



2013: A year
of accomplishment
and encouragement

The CMTA Annual Report



a committed team

success can only be achieved by passionate, dedicated individuals

It is my pleasure and my honor to serve as the CEO of the CMTA. Each of the staff members, myself included, either have CMT or have a child, husband or close friend who has been diagnosed with CMT. Who better to work tirelessly to find the treatments and ultimate cure for this debilitating disorder? Each of us is driven by passion and the need for urgency in finding the solution to CMT for ourselves and our loved ones. We are supported by a core of researchers who have dedicated their entire lives to unraveling the mystery of CMT and putting an end to its destruction. Won't you join us, as well, to accomplish our mission of the development of new drugs to treat CMT and improve the quality of life for everyone with CMT?

CHARCOT-MARIE-TOOTH ASSOCIATION

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

thanks to our community...

- ★ One drug candidate showing PMP22 down-regulation in a long-term CMT1A study could be the first candidate medication making it through the STAR process for potential human trials in 2014.
- ★ The CMTA added new Support and Action Groups in four new states, so we now have 64 groups in 34 states!
- ★ During CMT Awareness Month, over 24,000 people visited our website, we held our first live webinar featuring Dr. Michael Shy, and more than \$45,000 was raised by our community members.

Your commitment to us has allowed the CMTA to realize these and other successes we only dreamed possible five years ago, and I am continually overjoyed by the abundant support from our community—in raising awareness, sharing stories, and financially supporting the CMTA.

Because our ultimate vision is a world without CMT, I am especially proud of our scientific team and the major milestones we reached this year in drug discovery. In addition to pre-clinical testing of the medication mentioned above, we obtained agreements for collaboration with two major pharmaceutical companies, and we are continuing conversations with three others to help with the research and development of pharmaceutical therapies.

We also added several world-class investigators to the STAR network, including Dr. Robert H. Baloh at Cedars-Sinai in Los Angeles and Dr. Mario Saporta at LaNCE in Brazil, and we now have 17 Centers of Excellence worldwide, including Nemours Children's Hospital in Orlando, Florida.

Our Support and Action Groups continue to be a vital part of the CMTA community, reaching over 11,000 people, providing them with information and resources, and allowing them to share stories, experiences and ideas for coping with CMT. In 2013, these members of the CMTA community also took awareness and fundraising by storm—from dinners and walks to minor league baseball awareness events, our groups raised close to \$375,000!

In closing, I urge you to continue to fight for those with CMT. Tell your story about why you are a part of our family, share information via social media about the CMTA, connect locally and nationally with people in our family and continue to provide the support you have. Together, as a family, we will share more tremendous success throughout 2014.

Patrick A Livney
Chief Executive Officer



2014 goals

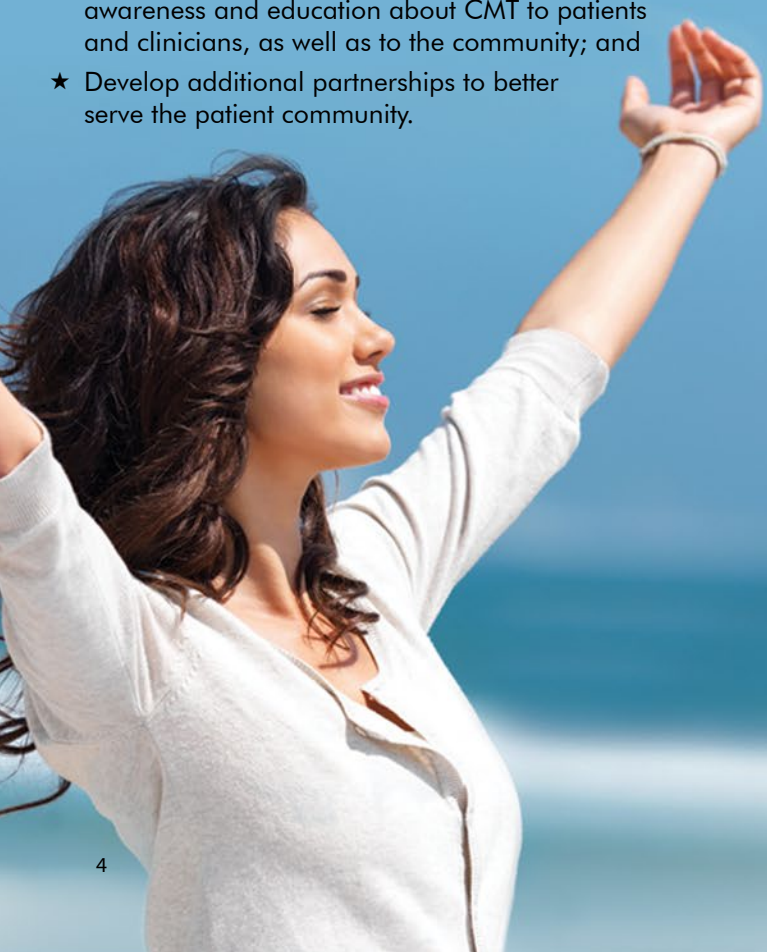
The CMTA's mission is to support the development of new drugs to treat CMT, to improve the quality of life for people with CMT and, ultimately, to find a cure. Our goals for 2014 are poised to bring greater success to the CMTA and to the communities we serve.

