CMTA
Charcot-Marie-Tooth Association

A year of growth and collaboration

2014 Annual Report

CHARCOT-MARIE- TOOTH ASSOCIATION

> *The great coach Vince Lombardi said that individual commitment to a group effort is what makes a team work. I count myself lucky to lead a team of co-workers, volunteers and researchers whose commitment to a world without CMT is deep and unshakeable.*

My co-workers' individual commitments to our group effort are driven by the fact that everyone who works for the CMTA, myself included, either has CMT or has a child, spouse, or close friend with the disease.

My co-workers' passion drove them to work tirelessly in 2014 to raise funds for research and to improve the lives of all those who have CMT. They also provided education, tools and support to our ever-growing community through our Support and Action Groups, the main point of contact with our members. They did such a good job of it in 2014 that the CMTA earned Charity Navigator's Four-Star rating, demonstrating their commitment to ethics, accountability and transparency.

Our passionate, committed team

The CMTA's Support and Action Groups demonstrated the same sense of commitment to our goal in 2014. To give just one example, in 2013 the Support and Action Groups raised close to $375,000 by organizing events like walks, rides, swims and dinners. In 2014, the groups raised an astonishing $302,930 during a single month—Awareness Month in September. That figure helped push our total 2014 revenues above $3 million for the first time, only a year after touching the $2 million mark for the first time.

The majority of those monies went directly to our expert team of researchers, who now span the globe. They are a dedicated group and share our commitment to finding a treatment, and ultimately a cure, for CMT. Many have devoted their entire professional lives to unraveling its mysteries. By funding the top researchers in the field—and not reining them in with the typical proposal and publish requirements imposed on scientists—we've been able to push the research forward to the point where actual tangible results are becoming evident, as detailed in the coming pages.

The CMTA also grew through collaboration in 2014. In September, the CMTA announced an alliance with Genzyme, a Sanofi company, to discover therapies for CMT1A using the company's high-throughput screening facility in Tucson, Arizona. In October, the CMTA announced a partnership with Addex Therapeutics for evaluating the pharmacology of ADX71441 in a battery of preclinical models of CMT1A. In December, the CMTA formed a broad collaboration with the New York Stem Cell Foundation (NYSCF) to develop cellular models of CMT for individual patients.

Our online community also expanded in 2014: The number of Facebook fans grew from 12,000 to 20,000; we created a CMTA Facebook group for Spanish speakers; and started a CMTA LinkedIn page to raise awareness in the corporate world.

