

Community Members Taking Action



2015 ANNUAL REPORT Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has.

- Margaret Mead

Your Team



Ori BashWest Coast Regional
Branch Manager



Michelle Hayes Midwest Regional Branch Manager



Kim Magee Director of Finance



Bethany Meloche Director of Social Media



Elizabeth Ouellette Board Member Full-Time Volunteer



Susan Ruediger
Director of Development



Marcia Semmes
Director of Print
Communications



Jeana SweeneyDirector of Community
Services

I am happy to report to you that our small group of thoughtful, committed citizens is changing the world of CMT. In 2015, the Charcot-Marie-Tooth Association saw improvement in every meaningful metric in our march toward a first treatment for CMT. Along the way, we also provided help to increase the quality of life for the people who have it!

Together, we raised \$3,799,969 in 2015, another record for the third year straight and up more than half a million dollars from the previous year. Where did that money come from? Community members taking action. The branches led the charge as more than 500 people participated in the first-ever All-Star event in September, walking, rolling, running and biking "4CMT" and raising \$218,000 in the process. The CMTA branch system, which grew to 77 branches by year's end, didn't limit its efforts to Awareness Month though: All in, CMTA branches contributed an astounding total of \$624,373 to the CMTA and STAR in 2015. CMTA board members also got in on the fundraising efforts—just four board events contributed almost half a million dollars to CMTA coffers.

Another \$1.2 million came from the efforts of two generous donors, who recognized that funds were needed as CMT 1A research efforts moved closer to clinical trials. They understood that projects were needed to determine how to quickly measure clinical improvements, how to determine why people with CMT 1A have varying symptoms, even in the same family, and how to identify the most promising compounds for testing. They challenged the CMT community to match their donations and fund that work. Each time, community members answered the call.

Where did the money go? In 2015, the CMTA spent some \$2.1 million on translational projects focused on CMT 1A, 2A, 1B, 2E, 1X, and 4 that cover more than 90 percent of CMT cases. The CMTA collaborated with several pharmaceutical companies that have bought into the CMTA's STAR consortium model in our search for a first drug to treat patients. Nearly 2.5 million compounds have now been screened and testing on the resulting hits is ongoing. CMT Centers of Excellence, subsidized by the CMTA, NIH, and MDA, now number 21 globally, providing multidisciplinary touch points for CMT patients and their families under one roof.

The CMTA also allocated resources in 2015 to continue identifying the exploding numbers of CMTA patients. Our goal is to identify as many CMT patients as possible, as well as the type of CMT that affects them.

My gratitude goes to all members of our invested and committed group—our donors, the seven-person staff of the CMTA, our 77 branch leaders, the invested and voluntary Board of Directors, active Advisory Board members, and dedicated STAR Scientific Board and Therapy Board members. Their valuable and treasured participation and contributions made 2015 a remarkable and record year! Together we can achieve a world without CMT.



Patrick A. Livney
Chief Executive Officer



Together, we raised \$3,799,969 in 2015, another record for the third year straight and up more than half a million dollars from the previous year.



In 2015, the CMTA earned Charit Navigator's four-star rating for the second year in a row. Only 21 percent of the nonprofits rated by Charity Navigator get two consecutive 4-star evaluations, indicating that the CMTA 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. The CMTA has also consistently been awarded the Independent Charities Seal of Excellence by the Independent Charities of America an award given to organizations that annually meet the highest standards of public accountability, program performance and cost-effectiveness.





RESEARCH

STAR, the CMTA's research initiative, was created with one goal: To find treatments to slow, stop or even reverse the progression of CMT. The collaborative research is done by the world's leading CMT experts in university labs, large pharmaceutical companies and small research organizations, creating groundbreaking, efficient results at each step of discovery.

STAR - CMT Type 1A

CMT Type 1A is caused by the duplication of the Peripheral Myelin Protein 22 (PMP22) gene, which leads to the demyelination of the peripheral nerves. 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.

STAR - CMT Type 1B

Innovative work at the University of Iowa and the National Institutes of Health is underway to determine the role of Myelin Protein Zero (MPZ) in causing 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. In addition, a further effort to develop animal models of CMT1B is now underway at the University at Buffalo.

