



a year of hope & promise



2012 ANNUAL REPORT



a committed team

success can only be achieved by passionate, dedicated individuals

It has been my honor and privilege to serve as the CEO of the CMTA. The women I work alongside (Jeana, Susan, Bethany, Kim, Pat, and Elizabeth), day in and day out, work tirelessly to educate, engage, and energize the very people that will be essential for making the first treatment of CMT a reality....you. Our world-class scientific team, led by Drs. Shy, Scherer, and Svaren, continues to push the translational research, which is producing some early promising results. Our accomplishments are nothing short of amazing...

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

a message to our community...

- * Despite a distracting election year, the CMTA surpassed 2011's record year in fundraising!
- * STAR (Strategy to Accelerate Research) has identified compounds that down regulate PMP22 both in vitro and in vivo!
- * Support and Action Groups have grown to 53 in 40 States, with Canada and Mexico now aboard!

The CMTA is funding a record amount of research, with a focus on types 1A,2A, 2E and X.

To complement the ongoing research, the CMTA has added Dr. Lawrence Wrabetz, University of Buffalo, a world-renowned expert on CMT, and Dr. Mark Scheideler, Founder, HumanFirst Therapeutics, to the STAR team. Mark will help guide the CMTA's drug development path forward, helping to forge the necessary partnerships with the pharmaceutical industry in 2013!

Most importantly, the STAR process, namely replicating the various types of CMT in cellular assays suitable for High Throughput Screening of compounds, followed by animal model confirmation, ultimately leading to an approved treatment drug, has been proven valid by the Cystic Fibrosis Foundation. Their first FDA approved drug for treatment of a devastating genetic disease went on the market this year, and was discovered using a similar process to the one that the CMTA calls STAR!

I'd like to close by imploring everyone to reach out to all the contacts they have. Talk about CMT! Ask them to make a difference! Spread the word! Contribute to a movement underway that absolutely will be successful and will prove so meaningful to CMT patients around the world!

I hope you are pleased with the progress of the CMTA. I look forward to sharing with you our success in 2013.

Best Regards,
Patrick A Livney, Chief Executive Officer
Charcot-Marie-Tooth Association



2013 goals

The mission of the CMTA 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. In 2013, the CMTA will reach the following milestones:

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

Raise \$25M for CMT Pathways: the capital campaign to fund the first treatments for CMT.

Provide funding to finish testing laboratory models for 1A and move into pre-clinical investigation.

Provide funding to build a viable cellular assay and laboratory model for types 2A and 2E.

To improve the quality of life for people with CMT:

Grow the Support and Action Groups to 70 across the United States.

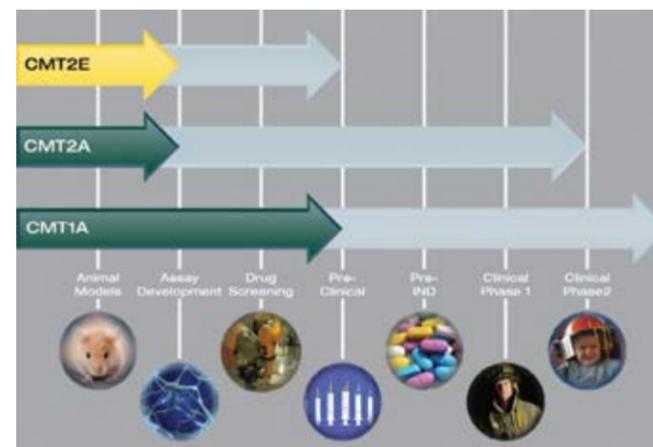
Expand the awareness and education about CMT to the community.

Expand education about CMT to the clinician community.

Develop additional partnerships to better serve the patient community.