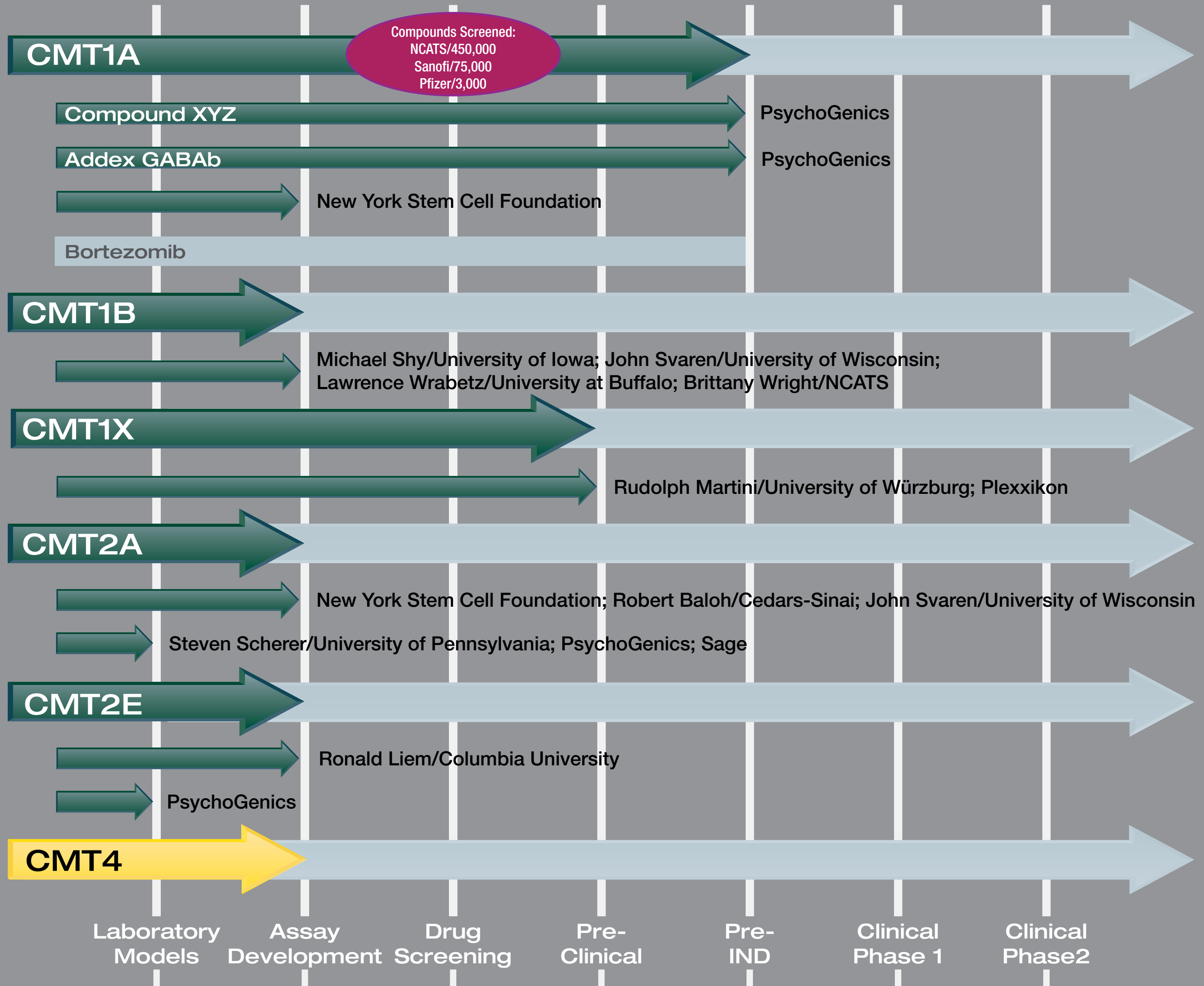
On a personal note, I would be remiss in not mentioning one member of the team who is no longer with us. For almost a quarter of a century, Pat Dreibelbis was the voice of the CMTA and the first point of contact for anyone who called the organization. Her knowledge of CMT was second to none and her commitment to our goals unmatched. We will miss her for a very long time, but will always feel her presence.

To sum up, the entire team is working hard and their efforts are paying off. I urge you to join us in fighting for those with CMT. Tell your story, share information via social media about the CMTA, connect locally and nationally with people on the team. Continue to support our search for treatments and our efforts to improve the quality of life for everyone with CMT. Your commitment to our joint effort is what will make it work.

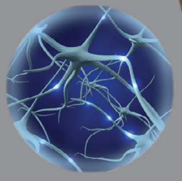
Patrick A. Livney
Chief Executive Officer

The Strategy to Accelerate Research (STAR)

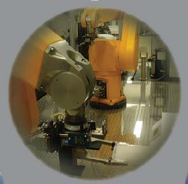
The CMTA is aggressively fighting to find a pharmaceutical treatment, and, ultimately, a cure for all types of CMT. We currently have protocols for developing treatments for the types of CMT that affect approximately 90 percent of all people with CMT. The chart below shows the status of each of those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



Screen the cellular assays using the chemical libraries at the National Institutes of Health and major pharmaceutical companies.

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

Test the compounds for dosage, toxicity and viability in private and academic laboratories to determine the exact dosage that will improve the root cause of CMT.

Conduct pre-clinical phase 1 human trials. A small group of strong, healthy people without CMT will take the dose of the compounds to test for human side effects and toxicity.

Conduct pre-clinical phase 2 trials. Recruited through the CMT Centers of Excellence, small groups of people with type-specific CMT will participate in pharmaceutical trials to determine the effect the drug will have on CMT.