STAR - CMT Type 1X

Dr. Rudolph Martini at the University of Würzburg, Germany, has been investigating whether human mesenchymal stem cells can ameliorate the neuropathy in a genetically authentic rodent model of CMTX. This is a novel type of treatment for CMT as it seeks to modify the immune system to improve nerve function. His laboratory has also tested in this model compounds obtained via a pharmaceutical collaboration and thought to target immune processes likely affecting CMTX disease progression. Early positive results from antiinflammatory compounds have been demonstrated in laboratory models and further investigation is underway.

STAR - CMT Type 2A

CMT2A is caused by dominant mutations in MFN2. 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. Assays that can measure rescue of the 2A defect at a cellular level are underway in preparation for a high-throughput screen.

STAR - CMT Type 2E

CMT2E is caused by dominant mutations in the neurofilament light protein (NEFL). One result of the mutations is to prevent the neurofilament light proteins from assembling properly, resulting in the formation of aggregates or abnormal filaments. 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.

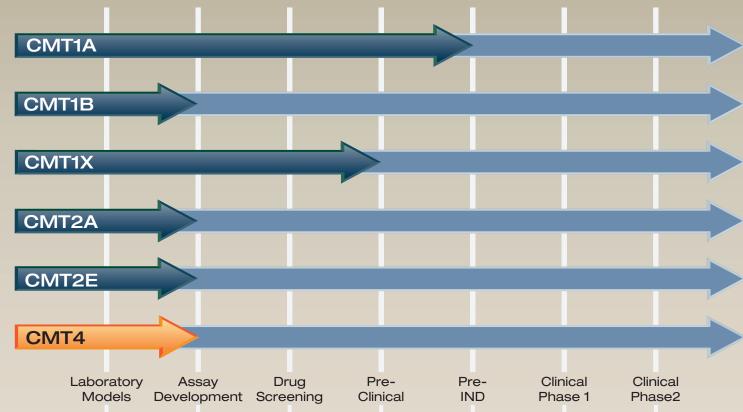
STAR - CMT Type 4

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.

STAR Progress Report

The Strategy to Accelerate Research (STAR)

The CMTA is aggressively fighting to find a pharmaceutical treatment, and, ultimately, a cure for all types of CMT. We currently have protocols for developing treatments for the types of CMT that affect approximately 90 percent of all people with CMT. The chart below shows the status of each of those projects.



For each type of CMT, develop a robust population with offspring that exhibit symptoms and can be used to test candidate pharmaceuticals.

Build cellular assays, reproducing cells with each type of CMT that can be used for the rapid testing of many different chemical compounds.

at the National Institutes of Health and major companies.

Test the candidate drug compounds by giving doses to the symptomatic laboratory models and measuring their improvement.

est the compounds or dosage, toxicity and viability in private and academic laboratories to determine the exact dosage that will improve the root

Conduct pre-clinical A small group of strong, healthy people without CMT will take the dose of the compounds to test for human side

phase 2 trials. the CMT Centers of Excellence, small

Recruited through groups of people with type-specific CMT will participate in pharmaceutical trials to determine the effect the drug will have on CMT.

The Scientific Advisory Board provides scientific input for projects that are ongoing or proposed. The members are:

John Svaren, PhD, Chair, University of Wisconsin

Frank Baas, MD, PhD,

University of Amsterdam, the Netherlands

Robert H. Baloh, MD, PhD, Cedars-Sinai Medical Center

M. Laura Feltri, MD, University at Buffalo

Jun Li, MD, PhD, Vanderbilt University

Rudolph Martini, PhD, University of Würzburg, Germany

Albee Messing, VMD, PhD, University of Wisconsin

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

Michael E. Shy, MD, University of Iowa

Gabsang Lee, PhD, Johns Hopkins University

Lawrence Wrabetz, MD, University at Buffalo

Stephan Züchner, MD, PhD, University of Miami

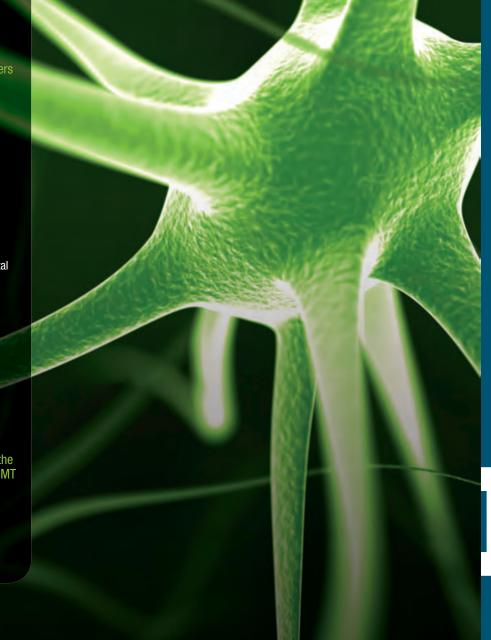
The Therapy Expert Board is responsible for ensuring that each research project has translational value for the CMTA's STAR mission of developing therapeutics for CMT patients. The members are:

Mark Scheideler, PhD, Chair, HumanFirst Therapeutics LLC

Lars J. Knutsen, PhD, Discovery Pharma Consulting LLC

Claes Wahlestedt, MD, PhD, University of Miami

Tage Honore, PhD, Aestus Therapeutics Inc.



The CMTA's funding and operations focus is on research that will lead as directly as possible to therapeutic treatments of CMT. To further this goal, the CMTA revamped its Scientific Advisory Board in 2015, bifurcating it into two function-driven entities. The newly formed STAR Advisory Board comprises a Scientific Expert Board (SEB) and a Therapy Expert Board (TEB).





Addex Therapeutics is collaborating with the CMTA to evaluate the efficacy of ADX71441 as a pharmacological agent in preclinical neurological and behavioral models of CMT1A and to assess its potential to impede the development of motor and sensory control defects associated with normal disease progression.

Genzyme, a Sanofi company, is collaborating with STAR investigators to leverage the Sanofi U.S. high-throughput screening facility in Tucson, Arizona. The first round of screening, which included compound libraries with more than 2 million small molecules, has been completed and additional screening is underway.

Ionis Pharmaceuticals is the leading company in antisense drug discovery and development, and is working with the CMTA to exploit a novel drug discovery platform that provides a direct route from genomics to drugs.

The NIH Molecular Libraries Program provided its complete library of 500,000 compounds for screening. The first round of screening has been completed and further testing of selected compounds in both rodent and stem cell models has begun.

The New York Stem Cell Foundation collaborated with the CMTA to develop a bank of induced pluripotent stem cell (iPSC) lines for a variety of neuropathy disorders of known genetic causation.

Pfizer-Neusentis provided a selected subset of the Pfizer Chemogenomic library for screening in the CMT1A project. The CMTA has completed screening several thousand of these compounds. A number of potential drug targets that regulate PMP22 expression were identified and are being further validated.

PsychoGenics, a leader in specialty preclinical contract research and drug discovery services, provides biomarker and behavioral testing support to the STAR network.

ReNovo Neural is a leading specialized service company that provides expert histological and pathological analysis for animal models of neurological disease. They support the CMTA via the characterization of new animal models of CMT and by evaluating the effects of experimental drug treatment in these models.



CMTA branches are the main touchpoint between the national organization and its members: The CMTA provides members education and support and members raise funds and awareness for the parent organization.

At the beginning of 2015, we renamed our Support and Action Groups CMTA Branches in recognition of the vital and organic role they play in furthering our mission. By year's end, there were 77 branches providing help and hope to members who met regularly to share their own personal experiences and hear from leading clinicians and scientists provided as guest speakers by

The branches also raised awareness and funds for the **CMTA. Awareness Month 2015** was the biggest ever. More than 500 people participated in 34 All-Star events in September, walking, rolling, running and biking "4CMT" and raising an amazing \$218,000 in the process.

In addition to the All-Star events, a record 28 states proclaimed September 2015 CMT Awareness Month, as did scores of cities and counties. Thirty people signed up to "Go Blue 4 CMT," dying their hair and raising more than \$20,000 for the CMTA. Community members also came up with other creative ways to raise awareness about CMT: They decorated cakes, wrapped trees in blue ribbon and put the letters "CMT" up in lights for all to see.

Community members held a number of other fundraisers and events throughout 2015, proving that anything—a birthday party, a fishing tournament, or a volleyball game—can be turned into a fundraiser.

