



expanding the STAR alliance

annual report 2018





our mission

To support the development of 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.

The Charcot-Marie-Tooth Association is a non-profit organization dedicated to finding a cure for CMT. The CMTA's Strategy to Accelerate Research (STAR) program brings top researchers together with pharmaceutical and biotechnology partners and patients to accelerate scientific breakthroughs and develop therapies. The CMTA also offers inclusive community services to help patients and families live their best lives with CMT. These include more than 70 local branches, a robust Advisory Board, a camp exclusively for kids with CMT, annual Patient/Family Conferences and online support communities.

dear friends,

The year 2018 was a momentous one for the Charcot-Marie-Tooth Association. We celebrated two major milestones: our 35th anniversary and the tenth anniversary of our signature research initiative, the Strategy to Accelerate Research (STAR). When the CMTA was founded in 1983, gas cost \$1.25 per gallon, Motorola introduced the first mobile phone, and the Chicken McNugget debuted at McDonald's. One other momentous event happened: Dr. Howard Shapiro founded the CMTA's predecessor, The National Foundation for Peroneal Muscular Atrophy, following a meeting of patients, physicians and researchers dedicated to ridding the world of CMT.



Amy Gray
Chief Executive Officer

In 2008, the CMTA's research efforts were consolidated under the STAR banner, which applied sound business principles to managing research. In the ensuing decade, the STAR alliance has expanded to more than 20 pharmaceutical, biotech and service partners, double the number in 2017 (which was double the number in 2016). The CMTA has spent more than \$12 million to finance dozens of research projects, \$2.021 million in 2018 alone. The CMTA carefully stewards every donation, spending 85 percent of every dollar on mission and earning top ratings from Guidestar and Charity Navigator for its transparency, accountability and governance. The CMTA's research tools, network of experts, testing infrastructure and patient partnerships make it the premier CMT research organization.

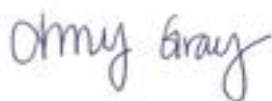
Another momentous event took place in 2018: In July, the CMTA convened a gathering of 20 of the top gene therapy experts in the world to talk about using gene therapies to treat the root causes of CMT. Immediately after that summit, the CMTA began assembling experts to formulate gene therapy strategies for CMT2 and CMT1 subtypes, and four gene therapy experts were appointed to the CMTA's Scientific Advisory Board.



Gilles Bouchard
Chairman of the Board

In addition to groundbreaking research, the CMTA is committed to helping the members of the CMT community. Some 73 CMTA branches provide information and encouragement to patients nationwide, while 32 CMTA Centers of Excellence provide patients with world-class, multidisciplinary care. Educational efforts like our Patient/Family Conferences and a summit for the leading foot and ankle orthopedic surgeons were also on the menu in 2018.

With your help, the CMTA expanded on almost every front in 2018—research projects, research partners, branches, Centers of Excellence and Camp Footprint. We are so grateful for the support that powered the expansion. We reached or exceeded all our goals for the year, then set new ones. Working together, we believe that we can accomplish our ultimate goal of a world without CMT.



Amy Gray, Chief Executive Officer



Gilles Bouchard, Chairman of the Board

goal:

add gene therapy to STAR arsenal



At the CMTA, we believe that in the not too distant future gene therapy for CMT will be a reality beyond the lab. That's why we expanded our Strategy to Accelerate Research program and STAR Advisory Board in 2018 to include four gene therapy experts and a number of research projects. The current momentum points to Food and Drug Administration approval of several new CMT therapies in the next five years, and the FDA forecasts as many as 20 cell- and gene-therapy approvals per year by 2025. In addition, the CMTA has already approved or is reviewing several gene therapy projects that cover TYPES 1, 2 and 4.

Work with other motor neuron diseases may provide a roadmap for the CMTA. For example, researchers successfully used AAV9 (adeno-associated virus) to target the gene responsible for a fatal motor neuron disease in infants called spinal muscular atrophy. AAV9 containing the *GAN* gene is in a clinical trial for a rare recessive disease known as giant axonal neuropathy that affects both central and peripheral neurons. The CMTA has initiated discussions with several scientists and

companies with expertise in AAV vectors to further develop this mode of treatment.

While the first approach in gene therapy is to replace the defective protein with the correct one, it can also be used to “silence” a disease-causing mutation or to edit out a defective gene using CRISPR. The CMTA is actively supporting this type of therapy development.