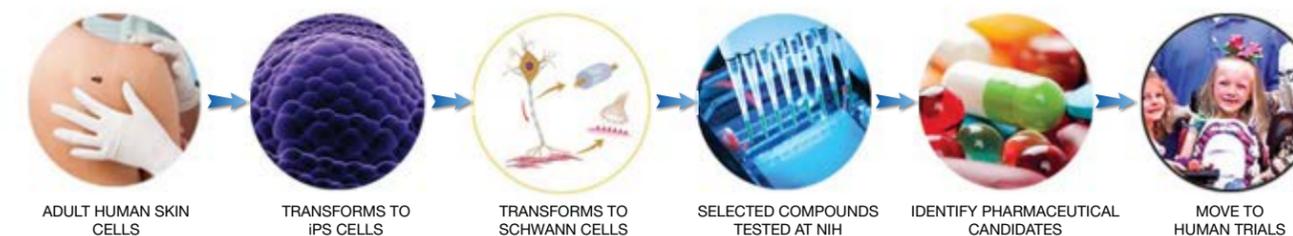
7 STAGES OF DRUG DEVELOPMENT



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



the STAR's project is shining brightly...



The Time Truly is Now!

CMT1A - The laboratories at NIH/NCATS, The University of Gottingen, Germany, and the University of Wisconsin have developed new cell-based screening assays, which can identify drugs that can reduce levels of PMP22, the protein which causes the myelin to degenerate, leading to impaired nerve function. These new screens have been used to screen several drug collections, including FDA-approved compounds and specialty collections from pharmaceutical partners. Preclinical studies were initiated with the most promising compounds to date, using an excellent laboratory model of CMT1A developed by STAR partners in Germany. Initial contacts have been made with several drug companies that are partnering with the CMTA to develop new therapies for CMT1A and other forms of CMT.

Transformation Project - Dr. John Svaren and his team at the Waisman Center at the University of Wisconsin developed human stem cell (iPS cell)-derived Schwann cells that have high levels of PMP22, and these will be used as an independent test of drug efficacy. This technology introduces human cells for screening at the cellular level, assisting in the pathway toward clinical trials.

CMT2A - Dr. David Chan at Caltech is working with Dr. Inglese and his group at The National Institutes of Health /NCATS to develop new drug screening assays that are based on Dr. Chan's exciting findings that expression of Mitofusin 1 can compensate for the mutations in the Mitofusin 2 gene that cause CMT2A. Dr. Stephan Zuchner at the University of Miami has developed a laboratory model of CMT2A and has reported results of his studies. He has found neurological deficits, and is working further to see if this can be used to detect any improvement in response to candidate compounds. In a parallel approach suggested by Dr. Chan, the CMTA has also partnered with a company that creates laboratory models of various genetic disorders. They have now initiated work to create a lab version of CMT2A, and the first few milestones have already been achieved.

CMTX - A novel stem cell trial is being directed by Dr. Rudolf Martini, in which human stem cells are introduced into an excellent laboratory model of CMTX in order to reduce the immune activation that accompanies this type of CMT. The stem cells have been injected and the results of this trial will be announced in 2013.

CMT 2E - Dr. Ron Liem at Columbia University is building a 2E cell line to replicate the abnormality which causes axonal damage, thereby producing the neuropathy of CMT2E. Having cells that replicate the disease will enable Dr. Liem and the CMTA team to find molecules that can block the formation of the abnormalities, thus preventing nerve damage. This will take place in collaboration with Dr. Inglese and his state-of-the-art drug screening facility at the National Institutes of Health /NCATS.



support & action group news

The Support and Action Group network is growing every year.

In 2012 we started 7 new groups, with three groups in Canada (British Columbia, Ontario and Quebec) and one in Mexico City.

Support and Action Group Facilitator Conference

The Support and Action Group Facilitator Conference was held in Chicago, November 8-11, including 75 Support and Action Group Facilitators, Drs. Mike Shy, Steve Scherer and John Svaren, most of the Advisory Board and Board Members Elizabeth Ouellette, Herb Beron and CEO Pat Livney. The weekend was spent motivating, educating and inspiring the facilitators to reach their communities to empower them to reach the goal of a world without CMT.