Pete and Debbie McHugh used the occasion of Pete's **70th birthday to raise money for the CMTA:** Pete wanted to do something special for their 11-year-old granddaughter Lucy, who was the first in their family to be diagnosed with CMT. The whole family pitched in and with the matching fund donation, Pete's birthday raised more than \$10,000. "Expect more events like from our family and friends," Debbie said afterward. "It's an easy and fun way to increase awareness and support the important work of the

Sam Houston State University hosted its first-ever CMT Awareness Night during the Women's Volleyball Team's home game against Texas State in honor of 7-year-old Boston Gray. Coach Gray's grandson, who has CMT. CMT STARs were sold throughout the town, campus and the night of the event for \$10 apiece with all proceeds going to CMT research. The game raised more than \$21,000 for CMT research and plans are in place to make it an annual event.

In Matlacha, Florida, Ellen Eagle teamed up with Little Caesars franchise owners Jim and Maralisa Addis for the 2nd Annual CMTA Shark Fishing Tournament on September 19. Ellen, her son Joel Lodolce and her sister Patty Bolton started the tournament because they all have CMT and recognize just how crucial funding the research is. Ellen was scouring the community for sponsors - and had almost reached the end of her

rope—when she reached out to the local Little Caesars. The request hit close to home for Jim and Maralisa: Unbeknownst to Ellen, CMT touches three generations of the Addis family. The tournament, raffle and silent auction raised \$3,404 and at the close of the afternoon, the Addis family presented the CMTA with a \$10,000 check from Little Caesars, family and friends.

All in, CMTA branches contributed an astounding total of \$624,373 to the CMTA and STAR in 2015.

Seven Patient/Family Conferences in 2015

The Charcot-Marie-Tooth Association is all about making **connections**—between patients and doctors, among the doctors and researchers who do the vital research on a treatment for CMT, and between patients and other patients. Nowhere is this more evident than in the Patient/Family Conferences the CMTA holds each year, often in collaboration with a CMT Center of Excellence or multi-disciplinary CMT clinic.

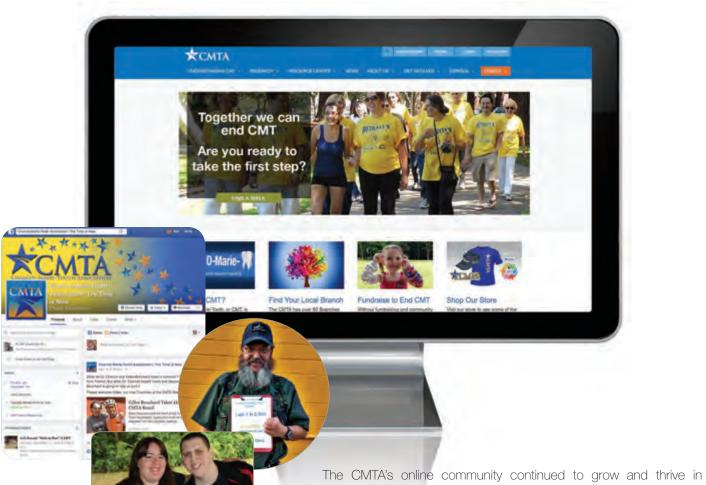
These conferences allow people with CMT and their families to get up-to-date information on the CMTA's Strategy to Accelerate Research (STAR) from the scientists leading the effort to find treatments for CMT. They also feature presentations from local clinicians and health care providers on topics such as the causes and diagnosis of CMT, physical and occupational therapy, orthotics and bracing, orthopedic surgery, genetic counseling and more. Just as importantly, the Patient/Family Conferences allow people who have CMT to connect with other people who have CMT, some of them for the first time in their lives.

In 2015, the CMTA hosted a record seven Patient/Family **conferences**—full days in collaboration with Massachusetts General in Boston and Vanderbilt in Nashville and half days in Los Angeles, Charlotte, Dallas, Denver and Chicago. More than 700 people heard presentations by experts in a wide range of medical disciplines, CMT advocates and motivational speakers.

The two full-day conferences also included youth outings—an amphibious "duck tour" of Boston and The Escape Game in Nashville.



CMTA branches are the main touchpoint between the national organization and its members



2015, adding members, services and media. We launched a new website in 2015 (with a new, user-friendly Resource Center) that includes a revamped, private Emotional Support Group moderated by a psychotherapist with CMT.

- Our Facebook community has grown to more than 25,000 followers (up from 20,000 in 2014) who take part in a constant stream of conversation, posting hundreds of comments daily.
- In addition, the CMTA has 2,100 Twitter followers: 1,500 Pinterest followers; and 400 LinkedIn professionals. In the summer of 2015, an intern started an Instagram account to help the CMTA reach a younger demographic and it now has 450 followers.
- And in an online Awareness Month campaign, more than 700 people took to Facebook in September to proudly proclaim to the world that each was "1 in 2,500." The resulting photos were gathered in a huge album that illustrates that community members truly are one big family, connected to one another and committed to finding a solution to CMT together (on.fb.me/1gSRi2D).