To support the development of new drugs to treat CMT, and, ultimately, to find a cure, we will:

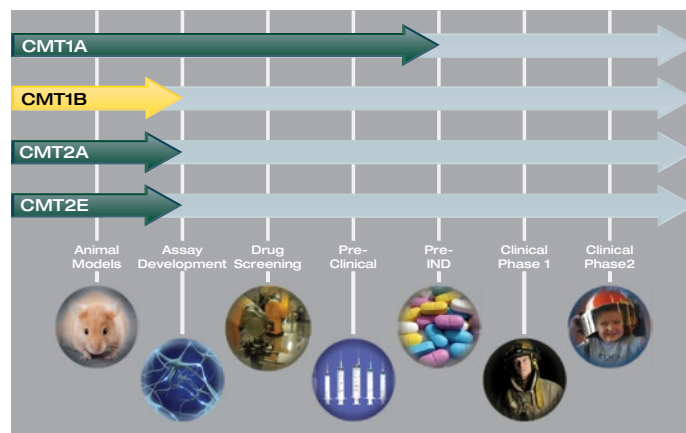
- ★ Launch a STAR program for CMT Type 1B in the first quarter of the year;
- ★ Have laboratory models ready for candidate drug testing by the second quarter of 2014;
- ★ Plan to develop the first human trial for a candidate compound for Type 1A; and
- ★ Continue to grow our partnerships with pharmaceutical companies as our research continues to hit major milestones in the search for treatments and cures.

To improve the quality of life for people with CMT, we will:

- ★ Host two Patient/Family Conferences in California;
- ★ Grow the number of Support and Action Groups across the United States and North America;
- ★ Publish the *CMT Survivor's Guide* and expand awareness and education about CMT to patients and clinicians, as well as to the community; and
- ★ Develop additional partnerships to better serve the patient community.



7 STAGES OF DRUG DEVELOPMENT



In addition to the definition of protocols for CMT1B, our 2013 progress included two more checkmarks for CMT1A:

- Develop Laboratory Models
- Build Cellular Assays
- Screen Known Compounds
- Pre-Clinical Investigation
- Pre-IND Validation
- Clinical Trials, Phase 1
- Clinical Trials, Phase 2



The Time Truly Is Now!

the CMTA's STAR is shining brightly...

CMT1A - Use of CMTA-sponsored assays at the state-of-the-art drug screening facility directed by Dr. James Inglese at the National Institutes of Health National Center for Advancing Translational Science (NCATS) previously identified four compounds for treating Type 1A that are FDA approved. Validation and testing of such compounds was initiated in the laboratory of Dr. Michael Sereda and Dr. Klaus-Armin Nave using one of the best laboratory models of CMT1A. Positive early results in lowering PMP22 have been confirmed for multiple drug candidates. Dr. Michael Shy and the STAR team are conducting safety and toxicity tests to prepare for early stage human clinical trials. Additionally, the STAR team has developed a validated CMT Neuropathy Score which will be used to assess the efficacy of these compounds in clinical trials.