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 2015 include:

- ★ Build human cell lines for 1A, 1B, 2A, 2E, X, and 4 to expedite drug discovery.
- ★ Succeed in building assays for CMT1B to be used to screen potential drugs
- ★ Begin the STAR strategy for type 4
- ★ Share our resources with an additional 10,000 people worldwide
- ★ Host national Patient and Family Conferences in Boston and Nashville
- ★ Grow our reach to 100 CMTA Branches in North America
- ★ Grow the CMTA's Clinical Centers of Excellence worldwide to 19 from the current 17
- ★ Host 10 public webinars with CMT experts
- ★ Expand our outreach to Latin America, where CMT is prevalent

THE STARS ARE ALIGNING…

The CMTA continued its multi-pronged approach to research in 2014, testing multiple compounds simultaneously and moving forward on a number of fronts:

CMT1A is caused by the duplication of the Peripheral Myelin Protein 22 (PMP22) gene, which leads to the demyelination of the peripheral nerves. Through STAR's efforts, the CMTA identified lead candidate compounds that may slow, stop or reverse the progression of the demyelination and began the process of scrutinizing those compounds for toxicity and appropriate dosing, a necessary step before the compounds can proceed to testing in human trials.

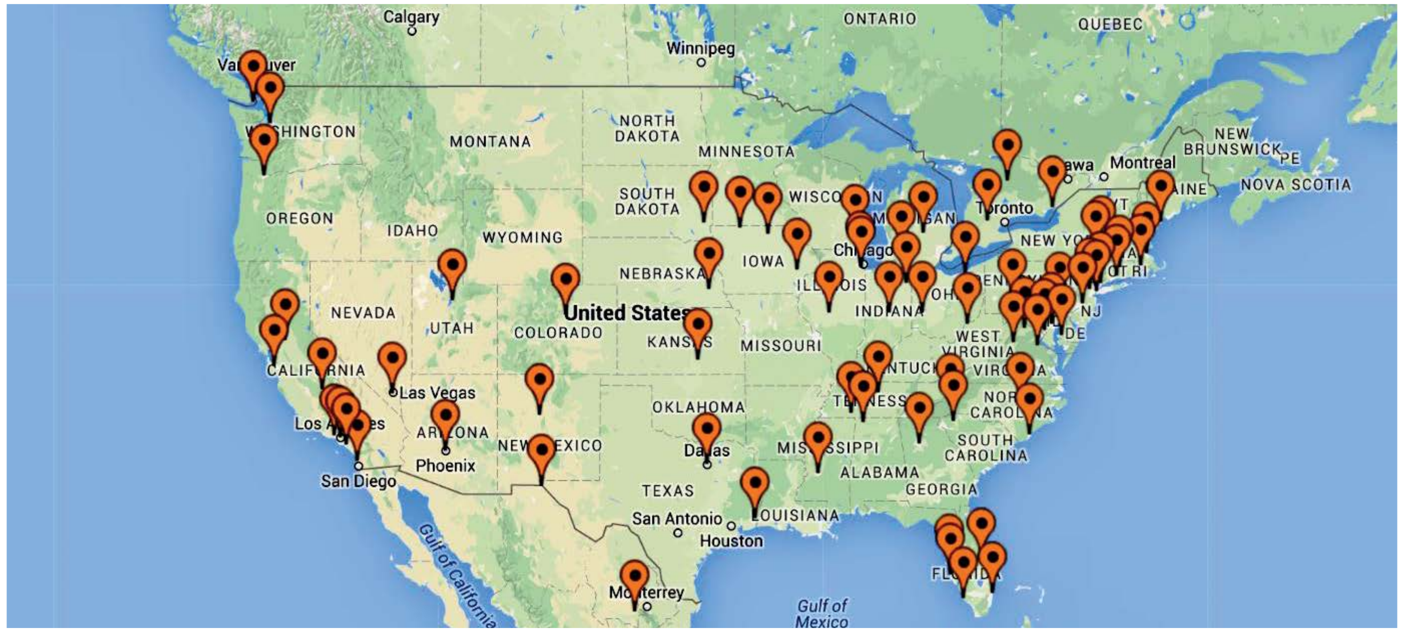
CMT1B: The CMTA initiative to develop a CMT1B project team paid off in 2014 with the funding of a project to develop cellular assays to screen compounds. This work is focused on creating reporters of cellular pathways suspected of a causative role in CMT1B, and being done via collaboration of laboratories at the University of Iowa and the NIH (NCATS). In addition, a further effort to develop animal models of CMT1B is now underway at the University at Buffalo. A CMT1B stem cell effort is also in progress, aimed at determining how many of the many mutations in the Myelin Protein Zero (MPZ) gene cause neuropathy. Patient skin cells representing different mutations in MPZ, banked at the University of Iowa, have been sent to the New York Stem Cell Foundation for transformation into induced pluripotent stem cells (iPSCs) that can then be further differentiated into mature Schwann cells.

