COMMUNITY MEMBERS TAKING ACTION

2016 ANNUAL REPORT
In 2008, the CMTA’s Board of Directors launched its Strategy to Accelerate Research, or STAR, with the aim of translating scientific research into therapies for CMT. STAR was based on two key principles: 1) that because the genes that cause CMT have been identified, they can be duplicated in the laboratory and 2) that research should be managed according to sound business principles.

Those key principles include: developing a strategy based on knowing the cause of the disease; finding the best researchers in the world and asking them to implement projects that support the strategy; creating accountability; demanding collaboration; and encouraging partnerships.

Since the inception of the STAR program, the CMTA has moved from basic scientific research to partnering with top pharmaceutical companies on a promising pipeline of potential drug candidates. As we continue to fill and extend this pipeline to more types of CMT, we are also preparing for clinical trials, always the riskiest and most expensive phase of drug development. The CMTA has made amazing progress toward therapies for the most common types of CMT, which affect approximately 90 percent of diagnosed cases. In 2016, the CMTA spent $1.5 million on research.

Where did the money to fund the research come from? Once again, it came from community members taking action. Together we raised $3,177,029 in 2016. The branches led the charge, contributing an astounding total of $812,895 to the CMTA and STAR in 2016. CMTA board members also got in on the fundraising efforts—just four board member events contributed $563,197 to CMTA coffers in 2016.

In addition to funding research, the CMTA delivered high-quality education and awareness resources to the CMT community in 2016, funding two Patient/Family Conferences, a wide variety of educational materials for people living with CMT, 70 branches and a robust virtual presence. And, for the first time ever, the CMTA mounted a weeklong sleepaway camp for kids with CMT, funded by the Pennsylvania Department of Health.

I want to express my deep gratitude to the community members who moved us closer to our goal of a world without CMT in 2016—our donors, the CMTA staff, our dedicated branch leaders, the Board of Directors, Advisory Board members, and dedicated STAR Scientific Board members. Together we can achieve what once seemed impossible.

Gilles Bouchard
Interim CEO

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Start by doing what’s necessary; then do what’s possible; and suddenly you are doing the impossible.

- St. Francis of Assisi
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, resulted in screening their entire compound collection (almost 2 million compounds) and has identified some candidate compound series for treating CMT1A, which are being tested in a variety of secondary assays and animal models. In addition, both laboratory and animal models of CMT1A have been made available to additional CMTA alliance partners for testing of therapeutic compounds, and we hope to be able to share some of these promising data in the very near future. These models include a human stem cell model of CMT1A, made in collaboration with the NYSCF, which, in addition to therapeutics testing, is being made available to the research community. Dr. Michael Shy is leading efforts, together with the members of our Clinical Expert Board (CEB), to develop the best outcome measures for clinical trials of CMT1A therapeutics.

This CMT subtype is caused by mutations in Myelin Protein Zero (MPZ). Board members Dr. Michael Shy and Dr. Lawrence Wrabetz are collaborating with Dr. James Inglese at NIH to explore the unfolded protein response pathway, which plays a causative role in CMT1B. Studies of this pathway have yielded some candidate compounds for treatment, which have been shown to be effective in one model of CMT1B. We are developing studies to see if this approach will treat the three major clinical presentations of CMT1B. Another approach in development is to inhibit the immune response to the nerve damage that occurs in CMT1B.

CMT Type 1X is caused by mutation of the GJB1 gene. Dr. Rudolph Martini at the University of Würzburg, Germany, has found that inhibiting the macrophages associated with inflammation has a very positive effect in a mouse model of CMT1X, which is caused by mutation of the GJB1 gene. Based on his studies, we are developing approaches to inhibit macrophages as a clinical treatment. In addition, the work of Dr. Kleopas Kleopa at the Cyprus Institute of Neurology and Genetics has shown the first example of a successful gene therapy in a CMT1X mouse model, and he is continuing these studies to optimize this novel type of therapy for not only CMT1X but also CMT4.

