918

Shenandoah Area

Teresa Frederick
540-336-4496

WASHINGTON

Seattle Area

Lynn Ronald
206-546-8923

WISCONSIN

Madison Area

Debi Weber
608-712-8709

Jessica Wells

714-333-8031

Milwaukee Area

Susan Moore
414-604-8736

CANADA

British Columbia

Melanie Bolster
250-888-7713

Neville Tate

250-656-2547

Northern Ontario

Brenda Spencer
705-788-0408

Southern Ontario

Kelly Hall
519-843-6119

BRANCH LEADER SPOTLIGHT: Rachel Rivlin, Sarasota, FL

Rachel Rivlin inherited her height and her love of sports from her father, who played professional basketball in Maine. From her mother, she inherited CMT. While her mother was never officially diagnosed, Rachel remembers that she had high arches, tripped a lot and staggered when she stood up from a chair. Doctors said her mother had arthritis and maybe Parkinson's disease.

Rachel was an all-around athlete in her youth. She was a swimmer and played basketball, softball and volleyball. Later, she was a professional belly dancer with paying gigs in restaurants and at private parties. Shifting from physical to mental contortions, Rachel became an attorney and ran the legal department at a large insurance company. She also served on a number of professional associations and chaired committees of the American and Boston Bar Associations.

In her late forties, Rachel began noticing numbness in the balls of her feet. She told her doctors that there was something wrong with her feet, but they mostly ignored her. One orthopedist told her she had tarsal tunnel syndrome and that he could perform surgery, but when she asked if it would help, he said probably not.

Frustrated with the lack of a diagnosis, Rachel finally went to one of the top neurologists in Boston. He had no more insight into the cause of her symptoms than anyone else, but eventually he did a nerve biopsy, taking a chunk of nerve out of her left leg. Weeks later he called with results. The good news was that Rachel did not have ALS or MS, as the neurologist had feared. The bad news was that she might have CMT. When she asked her neurologist for more information about Charcot-Marie-Tooth disease, he handed her a two-page pamphlet with little real information.

Curious about Rachel's diagnosis, her partner, Manuel Goldberg, went online and began researching CMT. He quickly found a website and began reading about the disease. It was soon obvious that Rachel's high arches, foot numbness, thin ankles and scoliosis represented a classic case of CMT. A neurologist later confirmed this conclusion. Rachel's immediate emotion was relief. At least she knew what it was, and that it was not life-threatening.

As Rachel's CMT progressed, she began to find it hard to walk on Boston's cobblestoned streets. Snow and ice were also a problem. She found that she was becoming very fatigued after a day of work and



an evening that might include a meeting. She often went directly to bed when she got home from work. Finally, during a Thursday appointment, her internist ordered her to stop working immediately. Rachel convinced him to allow her to finish the work week and by Monday she was on disability from her job.

The realization that her work life was over hit Rachel hard. She remembers becoming very emotional and crying at the doctor's office. While she had always tried to prepare for that day, she still found herself unready when it finally arrived. She was 59 years old when she went out on short-term disability. She later went on long-term disability thanks to insurance purchased years earlier through her work. She never thought she would have to use it, but it got her through.

Ori Bash Takes Reins of West Coast Branches

BY CLARK SEMMES

Ori Bash, the CMTA's new West Coast regional branch manager, brings a wealth of knowledge and the perfect skill set to his new position. If CMT stands for courage, motivation and tenacity, Ori embodies all three.

Ori, who will also work in fundraising, was born in Israel and raised in the San Francisco area, where his parents immigrated in their late 30s in pursuit of the American dream. Ori says his parents won the green card lottery thanks to the sponsorship of his uncle, who lives in Berkeley. The first few years were tough, but they persevered and built a great life in the United States. Ori attributes his strong work ethic and courage to the example his parents set in their times of adversity.

Ori attended business school in Chicago in pursuit of a finance career as an investment banker specializing in mergers and acquisitions. Disillusioned by the stress and values of life as an investment banker, Ori found a new job working for a valuation services firm servicing wealthy clients, family-owned businesses and private equity managers. The New York firm hired Ori as its first employee on the West Coast to build a presence in California.