We expanded our Strategy to Accelerate Research program and STAR Advisory Board in 2018 to include four gene therapy experts and a number of gene therapy projects.

goal:

increase patient role in research



The CMTA is more than just a research organization: It is a research organization with an energetic and engaged community behind it. At the CMTA, we never forget that patients are the reason for the research and that many of them have, quite literally, given of themselves with skin and blood samples. In recognition of that fact, the CMTA enhanced and formalized the patient's role in research efforts in 2018, launching its Patients as Partners in Research (PPR) initiative. PPR's goals are to advance the work of our Strategy to Accelerate Research (STAR) and to enroll the patient community in the critical work of furthering the development of treatments and ultimately a cure for CMT.

Some ways people can get involved in the CMTA's Patients as Partners in Research include:

- Completing surveys about symptoms and experiences with CMT
- Participating in focus groups with the CMTA and our strategic partners in the biotechnology and pharmaceutical fields
- Enrolling in CMTA-funded research studies with our clinical and scientific partners
- Joining clinical trials

Patients who join the initiative can build an online profile and check a new page on the CMTA website where current opportunities for participation are listed.



PPR's goals are to advance the work of our Strategy to Accelerate Research (STAR) and to enroll the patient community in the critical work of furthering the development of treatments and ultimately a cure for CMT.

goal:

expand STAR alliance

We are the Driving Force Behind CMT Research.



The CMTA has developed a unique alliance approach to connect our STAR research consortium to groups pursuing therapy development. Responding to the need for access to the knowledge, skills and specialty research resources the CMTA has developed, we created Advisory Boards of experts in the science, drug development and clinical treatment of patients with CMT. We have also assembled preclinical “toolboxes” of animal and human stem cell models of CMT disorders, and invested in their validation for use in the testing of potential therapeutics. The CMTA has placed these models in a network of testing services that employ specialized contract research organizations to complete work under the CMTA’s direction, using a single point-of-contact structure that simplifies and speeds the planning and execution of studies with pharmaceutical and biotech research alliance partners.

By the end of 2018, the CMTA had established working relationships with seven service partners, a network of private and non-profit organizations with expertise in disease models, genetic analysis, research tool creation and animal breeding. A number of animal models of Type 1 and 2 CMTs have been made or licensed and implemented in therapeutic evaluation, and a bank of patient stem cell lines representing six different CMT disorders has been established.

The CMTA has formed research alliance relationships with companies in small molecule and genetic therapies and is now actively engaged in therapeutic testing alliances with organizations interested in creating new therapies for CMT disorders or in re-positioning therapies developed for other diseases.

By the end of 2018, the CMTA was funding more than 50 active research projects and working with more than 20 research alliance and service partners (with a dozen more in early discussions). Two of these (Sanofi U.S. and Ionis Pharmaceuticals) represent ongoing research alliances for the development of novel CMT therapeutics. Some of the other testing alliances were initiated to evaluate potential therapies in both Type 1 and 2 CMT disorders. Of the alliance projects, half involve the identification and validation of Type 1 CMT therapies and half are directed at Type 2 therapy evaluation. The therapies being tested would benefit virtually all types of CMT, including those that do not have a definitive genetic diagnosis.

STAR Research Updates by Type

CMT1A

CMT1A is caused by the duplication of the Peripheral Myelin Protein 22 (*PMP22*) gene, which leads to the demyelination of the peripheral nerves. Ionis Pharmaceuticals, which pioneered an innovative drug called an antisense oligonucleotide (ASO), used it to halt the development of neuropathy in mouse and rat models with CMT1A. Rodent studies showed a dramatic improvement in two models of CMT1A, and Ionis is currently working on developing refined versions for testing in clinical trials. After screening the entire compound collection of Genzyme, a Sanofi company, the STAR Alliance is now testing additional molecules from other Sanofi programs. As a testimony to STAR's growing momentum, five additional CMTA alliance partners are currently testing therapeutic compounds for CMT1A. Dr. Michael Shy and the members of our Clinical Expert Board are leading the effort to develop the best outcome measures and biomarkers for clinical trials of CMT1A therapeutics.

CMT1B

This CMT subtype is caused by mutations in the Myelin Protein Zero (*MPZ*) gene. Scientific Advisory Board members Drs. Michael Shy, Lawrence Wrabetz and Maurizio D'Antonio are experts in this area and, in partnership with InFlectis BioScience, are engaged in further testing of a novel molecule called Sephin. The drug has shown dramatic improvement in the S63del mouse model of CMT1B. We now have mouse models of all three major

clinical presentations of CMT1B. In the late-onset type, we are testing how inhibiting axon degeneration pathways can stabilize motor and sensory neurons, an approach being explored by many of our partner companies. This will be the first test of such pathways in a CMT model and it is possible that this approach may have broad applicability to other types of CMT.