CMT2A is caused by dominant mutations in Mitofusin 2 (MFN2). The STAR team has developed two good rat models for CMT2A, which are being made available to the research community and represent an important tool to test potential new modulators of mitofusin activity. Stem cell models of CMT2A have also been developed for CMTA-sponsored research in the laboratory of Dr. Robert Baloh, Cedars-Sinai Medical Center.

CMT2E is caused by dominant mutations in the neurofilament light protein (NEFL) gene. Mutations in NEFL cause CMT2E, but other mutations in the same gene are associated with ALS, suggesting there may be a connection between them. One of the best mouse models of CMT2E, made by Dr. Ron Liem at Columbia University, has been extensively characterized by the CMTA and now represents an important model for therapeutics testing. Stem cells containing CMT2E mutations have been differentiated into motor neurons and used to create a test for therapies that prevent aggregations of neurofilaments seen in CMT2E.

CMT4 is caused when both versions of an important gene required for healthy myelin (SH3TC2) are deficient. To restore function of these genes, the gene therapy approach described above for CMT1X is also being tried for CMT4C by Dr. Kleopa. If successful, these studies may highlight a novel approach that will be applicable to other forms of CMT4.
The STAR Advisory Board

The CMTA's STAR Advisory Board comprises a Scientific Advisory Board, a Therapy Expert Board and a Clinical Expert Board.

The Scientific Expert Board provides scientific input for projects that are ongoing or proposed.

The members are:

John Svaner, PhD, Chair, University of Wisconsin
Frank Baas MD, PhD, University of Amsterdam, The Netherlands
Robert H. Baloh, MD, PhD, Cedars-Sina Medical Center, LA
M. Laura Feltri, MD, University at Buffalo
Gabsang Lee, PhD, Johns Hopkins University, Baltimore
Jun Li, MD, PhD, Vanderbilt University, Nashville
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 Poppko, MD, University of Chicago
Mario Saporta, MD, PhD, University of Miami
Steven S. Scherer, MD, PhD, University of Pennsylvania
University at Buffalo
Lawrence Wyszetz, 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
David Herrmann, MD
University of Rochester (New York)
Christopher Klein, MD
Mayo Clinic, Rochester, Minnesota
Lars J. Knutsen, PhD
Discovery Pharma Consulting LLC
Claes Wahlestedt, MD, PhD
University of Miami
Tage Honore, PhD
Aestus Therapeutics Inc.

The Clinical Expert Board (CEB) provides expert guidance and support to the CMTA’s alliance partners, helping to assure the success of clinical trials.

The members are:

Michael E. Shy, MD, CEB Chair, University of Iowa
Mary Reilly, MD, CEB Co-Chair, National Hospital London, England
Joshua Burns, PhD, University of Sydney, Australia
Richard Finkel, MD, Nemours Children's Hospital Orlando, Florida
David Herrmann, MD
University of Rochester (New York)
Christopher Klein, MD
Mayo Clinic, Rochester, Minnesota
Michael McDermott, PhD, Consultant
University of Rochester (New York)

The New York Stem Cell Foundation (NYSCF) Research Institute makes human stem cell lines that represent the genetic disease defect for a collection of CMT disorders. These cell lines were derived from patients with different CMT disorders and banked in a repository at the University of Iowa, then re-programmed by the NYSCF into induced pluripotent stem cells (iPSCs).

At the National Institutes of Health, the CMTA has close working relationships with the National Center for Advancing Translational Sciences (NCATS), the National Institute of Neurological Disorders and Stroke (NINDS), and the NIH Office of Technology Transfer. This has led to sponsorship of several joint projects that aim to support CMTA alliance efforts.

The Jackson Laboratory is an independent, nonprofit organization focused on mammalian genetics research, and is currently working with the CMTA to breed, cryopreserve and distribute rodent models of CMT that are used in the STAR alliance network.

PsychoGenics provides biomarker and behavioral testing support to the STAR network, working closely with the CMTA to design and execute preclinical drug testing studies in CMT animal models, and to characterize animal models destined for use in CMTA research efforts.

Horizon Discovery is a translational genomics company that develops and supplies patient-relevant drug discovery and diagnostic research tools, and has worked closely with STAR investigators to design and create several new rodent models of CMT, which are now entering into research and therapeutics discovery projects sponsored by the CMTA.