At the age of 28, Ori began to notice that he was tripping frequently and having trouble lifting the front of his feet. A podiatrist

tested his reflexes and immediately sent Ori to a neurologist who confirmed that he had CMT. Ori's diagnosis led to the discovery of CMT in both his father and his brother. In 2007, genetic testing narrowed the diagnosis to CMT1A. Stunned



*West Coast Regional Branch Manager
Ori Bash and Abbey*

by the diagnosis, Ori initially hid his condition from all but a few friends and family members, but eventually decided that being open about it was the better path. In time, he joined the San Francisco branch of the CMTA and later took over branch leadership from CMTA Board Member Elizabeth Ouellette.

Ori got his first braces in 2007 and a second pair in 2010. By 2012, the second set wasn't working any more. He was falling more frequently, was

fatigued and began to feel hopeless about his declining state. In September 2014, Ori decided to take a gamble and ponied up \$13,500 for a pair of Helios braces. After a battle, his health insurance company reimbursed him for 70 percent of the cost.

Ori says the Helios braces have been a "game changer" for him in terms of increasing his quality of life and allowing him to be much more active with more confidence. "They have given me renewed hope," he says.

Another thing that gave Ori hope in his very darkest hours was his rescue dog, Abbey, a Formosan Mountain Dog he adopted in 2009. Abbey was rescued by an Asian Humane Society that brought her and her littermates from the Taiwanese countryside. Ori says she almost single-handedly lifted him from the depths of depression. Thanks to

those Helios braces, Abbey is now getting significantly longer walks.

In September 2015, Ori and his Co-Branch Leader Tau O'Sullivan organized the Palo Alto Walk for CMT, which raised approximately \$55,000 for the CMTA. Ori plans to contribute to the CMTA in raising both funds and awareness. In particular, Ori hopes to assist the CMTA in identifying large donors, corporate sponsors and strategic partners. ★

CMTA BRANCH NEWS

CENTRAL ALABAMA

Todd Clay from the Hanger Clinic brought a wide variety of AFOs to the branch's October 24, 2015, meeting. Members were particularly excited about trying the company's new Silicone AFO, a custom brace molded to the wearer's foot. According to Todd, Hanger offers free evaluations to assess the benefit of orthotics or AFOs for CMTers.

NORTH BAY, CA

Eight people attended the branch's December 13, 2015, meeting, sharing stories and tips on their orthotics and orthotists, genetic testing, and insurance. They all said that they see value in having a group north of the Golden Gate Bridge, even if it is a small one. Attendees were encouraged to donate in any amount to the CMTA so that they are in the database and can be contacted for clinical trials and take advantage of all the other help offered. The next meeting will be in March, with a yoga instructor who specializes in chair yoga and adapting yoga for different ages and physical abilities/limitations.

SOUTHERN CONNECTICUT

The branch welcomed a guest speaker from the state's Muscular Dystrophy Association at its last meeting of the year on December 7, 2015. Nicole Ginolfi, health care services coordinator, provided information and answered questions about services available for those with CMT. Those services include four Connecticut MDA clinics, a loan closet with durable medical equipment that can be delivered free of charge, an equipment repair fund, a summer camp for children ages 6 to 17 and payment for one therapy session not covered by insurance.

CHICAGO SOUTH

Stephanie Gandomi, from the genetic testing company Invitae, spoke at the branch's November 14, 2015, meeting. Stephanie is board-certified by the American Board of Genetic Counseling and has more than 10 years of experience in research and clinical practice. Stephanie did a wonderful job explaining the genetics of CMT. She also explained the process and cost of Invitae's genetic testing, noting that new technology has lowered the cost and decreased the waiting period for results.

NORTHERN IOWA

Members met on October 17, 2015, to distribute new materials and catch up on new doctors' visits. They plan on contacting the Northern Iowa Community College's medical assistance class to come in for a presentation about CMT. They also talked about ways to promote the CMTA in the community, to get pamphlets about CMT out to the school nurses and about finding new members with CMT to encourage them to come to meetings.

BALTIMORE, MD

The branch welcomed three speakers to its December 6, 2015, meeting at the Towson Public Library—CMTA Board Member Steve O'Donnell, Johns Hopkins Neurologist Dr. Thomas Lloyd and Allard USA District Manager Jayme Brendle. Steve O'Donnell emphasized the importance of CMTA membership. Anyone can become a member of the CMTA for \$30 a year, and benefits include a subscription to the CMTA newsletter. Dr. Lloyd, the head of the CMT Clinic at Hopkins, spent almost an hour answering questions from the audience. In the second half of the meeting, Jayme explained the role braces can play in keeping people with CMT on their feet. The meeting ended with members trying on and comparing various Allard braces.