COMMUNIT

The CMTA is governed by a voluntary Board of Directors who bring both professional competence and personal commitment to their task. These business owners, executives, doctors and lawyers oversee the organization's operations and strategy for promoting awareness, funding research and finding a cure. Because they are all affected by CMT in one way or another, they are deeply committed to the organization and annually make significant financial contributions to ensure that it meets its goals. The Board of Directors welcomed one new member in 2015. Laura Fava, a lawyer in Toronto, joined the board in August, saying "[M]y hope is to contribute to the CMTA through fundraising and helping to initiate CMT Canada."

Herb Beron (Chairman) Executive Director , Financial Advisor Morgan Stanley Wealth Management Florham Park, NJ

Elizabeth Ouellette (Vice Chair)

Civic Volunteer and Community Relations Expert, Los Altos, CA

Gary Gasper (Treasurer)
Partner, Ernst & Young
Co-leader of the Washington Council Ernst & Young Group, Washington, DC

Steve Weiss (Secretary) Senior Vice President - Health Edelman, Washington, DC

Stephen Blevit, Esq. Partner, Sidley Austin, LLP Los Angeles, CA

Laura Fava

Owner/Lawyer Laura Fava Barrister & Solicitor Toronto, Ontario

Alan KorowitzExecutive Vice President of Sales Premiere Networks, a division of Clear Channel Media + Entertainment New York, NY

Steve O'Donnell President, Steven F. O'Donnell Inc. Elkridge, MD

Chris OuelletteVice President of Operations and Service Alpha Analytical

Shelburne, VT

Phyllis Sanders, Esq. Partner, The Sanders Law Firm Mineola, NY

Steven Scherer, MD, PhD

Professor of Neurology Perelman School of Medicine University of Pennsylvania Philadelphia, PA

Michael Shy, MD Professor of Neurology Co-Director of Neuromuscular Program, Co-Director of MDA Clinic Director of the CMT Clinic Carver College of Medicine
University of Iowa, Iowa City, Iowa

John Svaren, PhD Associate Professor

Department of Comparative Biosciences, Waisman Center University of Wisconsin-Madison Madison, WI

Peter I. Warfield, MD, MPH FAAP Spring Valley Pediatrics Washington, DC

Lawrence Wrabetz, MD
Professor of Neurology and
Biochemistry Director, Hunter James Kelly
Research Institute University at Buffalo, Buffalo, NY

BOARD FUNDRAISING EFFORTS AND EVENTS



Team Julia

Sixth Annual New York Cocktail Reception

The Second Annual Cycle 4 CMT

Sixth Annual New York Cocktail Reception
Board members Phyllis Sanders and Alan Korowitz held the Sixth Annual New York CMTA Cocktail Reception at the glamorous hotel Essex House on October 26, 2015 at 6:30 P.M. A buffet dinner, entertainment by mentalist Oz Pearlman, music and an auction with items such as concert tickets, sporting event tickets, trips, jewelry and memorabilia marked the evening, which raised \$185,246 for the CMTA.

The Second Annual Cycle 4 CMT

The Ouellette family engaged Vermonters and their enthusiasm for outdoor activity in the Second Annual Cycle 4 CMT on September 20, 2015, raising \$118,028 (which was doubled in the 1A match for CMT research) and increasing awareness among the 150 people who attended. Cyclists chose from a number of spectacular routes through the picturesque towns of Charlotte and Shelburne, Vermont.

On Sunday, September 6, 2015, Board Chair Herb Beron, his wife Rachael and other members of Team Julia '15 swam across Lake Valhalla in Montville, New Jersey, to raise funds for STAR. Now in its ninth year the swim has raised close to \$700,000 for the CMTA since its inception in 2007, \$74,457 in 2015 alone.

Board member Steve O'Donnell teamed up with Branch Leaders Clark Semmes and Missy Warfield for the Oxford Biathlon on May 30, 2015, in tiny Oxford, Maryland. Under a cloudless blue sky, 84 participants ran, walked, swam, and rolled for CMT, raising \$86,613. in the process.