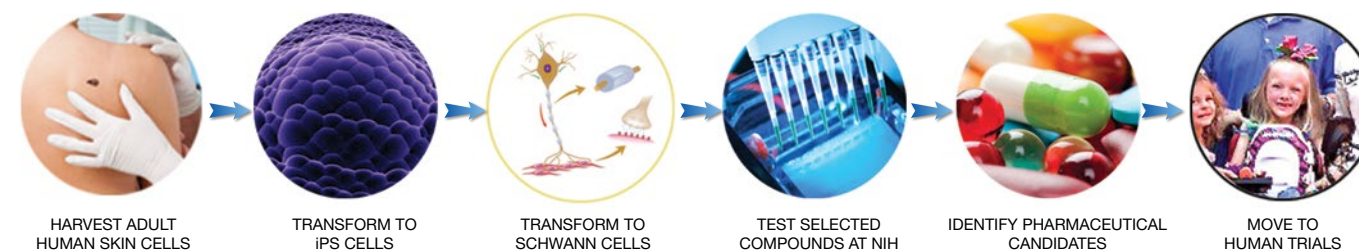
CMT1B - The CMTA is launching a major new initiative on CMT1B, which may be caused by many different mutations in the Myelin Protein Zero (MPZ) gene. CMT1B laboratory models are being developed by Dr. Lawrence Wrabetz and Dr. Michael Shy. One of the major questions for developing a treatment of CMT1B is to determine how many of the variable MPZ mutations cause neuropathy.

CMT2A - On one testing platform, the CMTA is funding a project to develop cell-based assays to detect compounds that increase the level of the MFN1 gene. These assays will be screened using state-of-the-art compound collections at NIH/NCATS, and laboratory "knock-in" models are being developed to evaluate the CMT2A pathological process in vivo and provide a tool for pre-clinical testing. In work on a second testing platform, Dr. Michael Shy is collaborating with Drs. John Svaren and Anita Bhattacharyya at the Induced Pluripotent Stem Cell service at the University of Wisconsin to develop several human stem cell lines derived from CMT2A patients. These stem cells will be differentiated into human motor neurons and will be used to evaluate candidate drugs for CMT2A.

CMT2E - In CMT2E, dominant mutations in the NEFL gene prevent the neurofilament light protein from assembling properly and result in the formation of aggregates or abnormal filaments. In collaboration with Dr. James Inglese, Dr. Ronald Liem is leading a project to find small molecules that decrease aggregate formation and/or promote the formation of normal appearing neurofilament. Demonstration of these effects would be identified by rescuing the effects of dominant NEFL mutants in cells derived from CMT2E patients and in laboratory models of CMT2E.

CMT1X - Dr. Rudolph Martini at the University of Würzburg, Germany, is investigating whether human mesenchymal stem cells can ameliorate the neuropathy in a genetically authentic laboratory model of CMT1X. This is a novel type of treatment for CMT, as it seeks to modify the immune system to improve nerve function.

STAR's Transformation Project



Another initiative is to use stem cell technology to test drug candidates in adult stem cell-derived human Schwann cells. This work was initiated in the laboratory of Dr. Anita Bhattacharyya at the University of Wisconsin, who has developed procedures to make the most mature Schwann cells from stem cells that originally come from adult human skin cells. Using the Schwann cells that were developed, Dr. Bhattacharyya was able to observe reduction of PMP22 levels with some of our lead compounds. Dr. Robert Baloh has achieved encouragingly similar effects in stem cell-derived Schwann cells from CMT1A patients. Therefore, these pioneering studies sponsored by the CMTA have yielded another valuable testing platform for candidate medications that will be used to prioritize drug candidates for human clinical trials.



2013 fundraising efforts...

BOARD MEMBER EVENTS

New York Athletic Club

Alan Korowitz and Phyllis Sanders organized the fourth CMTA Update event at the famous New York Athletic Club on October 28, 2013. More than 100 guests attended, and over \$159,000 was raised for STAR.

San Francisco Giants Fantasy Baseball Camp

Vasi Vangelos participated in a fantasy baseball camp in honor of the CMTA. His fundraising efforts raised \$34,350 for research.

