

# Charcot-Marie-Tooth Association

Annual Report  
2011

# Thanks to Our Donors ...



As the new CEO of the CMTA, I am excited to report the good news of our year. A world without CMT – that is the vision of the CMTA and the driving force behind our everyday operations. This year we realigned the management team to meet the goals of the CMTA: to develop the research to find a treatment for CMT, to provide resources to those affected by CMT, and to create awareness about CMT.

Our Support and Action Groups provide a forum for clinicians who treat CMT to speak to those affected as well as providing a place where families can come to talk about challenges and successes while living with CMT. Through the work of Jeana Sweeney, Director of Community Services, we have grown the Support and Action Group network to 50 groups in 40 states with the membership approaching 10,000.

When Herb Beron was elected as the new Chairman of the Board, his focus was to build an advisory board to encompass the various disciplines that address the needs of the CMT community. Through his and Board member Elizabeth Ouellette's efforts, the CMTA can proudly claim 14 dedicated professionals serving on the Advisory Board. In addition, the CMTA welcomed two new members to the Board of Directors; Steve Blevitt, a partner at Sidley Austin, brings his legal expertise, and Dr. John Svaren from The Waisman Center at The University of Wisconsin brings great insight to the investigative work being conducted worldwide under STAR.

In September, we launched the first annual CMT Awareness Month with efforts nationwide to generate buzz about CMT. With the help of CMTA Board member Alan Korowitz and the generosity of Clear Channel, we distributed radio announcements nationwide on Clear Channel stations, increased our website visits to over 72,000, and raised \$200,000 for our STAR initiative.

After reviewing our financial performance, you will notice that we raised a record amount of revenue with 65 percent going directly to programming and research. Regarding research, you will see on the pages summarizing our STAR initiative that the CMTA has made significant progress in realizing our mission of finding a first treatment for CMT. STAR researchers have successfully screened over 250,000 pharmaceutical compounds utilizing state of the art CMT-1A cellular assays and have identified several potentially viable candidates. We have also expanded our STAR initiative to include research protocols for types 2 and X.

The progress we have made as an organization over the past 12 months could not have been accomplished without the support of our many generous donors. As we continue to expand in 2012, we will create more partnerships to better serve the needs of those with CMT, bring more resources to patients and clinicians, and make more progress on STAR, our passionate journey towards a first treatment for CMT! Thank you for your continued support.

– Patrick A. Livney, CEO

# A World Without CMT



A World Without CMT:  
That's the CMTA's vision,  
and the driving force  
behind our mission.



We are devoted to:

- ☆ Generating resources to find treatments and a cure for CMT
- ☆ Creating awareness about CMT
- ☆ Improving the quality of life for those affected by CMT

# Meeting Our Mission

## Strategy To Accelerate Research - STAR



NCATS researcher Sung-Wook Jang stands next to the high-throughput screening robotic system used to identify potential treatments for Charcot-Marie-Tooth disease.

CMTA-funded initiatives have lead to:

- ☆ Identification of key parts of the PMP22 gene that cause it to be expressed highly in CMT1A
- ☆ Creation of drug screening assays that have been used at one of the premiere drug screening facilities in the world at the National Institutes of Health
- ☆ Identification of several FDA-approved compounds that lower PMP22 levels in cell-based assays
- ☆ Preparation for trials in a well-established rat model of CMT1A (ongoing in 2012)
- ☆ Development of partnerships with pharmaceutical companies to perform screening on their compound libraries
- ☆ The first NIH funding of further development of drug screening assays at the Svaren laboratory of The University of Wisconsin
- ☆ Completion of initial library screens using new assays

# STAR Corporate Partners



Through establishing strategic partnerships, the CMTA has been able to accelerate the pace of research, explore additional approaches to research and provide education and resources for patients.

## ☆ GlaxoSmithKline

- contributed a library of kinase inhibitors to help understand and address unmet medical needs

## ☆ Pfizer

- donated a subset of their Chemogenomic library to test at the NIH

## ☆ Sangamo Biosciences

- assisted in designing and introducing reporters in PMP 22 for future screens

# Meeting Our Mission



**Improving the quality of life  
for those affected by CMT**

## CMT Support and Action Groups

- ☆ Expanded our network to 50 groups in 40 states with almost 10,000 participants nationwide
- ☆ Invited specialists from varying disciplines to educate attendees about CMT
- ☆ Collectively raised over \$211,000



Orthotists Ken Cornell, CPO, and David Misener, CPO, at the Albany, NY Support and Action Group