CMT1X: 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.

CMT2A is caused by dominant mutations in MFN2. The CMTA's goal with CMT2A is to create an assay that can measure rescue of the 2A defect at a cellular level. The CMTA funded a project by Dr. Stephan Züchner to further refine the development of laboratory models to evaluate the CMT2A pathological process in vivo and provide a tool for pre-clinical testing. To improve the usefulness and applicability of laboratory models, the CMTA is also pursuing development of another model of CMT2A using some newly available technology, and this model is being evaluated for study of the disease and development of drugs.

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. The current research goal is to confirm the significance of these molecular events to the resulting disease pathology. The work is being funded in the laboratory of Dr. Ronald Liem at Columbia University. Most notably, the CMTA also acquired the best available mouse genetic model of CMT2E from Columbia University. This will now be profiled extensively at PsychoGenics to establish its disease phenotype in comparison to the human disease condition.

CMT4: The CMTA is developing a potential translational pathway for commonalities in the various subtypes.



65 support and action groups across North America, including Canada and Mexico

Support & Action Groups

In 2014, the number of people involved in Support and Action Groups increased from 11,000 to 15,000

The CMTA is a national organization, but many of our educational and support efforts take place at the local level. We began 2014 with 60 support and action groups across North America, including Canada and Mexico. By year's end, we had added 15 new ones, two of them in states previously not reached, and the decision was made to rename the groups CMTA branches in recognition of the vital and organic role they play in furthering the CMTA's mission.

The program continues to grow, enabling people with CMT and their loved ones to meet others with CMT and share their experiences, ideas and resources. Members acquire invaluable knowledge about the genetics of CMT, mobility issues, bracing needs, physical therapy, and assistance with other adaptive devices as they hear from other members and from leading clinicians and scientists provided as guest speakers by the CMTA.

Our local groups have also proved themselves adept at fundraising, empowering members to advance a cause that is close to their hearts and have a direct hand in the search for a cure. They are also noteworthy for the critical role they play in bringing awareness to every community they touch. This is especially true during September of each year when many are active participants in CMT Awareness Month activities and events.

Two Patient/Family Conferences in 2014

The CMTA also gathered its members for larger meetings in 2014, holding two Patient/Family Conferences in California. In February, an appreciative audience of some 175 attendees heard presentations by experts in a wide range of medical disciplines at the renowned Cedars-Sinai Hospital in Los Angeles. Northern California was the site of September's Patient/Family Conference as CMTA partnered with the Neuromuscular Disorders Program at Stanford Hospital & Clinics for an exciting program featuring some of the most prominent minds in the fields of CMT research and treatments. Attendees also heard from CMT advocates, motivational speakers and treatment specialists in respiratory, occupational and physical therapy.

Both conferences included fun youth outings—mini-golf in Northern California and an evening of bowling and pizza in Southern California.

2014 Fundraising Efforts and Events…

BOARD MEMBER EVENTS

The year saw the return of some of the CMTA Board's most popular and successful fundraisers and the birth of a new one that could serve as a model for many years to come:

The New York Palace was the elegant setting for the fifth annual CMTA Update Event. Hosted by board members Alan Korowitz and Phyllis Sanders, more than 200 people attended the event, which raised $170,737.

TeamJulia, now in its eighth year, raised $70,552 for the CMTA. Some 30 swimmers participated in the Swim for the Cure hosted by Board chaiman Herb Beron at the Lake Valhalla Club in Montville, N.J. over Labor Day. Corporate partner Aetrex has become the official sponsor of the swim, and its chairman, Larry Schwartz, once again participated, along with other Aetrex employees.

The Swim for the Cure became the Oxford Biathlon as Board Member Steve O'Donnell moved his 13th swim for the CMTA to a different part of the Chesapeake and added a bike ride. The biathlon raised $111,485 for CMT research.