TeamJulia

As part of a Labor Day tradition, Chairman of the Board Herb Beron hosted the 7th annual TeamJulia Swim for the Cure at the Lake Vahalla Club in Montville, NJ. The event included over 30 swimmers and raised approximately \$70,000.

Swim for the Cure

Board member Steve O'Donnell returned to swimming the Chesapeake Bay in 2013 and, supported by his family and friends, his twelfth year of swimming raised \$43,335 to help fund research projects.

SUPPORT AND ACTION GROUP EVENTS

The Warfield Challenge

Missy and Seth Warfield challenged the supporters of the CMTA to match their donation of \$150,000 to further the work of STAR. In 2013, the amount given in support of the match was \$186,284. Overall, the Warfield Challenge raised \$346,716.

Baseball Fundraisers

Three teams, the Bowie, MD Baysox, the Camden, NJ Riversharks and the Somerset, NJ Patriots hosted fundraising nights at their baseball games, raising over \$12,000 for CMT research.

The CMTA Circles of Friends

The "Circle of Friends" campaign is based on a personal appeal. By reaching out to friends and family with a personal letter or email and asking them to make a contribution, the number of people who support the work of the CMTA has been greatly enlarged. There are 49 Circles on the CMTA's website and one of them, Vittorio Ricci's, has raised over \$34,000.

Canadian Fundraiser

On September 29th, the Third Annual Walk for Fergus Fundraiser was held with over 150 participants joining the walk, the face painting, an Archy coloring station and more. The total funds raised from this event came in at \$16,700.

Stepping it UP

Every year Jeana Sweeney organizes a CMT awareness and education event for elementary school children, which also raises funds for CMT research. In 2013, Jeana repeated her success with the Stepping it UP program and raised over \$11,900.

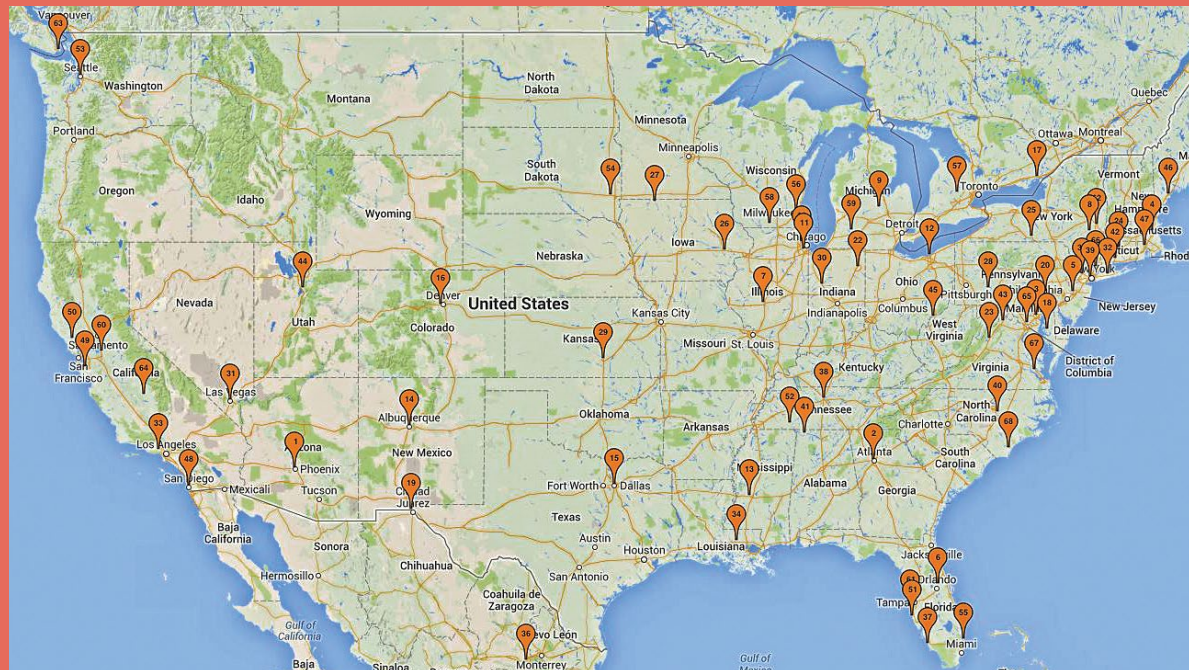


support & action groups

The CMTA Support and Action Group network is growing every year.