# Meeting Our Mission

## Improving the quality of life for those affected by CMT

- ☆ Hosted a Patient & Family Conference with over 200 attendees
- ☆ Developed an Advisory Board comprised of dedicated professionals
- ☆ Created a new website which serves as the primary source of information for those affected by CMT
- ☆ Developed first on-line clinician training specific to CMT
- ☆ Expanded the Centers of Excellence program worldwide
- ☆ Developed a relationship with Physiotherapy Associates to identify and train over 70 physical therapists nationwide about CMT



# Meeting Our Mission



## Creating Awareness About CMT



Member Jerry Cross from the Nevada Support and Action Group at an informational display during Awareness Month

- ☆ Awareness Week became Awareness Month
- ☆ Received 6 state & multiple city proclamations
- ☆ 80 countries participated
- ☆ Increased visits to the Awareness Month microsite by 2000 percent
- ☆ Added 451 new Facebook fans
- ☆ 30,000 videos viewed
- ☆ Clear Channel Radio broadcast Shadoe Stevens' CMT Public Service Announcement

# Member Testimonials



“To see and meet so many other people living with and acknowledging CMT was incredible. And what I heard was hopeful and inspirational -- it's amazing how people have learned to cope/deal with this disease. And also amazing to hear about the ongoing research in this field.” – Shelley, MD

“It is so refreshing to go somewhere that the others there are like me! The guest speakers have given us so much information that will help enrich our lives, solve some of our challenges, and presented us with solutions for problems that we may or may not have now and in the future. I have gained so much from just knowing others that are going through the same or similar struggles. It is truly a wonderful service the support groups give us.”

– Janice, GA

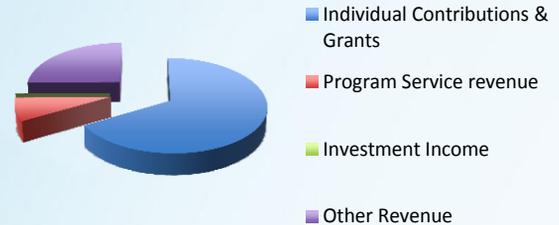
“The CMTA helped me find a physical therapist who knew CMT and treated me appropriately.” – James, GA

# Consolidated Statement of Financial Position

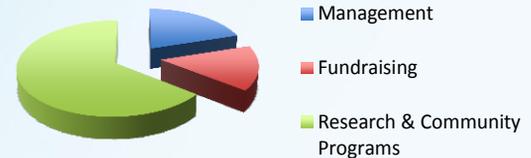


<b>Income</b>	
Contributions & Grants	\$1,172,843
Program Service revenue	\$141,742
Investment Income	\$1,290
Other revenue	\$335,777
<b>TOTAL INCOME</b>	<b>\$1,651,652</b>
<b>Expenses</b>	
Research & Community Programs	\$781,874
Management	\$243,503
Fundraising	\$186,679
<b>TOTAL EXPENSES</b>	<b>\$1,212,056</b>
<b>TOTAL Assets</b>	<b>\$865,547</b>
<b>TOTAL Liabilities</b>	<b>\$24,935</b>
<b>NET ASSETS</b>	<b>\$840,612</b>

## Income



## Expenses



# Thank You to Our Donors!

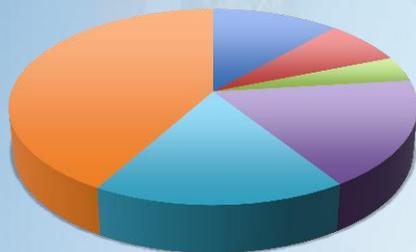


We are so grateful for your support which has allowed the CMTA to have such a successful year.

Thanks to our donors, the CMTA's revenues reached a record \$1,651,652.

Thank you!

## Total Amount Raised by Giving Levels



- \$1-\$99
- \$100-\$499
- \$500-\$999
- \$1000 - \$9999
- \$10,000 - \$99,999
- \$100,000 and above

Gift levels	Number of Gifts
\$1- \$99	4013
\$100 - \$499	1238
\$500 - \$999	125
\$1000 - \$9999	138
\$10,000 - \$99,999	14
\$100,000 and above	4

**Thank you for your support!  
We look forward to celebrating  
a successful 2012 with you.**



## CMTA Board of Directors

Herb Beron, Chairman, NJ  
Steve Blevitt, CA  
Gary Gasper, DC  
Alan Korowitz, NY  
Steve O'Donnell, MD  
Elizabeth Ouellette, CA  
Phyllis Sanders, NY  
Dr. Steven Scherer, PA  
Dr. Michael Shy, IA  
Dr. John Svaren, WI  
Vasi Vangelos, CA

## 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, Chief Executive Officer  
Patricia Dreibelbis, Director of Program Services  
Kim Magee, Director of Finance  
Susan Ruediger, Director of Development  
Jeana Sweeney, Director of Community Services



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