The First Annual Cycle4CMT, organized by Chris Ouellette, brought together friends, family and a few CMTA out-of-towners for a spectacular ride in the colorful fall countryside around Shelburne, Vermont in October. The race, which Chris hopes to replicate nationwide, raised $47,499 (which brought in $22,364 from our new donor matching grant for a total of $70,364) and earned Chris a seat on the Board of Directors.

SUPPORT AND ACTION GROUP EVENTS AND EFFORTS:

The SHARK-O-Marie-Tooth T-shirt became a CMTA Community favorite in 2014, turning a funky, fresh logo into a fun way to create awareness and raising $24,930 for the CMTA STAR program.

Our third annual Awareness Month in September 2014 grew by leaps and bounds. Originally conceived as a way to raise awareness about CMT, the CMT community and Support and Action Groups took the event one step further by not only creating awareness, but raising funds as well. An astonishing $302,930 was raised in September 2014, up from $45,000 in September 2013.

The first Words with Friends Tournament, a clever way to get the CMTA Facebook community involved with our efforts, kicked off in February 2014. Seventy people participated in the tournament, which raised $2,350.

A Circle of Friends created by an Atlanta, GA Support and Action Group member helped the CMTA hit one of its 2-for-1 matches. This amazing member raised $15,557 in two months, simply by asking her family and friends to give.

CMTA Board of Directors/Advisory Board

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.

Steven Weiss joined the Board of Directors in 2014. He has CMT type X, as do his daughter, his mother, and several relatives on his mother's side of the family. Steve became involved with the CMTA in 2010, when he and his wife started the CMT Support & Action Group for the Washington, D.C. metro area. Steve works in public relations for the advocacy arm of the American Cancer Society.

CMTA Board of Directors

Herb Beron, *Chairman*
Elizabeth Ouellette, *Vice Chair*
Gary Gasper, *Treasurer*
StevenWeiss, *Secretary*

Stephen Blevit
Phyllis L. Sanders
Vasi Vangelos
Bob DeRosa
Steven Scherer, MD, PhD
Peter Warfield, MD, MPH
Alan Korowitz
Michael E. Shy, MD
Lawrence Wrabetz, MD
Steve O’Donnell
John Svaren, PhD

The members of the CMTA Advisory Board come from a wide variety of backgrounds to offer insights from their areas of expertise into some of the important issues faced by people and families affected by CMT. Advisory board members touched the CMT community in a variety of ways in 2014, writing articles for *The CMTA Report*, speaking to Support and Action Group meetings nationwide and filming six webinars, up from three in 2013. The CMTA welcomed two new Advisory Board members in 2014:

Glenn Pfeffer, MD, is Director of the Foot and Ankle Center at Cedars-Sinai Medical Center. He is also a Co-Director of the Hereditary Neuropathy Program and Co-Director of the Cedars-Sinai/USC Glorya Kaufman Dance Medicine Center. Dr. Pfeffer has been treating foot and ankle problems in patients with Charcot-Marie-Tooth disease for 25 years.

Greg Stillwell, DPM, is a board certified podiatrist diagnosed with CMT1A, an inventor and the patent-holder of the Barefoot Orthotic™. He lectures internationally on foot and ankle topics, including CMT. Greg is dedicated to helping podiatrists in the United States and Latin America recognize CMT and become current on various diagnostic and treatment options.

The CMTA Advisory Board

Jonah Berger, Business Owner, The Rhythm Within
Gregory Carter, MD, MS – Physiatrist
Katy Eichinger, PT, DPT, NCS – Physical Therapist
Tim Estilow, OTR/L – Occupational Therapist
Shawna Feely, MS, CGC – Genetic Counselor
Sarah Kesty, MA – Special Educator
Sean McKale, CO, LO – Orthotist
Bethany Meloche – Youth Director
David Misener, BSc, CPO, MBA – Orthotist
Elizabeth Misener, PhD, LMSW – Social Worker
James Nussbaum, PT, PhD, SCS, EMT – Physical Therapist
Glenn Pfeffer, MD – Orthopedic Surgeon
Susan Salzberg, OTR/L, MOT – Occupational Therapist
Greg Stilwell, DPM – Board Certified Podiatrist
Carly Siskind, MS, CGC – Genetic Counselor
David Tannenbaum, LCSW – Psychotherapist

website visits grew from 235,212 in 2013 to 298,986 in 2014

Our Online Community

The CMTA’s online community expanded exponentially in 2014. The total number of website visits grew from 235,212 in 2013 to 298,986 in 2014, 62 percent of them new visitors.