Charles River, a global provider of contract animal research services, is working with the CMTA to breed, cryopreserve and distribute rodent models of CMT that are used in the STAR alliance network.

The Jackson Laboratory is an independent, nonprofit organization focused on mammalian genetics research, and is currently working with the CMTA to breed and distribute specialized mouse models of CMT in support of its research project efforts.

PsychoGenics provides biomarker and behavioral testing support to the STAR network, working closely with the CMTA to design and execute preclinical drug testing studies in CMT animal models, and to characterize animal models destined for use in CMTA research efforts.

Renovo Neural Inc., a specialized preclinical research organization offering expert histology and electron microscopy services to the CMTA, for use in both the characterization of new animal models of CMT, and the evaluation of new therapeutics in support of its pharmaceutical alliances.

ARQ Genetics provides real-time polymerase chain reaction services for client sample analysis, supporting CMTA efforts to analyze gene expression in animal disease models of CMT, and measure changes in molecular markers as predictors of response to potential therapeutics.
More than 70 branches in the United States and Canada provide help and hope to members who meet regularly to share their personal experiences and hear from leading clinicians and scientists provided as guest speakers by the CMTA. The branches are also the focal points for grassroots fund- and awareness-raising efforts.

In March 2016, the CMTA hosted its branch leaders at a Leader Conference in Tampa, Florida, providing workshops, training, and research updates during a three-day conference designed to educate and inspire them for the year ahead. The effort paid off in spades: All in, CMTA branches contributed a record $812,895 to the CMTA and STAR in 2016, up from $624,423 in 2015.

During Awareness Month 2016, branches held a total of 22 Walks, Cycles and Swims 4 CMT. More than 1,500 people nationwide came out to support and participate in these incredible events, raising $150,600 in September alone.

The Southeast Wisconsin Branch's 2nd Annual Walk 4 CMT was one of those events. More than 125 people gathered at the Pettit National Ice Center where they walked on an indoor track surrounding the ice rink and then gathered for food, great music and a silent auction, raising $18,157 for CMT research.

In October 2016, friends and family of the late Julianna Snow held a "Unicorn Walk 4 CMT," in memory of the 5-year-old girl with CMT who chose to die at home rather than go back to the hospital one more time. Dedicated to all those people with CMT who don’t feel like they fit in, the event raised $6,629.

Not all fundraisers were walks. Lily Sander, then 8, raised $19,684 to help fight her own disease by competing in the grueling Spartan mud race in Charlotte, North Carolina, traversing obstacles like creeks and mud pits on a bitterly cold April day.

The Harrisburg Branch surpassed the $5,000 goal for its first-ever fundraiser in 2016, raising $6,217 with a kids' fishing tournament featuring lots of great auction items and delicious barbecue.

Two Patient/Family Conferences in 2016

In 2016, the CMTA hosted two Patient/Family conferences—one in State College, Pennsylvania and the other in Houston. These conferences allow people with CMT and their families to get up-to-date information on 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.
Online community

The CMTA also provides education and support through a vibrant online community. Our Facebook community has grown to more than 27,000 followers, who take part in a constant stream of conversation, posting hundreds of comments daily. In addition, the CMTA has more than 2,400 Twitter followers; more than 1,700 Pinterest followers; and more than 500 LinkedIn professionals.

In an online Awareness Month campaign in September 2016, we created a fun #1in2500 Twibbon for people to upload to Facebook. The response was phenomenal. More than 2,300 people uploaded the Twibbon to create awareness on their social media pages!

Proving the power of the Internet as a fundraising tool, the CMTA's online community raised $40,000 for STAR research on Giving Tuesday (the Tuesday after Thanksgiving), meeting a $10,000 challenge grant many times over.

OUR ONLINE COMMUNITY

The CMTA is governed by a voluntary Board of Directors whose members 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, they are deeply committed to the organization and annually make significant financial contributions to ensure that it meets its goals.