SOUTHWEST MICHIGAN

Three members, including one new one, attended the branch's November 19, 2015, meeting. The group answered many of the new member's questions, discussing local physicians, diagnosis and genetics of CMT, current research and the services available through the CMT Clinics.

LINCOLN, NE

Six people came out for the branch's December 8, 2015, meeting. They discussed fundraising and several people said it would be difficult for them to participate in an event because of the distance they have to travel to get to Lincoln. The group decided to hold a fundraiser in early spring. Members also discussed having speakers at meetings and different types of AFOs. The discussions ended with one branch member sharing how important the group is to bring CMTers in the southeastern part of the state together to join forces for a cure. The branch decided to pass a cup around at each meeting to collect donations/dues. A donation box set up at the beginning of the meeting had \$37 in it by meeting's end. The group's next meeting will be held on March 8, 2016.

ALBUQUERQUE, NM

At the branch's November 7, 2015, meeting, Branch Leader Gary Shepherd updated the 10 participants (three of them new) on the exceptional breadth of CMT research currently going on. He noted that more has been accomplished in the last

few years than in the preceding 100. He also stressed the importance of keeping research money flowing into the CMTA and of joining the CMTA as a paid member since these dues are used to cover the organization's administrative expenses. Gary then welcomed Jonah Berger via Skype who gave a wonderful presentation on making over one's mindset. Jonah had a great deal to share, and his talk was very uplifting and much enjoyed by the attendees. The first meeting for 2016 will be on Saturday, January 30. The other quarterly meetings will be April 30, August 6, and November 5.

MORRIS COUNTY, NJ

The group held its last meeting of the year on December 6, 2015. They also had a great sale with Arbonne, Mary Kay, Fuzzy Wuzzy Designs (see related story p. 13), Angry Tea Room and Lori Mattheiss' gorgeous jewelry! Arbonne, Mary Kay and Fuzzy Wuzzy all donated part of their sales to the CMTA.

BUFFALO, NY

The branch spent most of its December 3, 2015, meeting brainstorming future directions for the group. They discussed becoming CMTA members for either \$30 or \$100 and also making donations in whatever amount possible, from \$1 on up. The group will be setting up a Facebook page for meeting announcements, CMT updates and news. The group also discussed raising awareness of CMT on a daily basis, whether through social media, email or a conversation standing in line.

(continued on page 18)

THANKSGIVING HOLIDAY HUDSON RIVER WALK FOR CMTA

Our neighbors and fellow members of the Fort Miller Reformed Church in Fort Miller, New York, meet annually on Thanksgiving morning for a brisk 3.5K walk, ride or run along the Hudson River. We do it to raise funds for a different cause each year, as well as some pre-Thanksgiving meal exercise. This year we walked in support of the CMTA. My son was diagnosed at 14 with CMT. He's now 30 and after numerous foot and ankle surgeries, continues to work as a cabinet maker, lighting technician and musician/songwriter for the group Eastbound Jesus. He faces daily challenges but is determined to do everything he is physically capable of and more. This year our small group raised not only heart rates, but \$200 for the CMTA.

—Debbie Anderson, music director
at Fort Miller Reformed Church

CMTA BRANCH NEWS
(continued from page 17)

WESTCHESTER, NY

The branch held its third annual luncheon and fundraiser to raise money for STAR on November 7, 2015. This year's event was the biggest ever with 87 people in attendance. The auction included more than 40 gift cards, gift baskets and other donated items and was presided over by a professional auctioneer, which made for a fun-filled afternoon. There was also a 50-50 raffle, jiggered to become a 40-20-20-20 raffle so that there would be more winners. Two of the three winners gave their winnings back to the CMTA. The event raised more than \$7,000 which was matched dollar-for-dollar, so that over \$14,000 was raised for research. Branch Leader Bev Wurzel says they have already started the process of looking for a bigger venue for next year's event.

★
PORTLAND, OR

Members discussed speakers, transportation and Awareness Month at the October 15, 2015, meeting. Everyone present expressed an interest in having speakers at meetings. Members also discussed how to get members from outlying areas to the meeting location, either through carpooling or providing rides to those who need them. Warren Beals presented a video of CMTA CEO Pat Livney speaking about the wonderful research that's going on, which affects types 1A, 2A, 1X, 1B, 2E and 4. Branch leader Debbie

McHugh shared a terrific fundraising idea with the group—a birthday party fundraiser for her husband Peter's 70th birthday that raised over \$4,000 (and counting) for the CMTA. The next meeting will be held on January 21, 2016.