More than 700

people took to

Facebook in

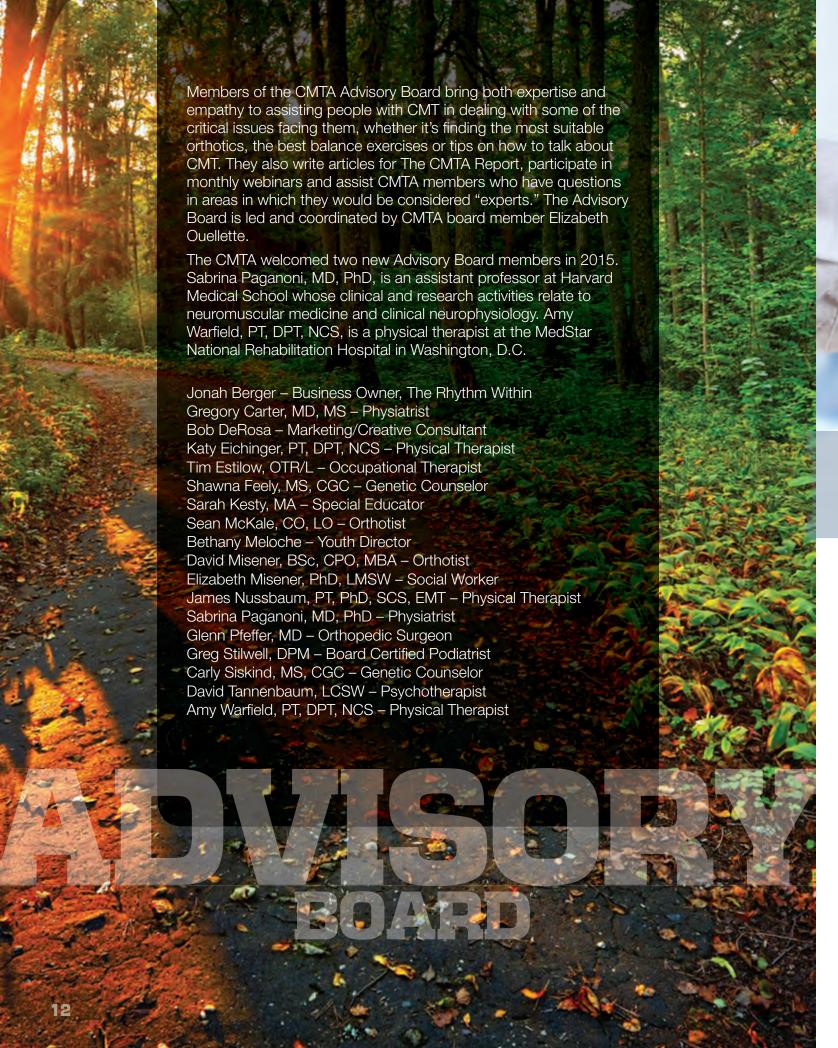
September to

proudly proclaim

to the world that

each was

"1 in 2,500."





The CMTA began 2015 with five corporate partners and added three over the course of the year. In addition to sponsoring the CMTA, each offers products or services that can improve the quality of life for CMTers

Aetrex Worldwide, Inc., founded in 1946, is a global leader in pedorthic footwear and orthotics. It offers attractive shoes that feature extra depth and width to accommodate in-shoe orthotics or AFOs.

Allard, USA distributes the ToeOFF Family of carbon composite devices designed specifically to assist with foot drop, ankle instability and proximal neuromuscular weakness, all symptoms of CMT.

Balance Walking is an all-encompassing health and fitness program incorporating walking poles that can be done in as little as 15 minutes per day.

CosySoles makes microwave heated slippers that provide comforting warmth and the freedom to stay mobile to millions who suffer from cold and painful feet caused by peripheral neuropathy.