In 2013, we started new Support and Action Groups and increased the number of people involved to approximately 11,000.

Now, with more than 60 Support and Action Groups across North America, including Canada and Mexico, the CMTA Support and Action network plays a critical role in helping members deal with the challenges they face every day because of CMT. Whether from members sharing their own personal experiences or from leading clinicians and scientists provided as guest speakers by the CMTA, attendees acquire invaluable knowledge about the genetics of CMT, mobility issues, bracing needs, physical therapy, and assistance with other adaptive devices.



Support and Action Groups are also remarkable for their ability to provide members with the opportunity to take part in the mission of the CMTA. Many groups organize fundraisers, enabling members to advance a cause that is close to their hearts and have a direct hand in the search for a cure.

The Orlando Patient/Family Conference

On November 11, 2013, the 200-plus attendees at a conference in Lake Buena Vista, Florida, listened to a presentation by Richard Finkel, MD, and Chief of Neurology at Nemours Children's Hospital in Orlando. His facility is the newest pediatric CMT Center of Excellence. He spoke about the need for more focus on muscle weakness, pain fatigue and joint tightness in children with CMT. Other presenters included Steven Frick, MD, Chairman of Orthopedic Surgery at Nemours, Sean McKale, CO, LO and David Misener, CPO, who discussed orthotic management of CMT. Stephan Züchner, MD, PhD, Chairman of Human Genetics at the University of Miami, discussed genetic sequencing technology, and Michael Shy, MD, Director of the CMT Clinic at the University of Iowa, discussed how doctors can measure changes in patients over time and the impact of the first human clinical trials.

Rounding out the Orlando weekend were two other events. On Friday night, Dr. Finkel hosted a "meet and greet" to show attendees around the new pediatric clinic he directs, and on Saturday, children and teenagers were treated to a CMTA Youth Outing and day of fun at the famous Orlando amusement park.

CMTAthletes - Triathlete Donna DeWick launched CMTAthletes, a program for all types of athletes to raise awareness and funds for the CMTA. Her Facebook forum offers support and advice for living an active lifestyle with CMT. This program also designs unique sports apparel and gear for any sport.

The CMT Forum is an online chat room on the CMTA website (www.cmtausa.org) where people living with CMT can post questions and answers, challenges and successes to share with the CMT community on a variety of topics.

The CMTA Website and Social Media continue to provide information to a wide range of site viewers. The social media community grew to over 12,000 people. The CMTA Website was visited 235,212 times throughout 2013. In December, the CMTA Parents Group was launched on Facebook. It is a place for parents to share and support each other. In all, the 12,000 people who have "liked" the CMTA's page on Facebook allows the CMTA to reach over 4 million friends of fans regarding CMT issues.

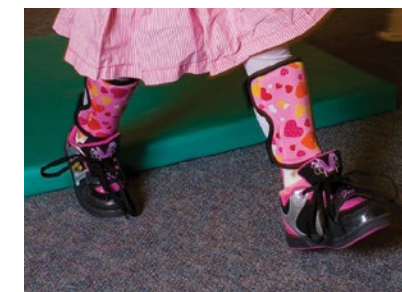
Memberships - The number of active CMTA Community Members totaled 7,050 in 2013 and Premium Members totaled 1,366. As part of our 2013 membership drive, our partner, Aetrex Worldwide, offered a free pair of their shoes to every new Premium Member.

CMT Awareness Month - September is CMT Awareness Month. On the first day of Awareness Month in 2013, over 47,000 people heard about CMT. Awareness Month messages attracted over 1,000 new Facebook fans. Our YouTube channel was viewed over 20,000 times, an increase of 7,000 views over the previous year.

...our corporate sponsors



Aetrex Worldwide, Inc. is widely recognized as the global leader in pedorthic footwear and foot orthotics. The partnership has focused on two critical areas: the delivery of comfortable, well-designed shoe solutions to people with CMT while creating awareness about CMT and the CMTA to the public and trade through Aetrex's broad reach of customers and professionals.