★
NASHVILLE, TN

Guest speaker Teresa Blake from GeneDX gave a wonderful presentation on genetic testing. Penny Powers, a physical therapist from the Pi Beta Phi Rehabilitation Institute at Vanderbilt Medical Center, and Dr. Renee Brown, Chair of the School of Physical Therapy at Belmont University, gave an update on their research project involving seating and said that they will be able to see children from the age of 8 and up. The group agreed to have next year's Awareness Month Walk on September 23, 2016, at the same location—Long Hunter State Park.

★
CHESAPEAKE, VA

Dr. Erin Jerlin, DPM, FACFAS, a podiatrist from Atlantic Orthopaedic Specialists, was the guest speaker at the branch's November 8, 2015, meeting. She had a number of suggestions for members, including:

- Use light weights to strengthen your muscles. Repetition is important. Heavy-duty strength training is NOT recommended for CMTers.
- Stand at your kitchen sink/counter with your toes propped up on a mat or rolled towel. This stretches the muscles and relieves stress on the balls of the feet.
- Consider physical therapy every three years as your feet continue to change.

This will help you learn how to walk on your “new” feet.

- Wearing support hose/socks helps provide information to the brain, helping to stimulate the nerves. Shoe inserts that “bring the floor up” to your foot also provide the brain with additional information as do gloves.

★
RICHMOND, VA

This new branch met for the first time on November 17, 2015. Branch leader David Collins encouraged everyone to join the CMTA to gain the benefits of the newsletter and other resources. One of the branch's goals is create an active Resource Directory to share information about various providers with CMT expertise. David also reminded members that during the holiday season they can make “automatic” donations by designating the CMTA as the recipient when making purchases through smile.amazon.com, lgive.com and Goodsearch.com.

★
SOUTHEAST WISCONSIN

Ten members, including three new ones, gathered on December 3, 2015, to hear Stacey Hanke, health care services coordinator for the Southern Wisconsin Muscular Dystrophy Association. According to Stacey, the Wisconsin branch sponsors summer camps for kids, has a lending “library” of durable medical equipment and has MDA-sponsored clinics in Madison, Milwaukee, Green Bay and Marshfield. It can also help with flu shots and repairing medical equipment.

RACHEL RIVLIN
(continued from page 15)

in Florida. After buying a house in Sarasota County, they spent five months painting it, sold their house in Boston, and moved to the Sunshine State for good. Shortly after moving, Manuel found a CMTA support group in nearby Tampa Bay that they attended for four years. It was more than an hour away, but they both enjoyed attending the meetings led by branch leader Vicky Polleya.

After four years, Rachel began thinking it would be nice to have a support group closer to home and started a branch. She hosted the first meeting of the Sarasota, FL CMTA Branch on February 22, 2014. They now get 10 to 25 people at each meeting, depending on the season. On November 1, 2014, Rachel helped organize the First Annual Sarasota, FL CMT Walk and Roll. The event featured a walk across the beautiful Ringling Bridge. Unfortunately, there were 40 mph wind gusts on the day of the walk and while some were able to walk across, some of the folks on scooters could not. The 2015 walk was held on November 7 at the Sarasota Square Mall, where wind was not an issue. With the help of a branch matching challenge, the event raised almost \$12,000 for the CMTA.

Rachel has enjoyed being a branch leader. She has met many people and has learned a lot. On October 27, 2015, she was interviewed on the Sarasota ABC affiliate television station before the branch's Walk and Roll. Now she is a bit of a local celebrity with strangers stopping her and exclaiming that they saw her on TV.

Thank you Rachel for all that you do for the CMTA and CMT Awareness! ★

“THE TRIBE OF THE THIN ANKLES”

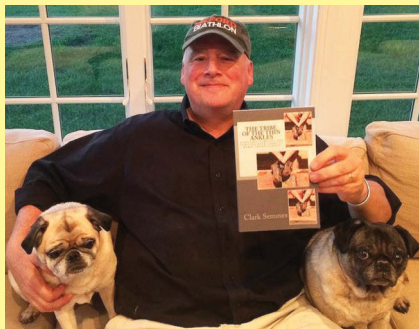
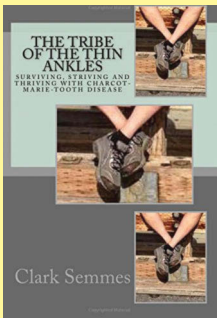
There's a new volume on the CMT bookshelf. Written by Baltimore Branch Leader Clark Semmes, “The Tribe of the Thin Ankles” is a compilation of profiles of individuals who are doing amazing things despite their CMT.