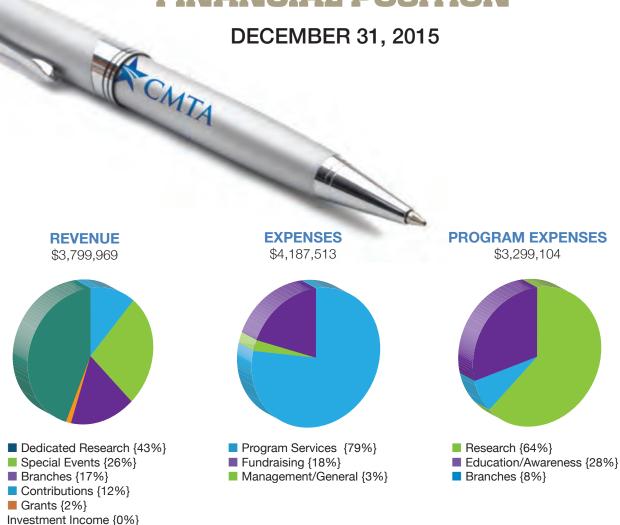
GeneDx launched the Hereditary Neuropathy Panel, a genetic testing panel aimed specifically at testing for different types of CMT, in 2014. With 53 CMT-causing genes available in the Neuropathy Panel, GeneDx delivers an exact genetic diagnosis for 50-70 percent of people with symptoms of CMT.

Hanger Clinic has more than 1,300 clinicians specializing in the provision of orthotic and prosthetic solutions. Its teams annually deliver effective clinical systems, innovative technologies and outstanding customer service to more than 1 million patients at over 750 Hanger Clinic locations nationwide.

Invitae is a genetic testing company with a comprehensive panel for CMT, as well as autosomal dominant, autosomal recessive, X-linked and HNPP (Hereditary Neuropathy with Liability to Pressure Palsies) panels.

Union & Fifth raises money for non-profits by selling donated, gently worn women's designer clothes.

CONSOLIDATED STATEMENT OF FINANCIAL POSITION



Revenues

Contributions
Special Events
Support Group
Investment Income
Grants
Dedicated Research Revenue

Total Revenues

Expenses

Program Services
Management and General
Fundraising
Total Expenses

Program Expenses

Research
Branches
Education & Awareness
Total Expenses

December 31, 2015

\$459,161 \$1,009,550 \$624,423 \$1,191 \$49,993 \$1,655,651 \$3,799,969

\$3,299,104 \$107,300 \$781,109 **\$4,187,513**

\$2,092,549 \$265,128 \$941,427 **\$3,299,104**

December 31, 2014

\$974,072 \$452,098 \$336,617 \$2,615

\$1,387,600 \$3,153,002

\$2,386,232 \$80,061 \$331,800 \$2,798,093

\$1,584,430 \$214,760 \$587,042 \$2,386,232 Two donors challenged the CMT community to match their donations in 2015. Each time, community members answered the call.

In a summer campaign, one CMTA family's generous friend challenged the CMT community to match his \$500,000 donation to the CMTA's STAR program for CMT1A. The community came through.

In a "Thanksgiving Match," another generous family donated \$100,000 to the CMTA1A program on the condition that it be matched dollar for dollar during the month of November. As the family said in their message to the community: "November is the month of Thanksgiving and those of us who daily live with CMT do, indeed, have much for which to be grateful. Even when we are tired, in pain, discouraged and feeling alone, there are scores of scientists around the world diligently working on our behalf, focused on finding a treatment for CMT."

CHALLENGE SPARITES

14

2015 TI FSTOR

Grew revenues from \$3,153,002 in 2014 to \$3,799,969 in 2015

Grew CMTA branches to 77

Grew Facebook fans by 25 percent, from 20,000 in 2014 to 25,000 in 2015

Increased total web site visits from 298,986 in 2014 to 314,205 in 2015, 32.4 percent of them new visitors

Increased research funding from \$1,584,429 in 2014 to \$2,092,549 in 2015

STAR produced a number of exciting results in 2015, chief among them authentic human mutation in-vivo models for CMT 1B, CMT 2A and CMT 2E. STAR CMT1A is closing in on a therapeutic treatment utilizing cellular assays the CMTA developed and used to screen compounds from libraries at Sanofi-Genzyme, the National Center for Advancing Translational Sciences (NCATS), and other collaborators. Projects were also funded in CMT 1X and CMT 4C! The CMTA's contract research organization partners, PsychoGenics and the NYSCF, in conjunction with the CMTA's consortium network enabled these important CMTA STAR developments. Stay tuned as we anticipate 2016 to be a breakthrough year!





Charcot-Marie-Tooth Association

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SOCIAL MEDIA LINKS:

Twitter: @CMTASTAR • www.facebook.com/CMTAssociation • www.linkedin.com/company/charcot-marie-tooth-association www.youtube.com/CMTAssociation