Type 1X

Until recently, there was only one mouse model of CMT1X, but it was not a direct replica of the human mutations in *GJB1*. The CMTA sponsored the development of four additional mouse models, one of them developed in partnership with Dr. Robert Burgess at The Jackson Laboratory. These models will be used to test therapeutic approaches like that taken by Dr. Rudolf Martini at the University of Würzburg, Germany, who has found that reducing inflammation has a very positive effect in a mouse model of CMT1X. This subtype is also characterized by degeneration of motor neurons, which makes it an ideal target for the axon degeneration therapies mentioned above for CMT1B. Finally, 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 is continuing these studies toward clinical trials for both CMT1X and CMT4.

Type 2A

CMT2A is caused by dominant mutations in Mitofusin 2 (*MFN2*). The STAR team has developed two excellent rat models for CMT2A that 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 at Cedars-Sinai Medical Center. As part of its Patients as Partners in Research initiative, the CMTA has sponsored a study with the University of Iowa CMT Clinic and CMTA Center of Excellence to look at pulmonary function for people who have CMT2A. In partnership with several companies, therapeutic approaches being studied include inhibition of axon degeneration and gene therapy. Finally, other candidate molecules have emerged from academic research, with planning underway to test these as well.

Type 2E

CMT2E is caused by dominant mutations in the Neurofilament Light Protein (*NEFL*) gene. With support from the CMTA, one of the best mouse models of CMT2E, made by Dr. Ronald Liem at Columbia University, has been extensively characterized in collaboration with Dr. Steven Scherer at the University of Pennsylvania. Both human and mouse stem cells containing CMT2E mutations have been differentiated into motor neurons and are being used in drug screens to identify therapies that prevent the aggregation of neurofilaments seen in CMT2E. Candidate compounds have been identified and are being further tested, and human stem cell cultures are being developed for larger chemical screens.

Type 4

CMT4C is caused when both copies of a gene (*SH3TC2*) required for healthy myelin are deficient. To restore function of these genes, the

gene therapy approach described above for CMT1X has also been tried for CMT4C by Dr. Kleopas Kleopa and has shown very positive results, which have been published. We anticipate this approach will be applicable to other forms of CMT4, and synergistic efforts to optimize Schwann cell gene therapy are in place to facilitate this approach for CMT4, as well as CMT1X and CMT1A.

Unidentified Types

Every year more types of CMT are identified by STAR researchers, but there are many left to discover. The CMTA is helping to identify the genes that cause CMT by supporting work led by Dr. Stephan Züchner of the University of Miami. Identifying mutations is an important first step in the process of developing treatments.

Cross-CMT Initiatives

Biomarkers: Clinical trials will require not only a potent therapeutic agent, but also strong biomarkers/outcome measures that can be used to assess a drug or agent's initial actions in order to justify larger and more definitive trials to assess functional improvement. In partnership with the Inherited Neuropathies Consortium (INC), the CMTA is funding development of strong clinical imaging outcome measures and biomarkers for CMT1A. Work is also underway to develop similar measures for CMT2A, CMT1B and CMT1X.