Available on Amazon.com, the book includes profiles of Pat Livney, Jeana Sweeney, Elizabeth Ouellette, Steve O'Donnell, James Cuizon, Anthony Zahn and lots of other CMT activists, athletes and scientists.

One customer reviewer on Amazon said, “If 1 in 2500 people have CMT, then 1 in 2500 people—plus their friends and families—would benefit

from this book. Semmes' collection of stories of people living with CMT is inspirational without being maudlin and conveys some complex information in a way that is easily understood. ‘The Tribe of the Thin Ankles’ is written with humanity and humor, and I highly recommend it for anyone touched by CMT.”

All profits from sales of “The Tribe of the Thin Ankles” will go to the CMTA. Those not wishing to make purchases on the internet can contact Clark directly at ClarkSemmes@Gmail.com.



The CMTA Report is published by the Charcot-Marie-Tooth Association, a registered non-profit 501(C)(3) health organization. © 2016, The CMTA. All rights reserved under International and Pan American Copyright conventions. No part of this newsletter may be reproduced in any form or by any electronic or mechanical means, including information storage and retrieval systems, without permission in writing from the publisher. The opinions expressed in the newsletter are not necessarily those of the Charcot-Marie-Tooth Association. The material is presented for educational purposes only and is not meant to diagnose or prescribe. While there is no substitute for professional medical care for CMT disorders, these briefs offer current medical opinion that the reader may use to aid and supplement a doctor's treatment.

CMTA MEMBERSHIP, PUBLICATIONS & ACCESSORIES ORDER FORM

NAME: _____ / _____ / _____
First MI Last

ADDRESS: _____

CITY: _____ STATE: _____ ZIP: _____

COUNTRY/POSTAL CODE (IF NOT U.S.): _____

DAYTIME PHONE: _____ EVENING PHONE: _____

EMAIL: _____

If you are a STAR member or are joining as a STAR member now, you may purchase publications and accessories at discounted prices. (Some exclusions may apply.) To check your membership status, please call 1-800-606-2682, ext.105.

Subscription Membership Benefits:

- Online access to valuable information about living with CMT
- Access to the CMTA's Online Community, including access to CMTA Branches and Discussion, Circle of Friends and CMTAthletes groups
- An information kit and a 10% discount at the CMTA store
- Bimonthly delivery of *The CMTA Report*, (electronic and/or hard copy)
- The *CMT Facts VI* series (electronic copy)

STAR Membership Benefits:

- All of the benefits of Subscription Membership
- PLUS**
- One free pair of Aetrex Shoes in the style of their choice (new STAR members only)
- 50% off all Aetrex shoes (new and renewing STAR members)
- An expanded and updated copy of the CMT Survivor's Guide
- A 20% discount at the CMTA Store
- One *You're a STAR* auto decal

JOIN THE CMTA:

| Subscription Membership | <input type="checkbox"/> \$30/year | <input type="checkbox"/> \$80/3 years | Choose your newsletter format (check one or both) | |
|---|-------------------------------------|--|--|--------------------------------|
| | | | | |
| STAR Membership | <input type="checkbox"/> \$100/year | <input type="checkbox"/> \$270/3 years | <input type="checkbox"/> PDF | <input type="checkbox"/> Print |
| | | QUANTITY | COST | TOTAL |
| | | Regular Price | STAR Member Price | |
| The Patients' Guide to Charcot-Marie-Tooth Disorders [CD Format] | | | \$10 | \$8 |
| My Child Has CMT | | | \$5 | \$5 |
| Teaching Kids about CMT...A Classroom Presentation (DVD/1 hour) | | | \$10 | \$8 |
| CMTA Titleist Hats Quantity and Color: Blue____ Lt. Blue____ Black____ White____ | | | \$25 | \$20 |
| CMTA T-Shirts <input type="checkbox"/> Navy Blue with white logo) <input type="checkbox"/> White with blue logo) Quantity and Size: S____ M____ L____ XL____ 2XL____ 3XL____ | | | \$15 | \$12 |
| CMTA Shark-0 T-Shirts Quantity and Size: S____ M____ L____ XL____ 2XL____ 3XL____ | | | \$20 | |
| NEW! CMTA Sweatpants Quantity and Size: Youth XL____ Adult S____ M____ L____ XL____ 2XL____ 3XL____ | | | \$20 | |
| Be a STAR Wristbands | | | \$1.50 each* | |
| Be a STAR Necklaces (Includes battery) | | | \$5 each* | |
| Washable CMTA Tattoo (Pack of 5) | | | \$1 per pack | |
| CMTA Pin | | | \$3 each | |
| CMTA Brochure & Neurotoxic Drug Card | | | FREE | |
| Donation to the CMTA (100% Tax-deductible) | | | | |
| Shipping & Handling (Orders under \$10, add \$3.50; orders \$10 and over, add \$7.50) | | | | |
| *Quantity discounts for these items available online | | | | |
| ORDER TOTAL | | | | |