Chicagoland Patient Family Conference

Over 250 people sold out the Chicagoland Patient Family Conference November 10, 2012 in Elk Grove Village, IL. Attendees heard from Drs. Shy, Scherer and Svaren about STAR, Certified Orthotist Sean McKale about orthotic management for CMT, and Dr. Gregory Carter about maximizing quality of life in CMT. The morning concluded with "Lunch with the Experts" where attendees had the opportunity to talk with members of the Board of Directors, the Advisory Board and the Medical Advisory Board.

2012 fundraising efforts...

BOARD EVENTS

Friars club

Alan Korowitz and Phyllis Sanders organized the Third CMTA Update at New York City's famed Friar's Club on June 4th. Over 150 guests attended and over \$146,000 was raised.

San Francisco Giants Fantasy Baseball Camp

Vasi Vangelos attended a fantasy baseball camp in honor of the CMTA. His fundraising efforts raised \$35,000 in 2012

TeamJulia and Swim for the Cure

On Labor Day weekend, Chairman of the Board Herb Beron and Board member Steve O'Donnell hosted the 6th Annual TeamJulia Swim for the Cure at Lake Valhalla Club in Montville, NJ. The event included over 30 swimmers and raised \$136,300.

SUPPORT AND ACTION GROUP EVENTS RAISED OVER \$156,000

Somerset Patriots

Saturday August 11th the Atlantic League Professional Baseball's Somerset, NJ Patriots welcomed members from the Morris County, NJ and Central, NJ Support and Action Groups to raise CMT awareness and almost \$15,000 for the CMTA.

Reach for the STARs - Walk and Roll for the CMTA

Dale Lopez and the Chicago Area CMT Support and Action Group sponsored their third annual walk and roll Saturday, September 8th. Over 100 people participated in the mile walk raising over \$18,000.

Canadian Fundraiser

Kelly Hall and the Southern Ontario CMT Support and Action Group held the 2nd Annual Walk for CMT Fergus Fundraiser on September 30th at the Victoria Park in Fergus, Ontario and it was a huge success! There were 80 participants the day of the event and they raised over \$5,600!

Stepping it UP

Every year Jeana Sweeney organizes a CMT awareness and education event for school children which also raises funds for the CMTA. In 2012, Jeana held 2 Stepping it UP events, reaching over 1,100 children and raising over \$12,000.



advisory board

Advisory Board

The CMTA welcomed 2 new members of the CMTA's Advisory Board: Dr. Elizabeth Misener and Dr. James Nussbaum. Elizabeth K. Misener, PhD, LMSW, is a licensed Master Social Worker with New York State. She provides coaching on a wide range of issues such as depression, stress management, anxiety, life-work balance and relationships. As a trained social worker, she supports individuals on their journey of self-discovery through self-reflection and goal setting.

James Nussbaum, PT, PhD, SCS, EMT, is the Clinical and Research Director of ProHealth & Fitness PT OT, a physical and occupational therapy organization in New York. James has a particular interest in finding ways to enhance and document patient improvements in ADLs, mobility, balance, function, and most importantly, quality of life.

The CMTA's Advisory Board now has 15 members including Jonah Berger, Dr. Gregory Carter, Dr. Katy Eichinger, Tim Estilow, Shawna Feely, Sean McKale, Bethany Meloche, David Misener, Michael Needleman, Susan Salzberg, Carly Siskind, Jason Steinbaum and David Tannenbaum.



social initiatives...

CMTAthletes Tri-athlete 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. The program also can design unique sports kits for any sport.

CMT Forum is an online chat room on the CMTA website where those living with CMT can post questions and answers, challenges and successes on a variety of topics.