We also reached an entirely new segment of our community, creating a page for Spanish language speakers on the web site and translating our materials into Spanish.

We increased our presence on Facebook from 11,000 friends in 2013 to more than 20,000 by the end of 2014 and created a Facebook group for Spanish speakers.

In addition to our Facebook efforts, the CMTA undertook several other online initiatives. We started a CMTA Pinterest with boards on Helpful Products, CMT Awareness, Inspiration, and more. And, to raise awareness in the corporate world, we started a CMTA LinkedIn page.

Our Corporate Sponsors

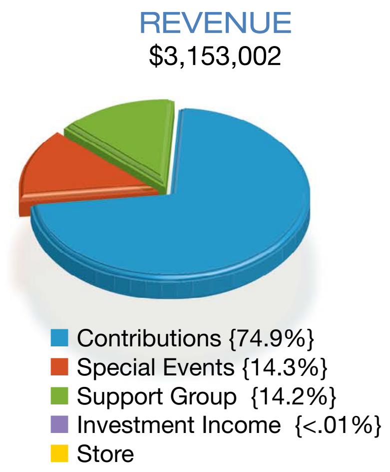
Aetrex Worldwide, Inc., founded in 1946, is widely recognized as the global leader in pedorthic footwear and foot orthotics. Originally known for the development of custom orthotics, medically oriented footcare products and innovations in over-the-counter insoles, Aetrex now offers attractive shoes in their product line that feature extra depth and width to accommodate in-shoe orthotics or AFOs.

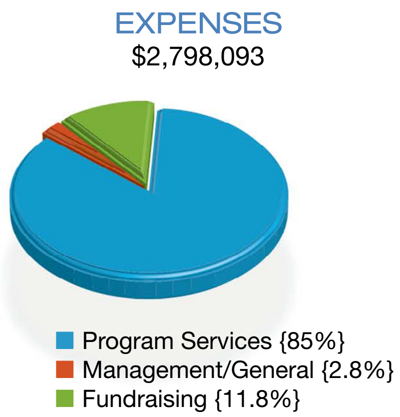
Allard, USA distributes the ToeOFF Family of carbon composite devices designed specifically to assist with foot drop, ankle instability and proximal neuromuscular weakness, all symptoms of CMT. Allard USA offers educational programs, training, and marketing support to increase both consumer and professional awareness of new technology that can offer life-changing options for individuals affected with neurological deficits.

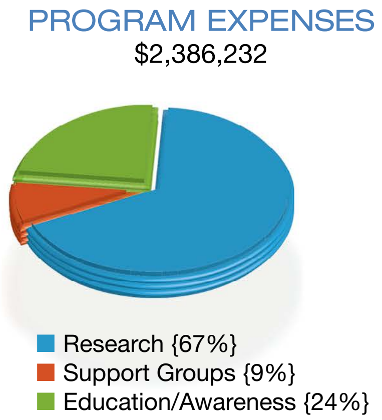
GeneDx launched the Hereditary Neuropathy Panel, a genetic testing panel aimed specifically at testing for different types of CMT, in 2014. With 28 CMT-causing genes available in the Neuropathy Panel, GeneDx delivers an exact genetic diagnosis for 50-70 percent of people with symptoms of CMT. Because CMT1A comprises 70 percent of all CMT, GeneDx also offers a single gene test for PMP22, the most common genetic cause of CMT and HNPP (hereditary neuropathy with liability to pressure palsy).