Gilles Bouchard (Chairman)
Former Chairman and CEO
Livescribe, Los Altos, CA

Gary Gasper (Treasurer)
Partner, Ernst & Young
Ernst & Young, Washington, D.C.

Herb Beron (Secretary)
Financial Advisor
Perth Amboy, NJ

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

Thomas W. Dubensky Jr., PhD
Chief Scientific Officer
Aduro Bio Tech
Berkeley, CA

Laura Fava
Owner/Lawyer, Laura Fava Barrister & Solicitor, Toronto, Ontario

Alan Korowitz
Executive 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

Elizabeth Ouimet
Civic Volunteer and Community Relations Expert, Los Altos, CA

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

Lawrence Wrailes, MD
Professor of Neurology and Biochemistry Director
Kety Research Institute
University at Buffalo
Buffalo, NY

Special Advisors to the Board
Bruce Chizen
Former CEO, Adobe Systems

Patrick Livney
Former CEO, CMTA

Seventh Annual New York Cocktail Reception
Board members Phyllis Sanders and Alan Korowitz held the Seventh Annual New York CMTA Cocktail Reception at the glamorous Essex House in October. A buffet dinner, music and an auction with items such as concert tickets, sporting event tickets, trips, jewelry and memorabilia marked the evening, which raised $254,021 for the CMTA.

Third Annual Cycle 4 CMT
More than 150 people attended the 3rd Annual Cycle 4 CMT in Charlotte, Vermont, on Sunday, August 28. With more than 500 donors, the event brought in $118,618, which will be dedicated to the CMTA’s STAR drug development program.

Oxford Biathlon
Board member Steve O'Donnell teamed up with Branch Leader Clark Emmens for the Third Annual Oxford Biathlon on June 12, 2016, in tiny Oxford, Maryland. Under a cloudless blue sky, more than 100 participants ran, walked, swam, and cycled for CMT, raising $115,291 in the process.

Team Julia
In September 2016, Board member Herb Beron, his wife Rachael and other members of Team Julia ’16 swim across Lake Valhalla in Montville, New Jersey, to raise funds for STAR. The swim raised $75,267 in its eleventh year.

BOARD OF DIRECTORS

BOARD FUNDRAISING EFFORTS AND EVENTS
Members of the CMTA Advisory Board bring both expertise and empathy to their positions, assisting people with CMT in dealing with some of the critical issues facing them, from finding suitable orthotics to the best balance exercises to tips on how to talk about CMT. They also write articles for *The CMTA Report*, participate in monthly webinars and assist CMTA members who have questions in areas in which they would be considered experts.

The Advisory Board welcomed two new members in 2016: community activist and Baltimore Branch Leader Clark Semmes and consultant and writer Tom Meloche.

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**CMTA’s corporate partners each offers products or services that can improve the life of 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.
- **Foot Solutions** is a leading international footwear retailer with more than 150 stores in 14 countries specializing in personalized assessments, stylish high performance footwear, custom-fitted, custom-crafted arch supports and accessories designed to help individuals live pain free.
- **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.
- **Kinetic Research** is an orthotic/prosthetic technology company that specializes in the application of carbon fiber and advanced materials into highly functional orthopedic appliances for the lower leg.
- **Union & Fifth** raises money for non-profits by selling donated, gently worn women’s designer clothes.
CONSOLIDATED STATEMENT OF FINANCIAL POSITION

DECEMBER 31, 2016

Revenues

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<tr>
<th>Revenues</th>
<th>December 31, 2016</th>
<th>December 31, 2015</th>
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<tr>
<td>Contributions</td>
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<td>Special Events</td>
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<td>Support Group</td>
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<td>Investment Income</td>
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<td>Grants</td>
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<td>Dedicated Research Revenue</td>
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<td>Total Revenues</td>
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Expenses

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<td>Program Services</td>
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<tr>
<td>Management and General</td>
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<td>Fundraising</td>
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Program Expenses

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<td>Research</td>
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<td>Branches</td>
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<td>Education &amp; Awareness</td>
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<tr>
<td>Total Expenses</td>
<td>$2,849,052</td>
<td>$3,299,104</td>
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