Allard USA Allard distributes the ToeOFF Family of carbon composite devices designed specifically to assist with foot drop, ankle instability and proximal neuromuscular weakness, all signs of CMT. Allard offers educational programs, training and marketing support to increase both consumer and professional awareness of CMT and the CMTA.



Physiotherapy Associates Physiotherapy Associates provided physical therapy, industrial rehabilitation and orthotics and prosthetics services to thousands of patients across the United States. The partnership was focused on educating clinicians about CMT and connecting them to those with CMT across the US.

the CMTA board of directors/ advisory board

The CMTA Board of Directors

A voluntary Board of Directors governs the CMTA, providing a unique combination of professional competence and personal commitment. As business owners, executives, doctors and lawyers, they oversee the organization's operations and its strategy for promoting awareness, funding research and finding a cure. As people whose lives are affected by CMT, each member also makes a significant annual financial commitment to the organization.

Peter Warfield, MD, MPH, joined the CMTA Board of Directors in 2013. He was diagnosed with CMT1A when he was eight years old, and his experiences growing up with Charcot-Marie-Tooth disease led him to a career in medicine. He is a board-certified pediatrician and father of three. He previously worked in public health as a researcher and educator.



The CMTA Advisory Board

The CMTA also welcomed a new member to the CMTA's Advisory Board. Sarah Kesty has a Masters in Special Education and two teaching credentials. She is a passionate advocate for people with disabilities and has experience teaching kindergarten through high-school aged children. She often speaks to groups of teachers, parents and students about disability awareness, advocacy, and maximizing life satisfaction.

Patient advocacy is one of the missions of the new leadership of the CMTA Board of Directors, and the 16 members of our Advisory Board, who come from a wide variety of backgrounds, offer insights from their areas of expertise into some of the important issues faced by people and families affected by CMT.



- Jonah Berger – Business Owner, The Rhythm Within
- Gregory Carter, MD, MS – Psychiatrist
- Katy Eichinger, PT, DPT, NCS – Physical Therapist
- Tim Estilow, OTR/L – Occupational Therapist
- Shawna Feely, MS, CGC – Genetic Counselor
- Sarah Kesty, MA – Special Education
- Sean McKale, CO, LO – Orthotist
- Bethany Meloche – Youth Director
- David Misener, BSc, CPO, MBA – Orthotist

- Elizabeth Misener, PhD, LMSW – Social Worker
- Michael Needleman, JD – Employment Discrimination Attorney
- James Nussbaum, PT, PhD, SCS, EMT – Physical Therapist
- Susan Salzberg, OTR/L, MOT – Occupational Therapist
- Carly Siskind, MS, CGC – Genetic Counselor
- Jason Steinbaum – US Government Advocate
- David Tannenbaum, LCSW – Psychotherapist



CONSOLIDATED STATEMENT OF FINANCIAL POSITION, DECEMBER 31, 2013

DONATIONS

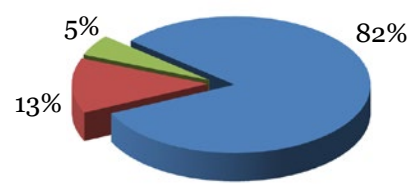
\$1,946,570



- Contributions
- Special Events Revenue
- Support Group Revenue
- Grants
- Investment Income