CONSOLIDATED STATEMENT OF FINANCIAL POSITION

DECEMBER 31, 2014







Revenues	December 31, 2014	December 31, 2013
Contributions	$2,363,470	$1,109,793
Special Events	$452,098	$455,097
Support Group	$336,617	$374,949
Investment Income	$2,615	$2,165
Store	$-1,798	
Grants	–	$5,000
Total Revenues	$3,153,002	$1,946,570
Expenses		
Program Services	$2386,232	$1,621,531
Management and General	$80,061	$99,300
Fundraising	$331,800	$256,275
Total Expenses	$2,798,093	$1,977,106
Program Expenses		
Research	$1,584,430	
Support Groups	$214,760	
Education & Awareness	$587,042	
Total Expenses	$2,386,232	

Our youngest fundraiser, 11-year-old Emily Goodwin

Thanks to all our donors, large and small...

Three wonderfully generous families made anonymous matching gifts to the CMTA in 2014. In April 2014, two families each agreed to match contributions to the CMTA's Strategy to Accelerate Research (STAR) up to $500,000, for a total of $1.5 million for CMT research. That meant that every research dollar raised until June 30, 2014 was matched not once, but twice, TRIPLING its impact. Post-announcement, gifts large and small began to pour in. Fundraisers were held across the country and on June 24th, 2014 the CMT community met the match, raising more than $1.5 MILLION for STAR!

The Sanofi Genzyme partnership with the CMTA not only pushed the research forward, it also helped the CMTA raise funds. A generous private family fund offered to match every dollar given by a new donor, up to $300,000. This challenge grant is paying for the CMTA's contribution to our alliance with Genzyme, which means that new donors' gifts had a major impact in finding treatments for CMT! By year's end, new donors had matched $161,171.

Our youngest fundraiser, 11-year-old Emily Goodwin, donated some $200 she earned by making lamps, coasters, napkin holders and stools of wood from around her family's place in New Hampshire, stopping only when it got too snowy to gather materials. She has another $300 in orders to fill when the weather warms.

Between our largest donors and our smallest, are the thousands of individuals who make up the CMTA's incredibly committed team. In 2014, they walked, rolled, bowled, rode and swam to raise funds for CMT. Without them, there would be no educational programs, outreach efforts, or research. Without them, there would be no CMTA. We are grateful to each and every one of them.

STAR TEAM

Michael Shy, MD (Chair), University of Iowa

Steven Scherer, MD, PhD (Co-Chair)
University of Pennsylvania

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

Anita Bhattacharyya, PhD
University of Wisconsin

Patricia Dranchak, PhD, National Center for Advancing Translational Sciences

M. Laura Feltri, MD, University at Buffalo

James Inglese, PhD
National Institutes of Health

Kristjan Jessen, MSc, PhD
University College, London, England

Lars Knutsen, PhD
Discovery Pharmaceutical Consulting

Ronald Liem, PhD, Columbia University

Ryan MacArthur, PhD, National Center for Advancing Translational Sciences

Rudolph Martini, PhD University of Würzburg, Würzburg, Germany

Albee Messing, VMD, PhD
University of Wisconsin

Rhona Mirsky, PhD, University College, London, England

Klaus-Armin Nave, PhD
Max Planck Institute for Experimental Medicine, Göttingen, Germany

Brian Popko, MD, University of Chicago

Mary Reilly, MD, National Hospital for Neurology and Neurosurgery
London, England

Mario Saporta, MD, PhD, National Stem Cell Laboratory, LaNCE UFRJ, Brazil

Mark Scheideler, PhD
HumanFirst Therapeutics

Michael Sereda, MD
Max Planck Institute for Experimental Medicine, Göttingen, Germany