☐ Check payable to the CMTA (US residents only; non-US residents, please use credit card or international money order.)

☐ Money Order ☐ American Express ☐ MasterCard ☐ VISA

Card Number: _____ Expiration Date: _____

Mail to: CMTA, P.O. Box 105, Glenolden, PA 19036; or fax to 610-499-9267

A copy of the official registration and financial information may be obtained from the Pennsylvania Department of State by calling, toll-free within Pennsylvania, 1-800-732-0999. Registration does not imply endorsement.



CMT PATIENT MEDICATION ALERT:

Definite high risk (including asymptomatic CMT):

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

Moderate to significant risk:

Amiodarone (Cordarone)
Bortezomib (Velcade)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddI, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Eribulin (Halaven)
Gold salts
Ixabepilone (Ixempra)
Leflunomide (Arava)
Metronidazole/Misonidazole (extended use)
Nitrofurantoin (Macrochantin, Furadantin, Macrobid)
Nitrous oxide (inhalation abuse)
Perhexiline (not used in US)
Pyridoxine (mega dose of Vitamin B6)
Stavudine (d4T, Zerit)
Suramin
Thalidomide
Zalcitabine (ddC, Hivid)

Uncertain or minor risk:

5-Fluorouracil
Adriamycin
Almitrine (not in US)
Chloroquine
Cytarabine (high dose)
Ethambutol
Etoposide (VP-16)
Fluoroquinolones (Cipro)
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 CMTA Report

The Charcot-Marie-Tooth Association

P.O. Box 105

Glenolden, PA 19036

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

www.cmtausa.org

NON PROFIT ORG

US POSTAGE PAID

TIFFIN, OH

PERMIT #52

WHAT IS CMT?

- ▶ CMT is the most commonly inherited peripheral neuropathy, affecting approximately 150,000 Americans.
- ▶ CMT may become worse if certain neurotoxic drugs are taken.
- ▶ CMT can vary greatly in severity, even within the same family.
- ▶ CMT can, in rare instances, cause severe disability.
- ▶ CMT is also known as peroneal muscular atrophy and hereditary motor sensory neuropathy.
- ▶ CMT is slowly progressive, causing deterioration of peripheral nerves that control sensory information and muscle function of the foot/lower leg and hand/forearm.
- ▶ CMT causes degeneration of peroneal muscles (located on the front of the leg below the knee).
- ▶ CMT does not affect life expectancy.
- ▶ CMT is sometimes surgically treated.
- ▶ CMT causes foot-drop walking gait, foot bone abnormalities, high arches and hammer toes, problems with balance, problems with hand function, occasional lower leg and forearm muscle cramping, loss of some normal reflexes, and scoliosis (curvature of the spine).
- ▶ CMT has no effective treatment, although physical therapy, occupational therapy, and moderate physical activity are beneficial.
- ▶ CMT is usually inherited in an autosomal dominant pattern, which means if one parent has CMT, there is a 50% chance of passing it on to each child.
- ▶ CMT Types that can now be diagnosed by a blood test include 1A, 1B, 1C, 1D (EGR2), 1E, 1F, 1X, 2A, 2B, 2E, 2F, 2I, 2J, 2K, 4A, 4C, 4E, 4F, 4J, HNPP, CHN, and DSN.
- ▶ CMT is the focus of significant genetic research, bringing us closer to solving the CMT enigma.