EXPENSES

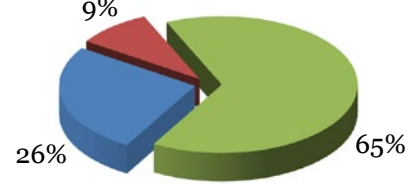
\$1,977,106



- Program Services
- Management
- Fundraising

PROGRAM EXPENSES

\$1,621,531



- Research
- Support & Action Groups
- Education & Awareness

Revenues	December 31, 2013	December 31, 2012
Contributions	\$1,109,793	\$847,651
Special Events Revenue (net of expenses)	\$455,097	\$626,558
Sales of Promotional Products (net of expenses)	(\$434)	(\$3,090)
Support Group Revenue	\$374,949	\$176,833
Investment Income	\$2,165	\$1,778
Grants Received	\$5,000	\$49,000
Total Revenues	\$1,946,570	\$1,698,730
Expenses	December 31, 2013	December 31, 2012
Program Services	\$1,621,531	\$1,028,983
Management and General	\$99,300	\$221,306
Fundraising	\$256,275	\$195,980
Total Expenses	\$1,977,106	\$1,446,000
Increase (Decrease) in Net Assets	(\$30,536)	\$252,730
Net Assets - Beginning of Year	\$1,093,341	\$840,612
Net Assets - End of Year	\$1,062,805	\$1,093,342

CMTA  PATHWAYS

We all hope for a world without CMT. Now, we want to pave the way for those who have been, and may someday be, diagnosed with CMT. We have learned that it is possible that with a bankroll of \$25 million the CMTA can fund the final necessary research to cure CMT1A within the next three-to-five years. Already, there are advanced projects in the works at the NIH and around the world.

Because of this exciting forecast, we have decided to make a pledge of \$150,000 to the CMTA. We will match donations up to that amount and challenge others to build on it. We want to fulfill the CMTA's mission and our dream of "a world without CMT."



—Seth and Missy Warfield, who jump-started the CMTA's \$25 million STAR Pathways campaign with the \$150,000 Warfield Family Challenge.

And, to all our donors, thank you!

While we are, of course, extremely grateful for the Warfield's generous and inspiring gift, we are reminded daily that we are an organization primarily sustained by the gifts, large and small, of our individual donors. Without each of you, the CMTA would not have been able to accomplish major milestones in research, program services and awareness.



CMTA Board of Directors

Herb Beron, Chairman
Steven Blevitt
Gary Gasper
Alan Korowitz
Steve O'Donnell
Elizabeth Ouellette
Phyllis Sanders
Steven Scherer, MD, PhD
Michael Shy, MD
John Svaren, PhD
Vasi Vangelos
Peter Warfield, MD, MPH
Lawrence Wrabetz, MD

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University of Iowa
Steven Scherer, MD, PhD (Co-Chair)
University of Pennsylvania
M. Laura Feltri, MD
University at Buffalo
James Inglese, PhD
National Institutes of Health
Kristjan Jessen, MSc, PhD
University College, London
Lars Knutsen, PhD
Discovery Pharmaceutical Consulting
Rhona Mirsky, PhD
University College, London
Klaus-Armin Nave, PhD
Max Planck Institute for
Experimental Medicine
Brian Popko, MD
University of Chicago
Mary Reilly, MD
National Hospital for Neurology
and Neurosurgery, London
Mark Scheideler, PhD
HumanFirst Therapeutics
Ueli Suter, PhD
ETH Institute, Zurich
John Svaren, PhD
University of Wisconsin
Vincent Timmerman, MD
University of Antwerp
Claes Wahlestedt, MD, PhD
University of Miami
Lawrence Wrabetz, MD
University at Buffalo

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Director of Program Services
Kim Magee
Director of Finance
Susan Ruediger
Director of Development
Jeana Sweeney
Director of Community Services
Bethany Noelle Meloche
Director of Social Media

2013 milestones...

Grew revenues from \$1,858,435 to \$1,946,570

Grew Support and Action Groups from 57 to 64,
including 3 in Canada and 1 in Mexico

Grew Facebook fans from 6,604 in 2012 to
over 12,000 people in 2013

Increased community memberships from 5,114 to 7,050

Increased premium memberships from 876 to 1,366

Web traffic:

Total visits – 235,212

Average visits per month – 19,601

Average unique visits per month – 13,896
61% new visitors

Increased research funding 223% to \$1,052,505

Multiple FDA-approved compounds have shown positive early results for CMT1A by lowering PMP22 production, and STAR researchers have launched a major new initiative in finding treatments for the neuropathy caused by MPZ mutations in CMT1B.



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Social media links:

Twitter: [@CMTASTAR](https://twitter.com/CMTASTAR) • www.facebook.com/CMTAssociation
www.linkedin.com/company/charcot-marie-tooth-association
www.youtube.com/CMTAssociation