Ueli Suter, PhD, ETH Institute
Zurich, Switzerland

John Svaren, PhD, University of Wisconsin

Vincent Timmerman, MD, University of Antwerp, Antwerp, Belgium

Claes Wahlestedt, MD, PhD
University of Miami

Lawrence Wrabetz, MD
University at Buffalo

Brittany Wright, PhD, National Center for Advancing Translational Sciences

Stephan Züchner, MD, PhD
University of Miami

CMTA STAFF

Patrick Livney, Chief Executive Officer

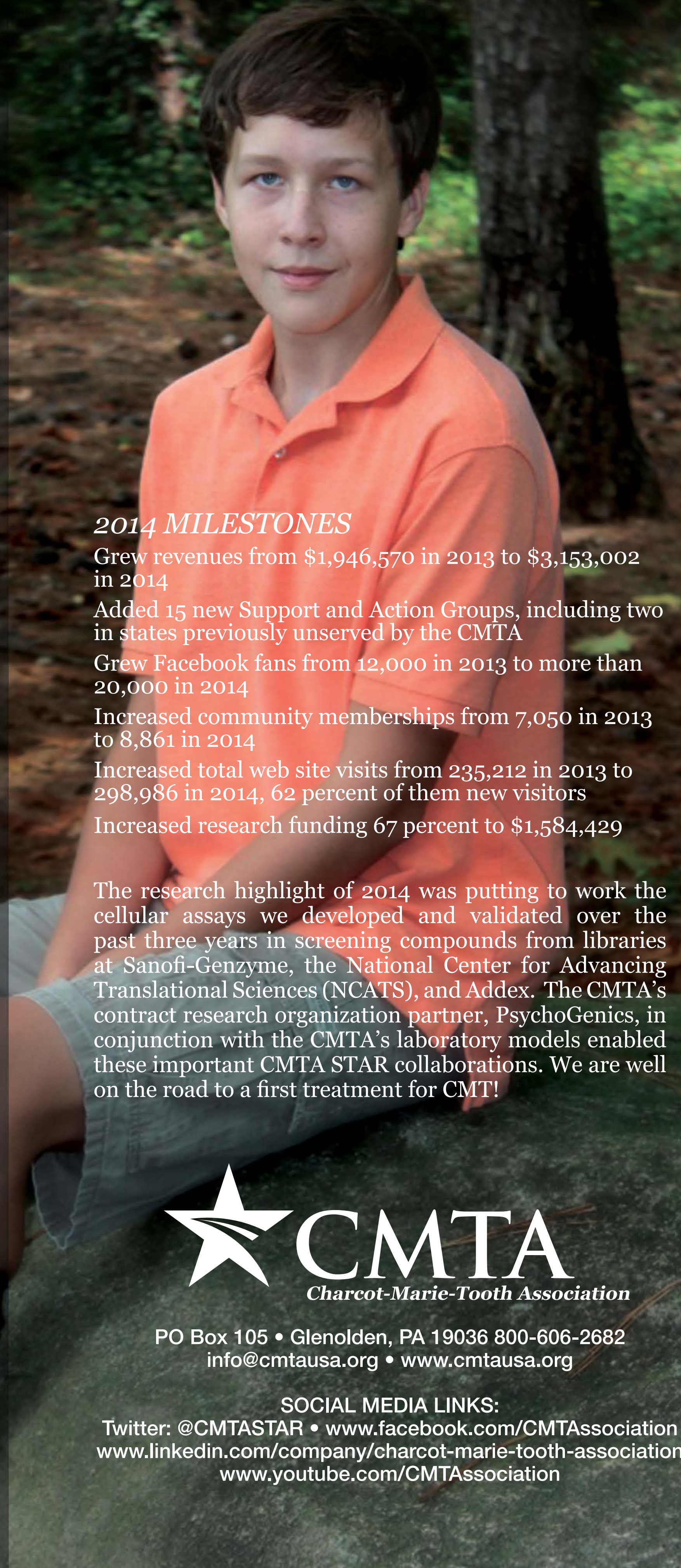
Patricia Dreibelbis,
Director of Program Services

Kim Magee, Director of Finance

Bethany Meloche, Director of Social Media

Susan Ruediger, Director of Development

Jeana Sweeney,
Director of Community Services



2014 MILESTONES

Grew revenues from $1,946,570 in 2013 to $3,153,002 in 2014

Added 15 new Support and Action Groups, including two in states previously unserved by the CMTA

Grew Facebook fans from 12,000 in 2013 to more than 20,000 in 2014

Increased community memberships from 7,050 in 2013 to 8,861 in 2014

Increased total web site visits from 235,212 in 2013 to 298,986 in 2014, 62 percent of them new visitors

Increased research funding 67 percent to $1,584,429

The research highlight of 2014 was putting to work the cellular assays we developed and validated over the past three years in screening compounds from libraries at Sanofi-Genzyme, the National Center for Advancing Translational Sciences (NCATS), and Addex. The CMTA's contract research organization partner, PsychoGenics, in conjunction with the CMTA's laboratory models enabled these important CMTA STAR collaborations. We are well on the road to a first treatment for CMT!

CMTA
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