Increased reach through website & social media • Website was visited 334,796 times

Increased community membership 44% and premium membership 18%

Facebook grew from 1,820 to 6,604 fans • Brought reach to Twitter and LinkedIn

Awareness Month

September is CMT Awareness Month. This year the CMTA reached over 173,000 people through Awareness Month messages. Our YouTube channel was viewed 13,000 times, attracted 500 new Facebook fans and raised \$1,200.

...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 Allard distributed 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 provides physical therapy, industrial rehabilitation and orthotics and prosthetics services to thousands of patients across the United States. The partnership has focused on educating clinicians about CMT and connecting them to those with CMT across the US.



CONSOLIDATED STATEMENT OF FINANCIAL POSITION DECEMBER 31, 2012



Thank you to our donors!

We are an organization primarily sustained by our individual donors. Without you, the CMTA would not be able to accomplish the major milestones in research, program services and awareness.

Thank you for your contribution!

Revenues	Unrestricted	Temporarily Restricted	Total 2012	December 31, 2011
Contributions	\$847,651		\$847,651	\$1,137,223
Special events revenue (net of expenses)	\$626,558		\$626,558	\$335,777
Sale of Promotional Products (net of expenses)	(\$3,090)		(\$3,090)	\$0
Support group revenue	\$176,833		\$176,833	\$141,742
Investment income	\$1,778		\$1,778	\$1,290
Grants received	\$49,000		\$49,000	\$0
Release restrictions	\$75,000	(\$75,000)		
Total Revenues	\$1,773,730	(\$75,000)	\$1,698,730	\$1,616,032
Expenses				
Program services	\$1,028,983		\$1,028,983	\$558,671
Management and general	\$221,036		\$221,036	\$518,080
Fundraising	\$195,980		\$195,980	\$99,685
Total Expenses	\$1,446,000		\$1,446,000	\$1,176,436
Increase (decrease) in net assets	\$327,730	(\$75,000)	\$252,730	\$439,596
Net assets – beginning of year	\$765,612	\$75,000	\$840,612	\$410,016
Net assets – end of year	\$1,093,342		\$1,093,342	\$840,612



2012 successes...

CMTA

Board of Directors

Herb Beron, Chairman
Steven Blevitt
Gary Gasper
Alan Korowitz
Steve O'Donnell
Elizabeth Ouellette
Phyllis Sanders
Dr. Steve Scherer
Dr. Michael Shy
Dr. John Svaren
Vasi Vangelos
Dr. Larry Wrabetz

STAR - Scientific Advisory Board

Dr. Michael Shy, Chairman,
University of Iowa
Dr. Steven Scherer,
Co-Chairman, University of
Pennsylvania
Dr. Laura Feltri,
SUNY Buffalo
Dr. Kristjan Jessen,
University College, London
Dr. Rhona Mirsky,
University College, London
Dr. Brian Popko,
University of Chicago
Dr. Ueli Suter,
ETH Institute, Zurich
Dr. Vincent Timmerman,
University of Antwerp
Dr. Larry Wrabetz,
SUNY Buffalo

CMTA Staff

Patrick Livney, CEO,
Patricia Dreibelbis,
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

Increased community membership 44% in 12 months
- as of 12/31/12 the total number of memberships was 5,114

Increased premium memberships 18% in 12 months
- as of 12/31/12 the total number of premium memberships was 876

Web traffic

- total visits – 334,796
- Average visits per month – 27,899
- Average unique visits per month – 16,636
- 60% new visitors

Grew Facebook fans from 1,820 to 6,604

Grew Support and Action Groups to 57 in 32 states,
including 3 in Canada and 1 in Mexico

Grew revenues 9.1%

From \$1,703,238 in 2011 to \$1,858,435 in 2012

**INCREASED DONATIONS TO INSTITUTIONS VIA
RESEARCH GRANTS 731% FROM \$64,380 IN 2011 TO \$471,261 IN 2012**



Mailing Address: CMTA P.O. Box 105 Glenolden, PA 19036
Phone Number: 1-800-606-2682 • Website: www.cmtausa.org

Social media links:

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