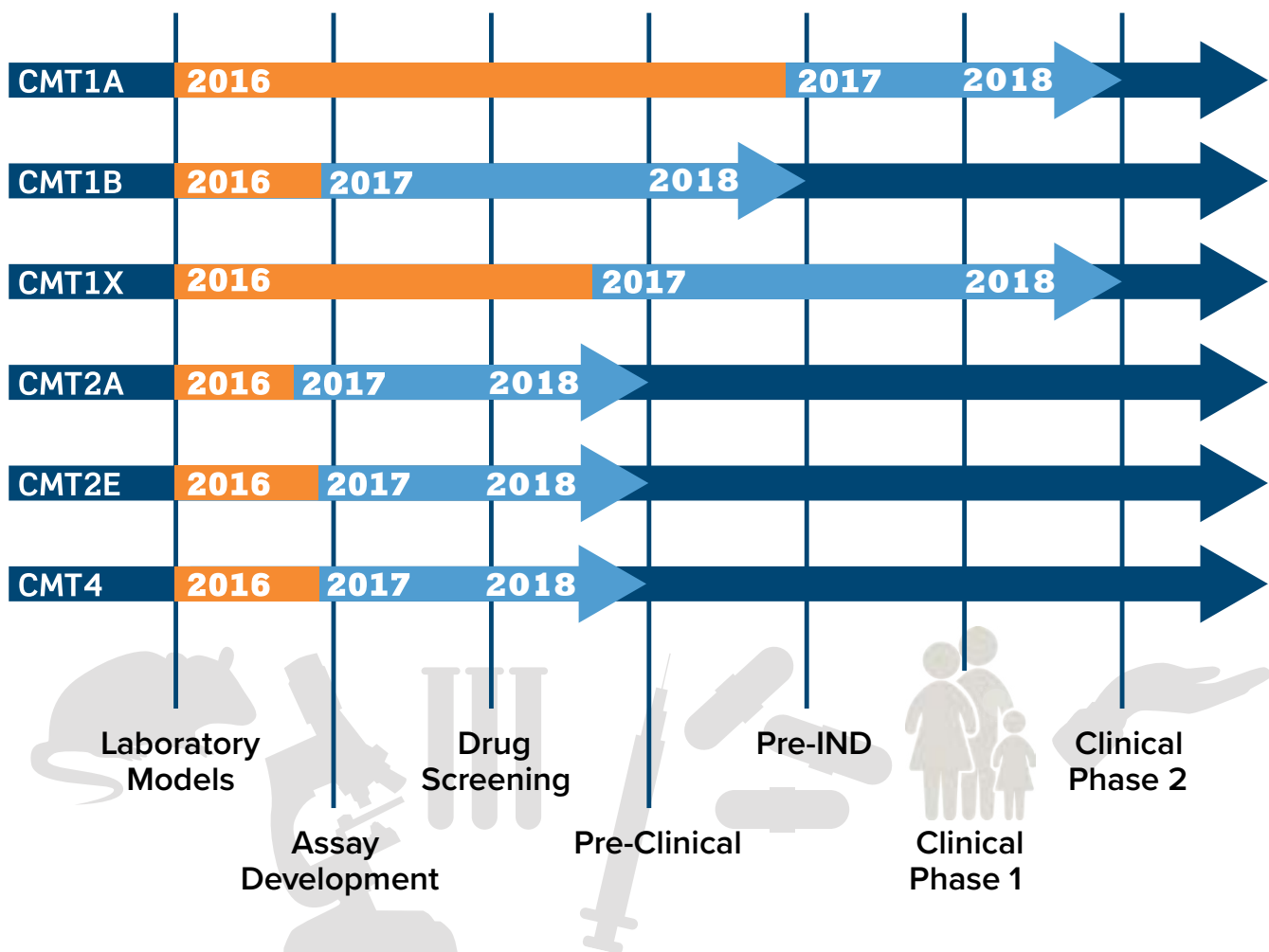
Axon Degeneration: Axonal loss is the final common pathway of all peripheral neuropathies, including all forms of CMT. Even in CMT1, axonal loss (and not demyelination) correlates with clinical disability. Genetic studies have identified specific genes that stabilize axons and prevent their degeneration and proof of principle studies have shown that specific genes can be targeted to enhance axon survival in some models of

peripheral neuropathy, such as diabetic neuropathy. The CMTA is collaborating with Dr. Jeff Milbrandt (Washington University) to perform tests in our rat model of CMT2A, and parallel efforts are in progress for CMT1B and CMT1X. Several companies have developed, or are developing, candidate drugs for this pathway in order to preserve axons and are testing them in partnership with the CMTA.

Gene Discovery: Although significant progress has been made to identify the more than 90 genes that are mutated in various types of CMT, roughly half of CMT2 patients do not yet have a definitive genetic diagnosis. The CMTA is supporting the work

spearheaded by Dr. Züchner, who performs whole exome sequencing of DNA from CMT2 patients to identify new disease-causing mutations in patients seen in the Inherited Neuropathies Consortium. He and his team have developed state-of-the-art techniques to analyze the data and created a user-friendly database that CMT neurologists and scientists worldwide use to analyze and share data, add their own detailed clinical observations, rate the pathogenicity of genetic variants and suggest the addition of new CMT genes. The project is equally important for diagnosis recommendations and for continued discovery research as each new cause of CMT yields important clues and potential drug targets for CMT2 therapy development.

The CMTA is working vigorously to find treatments, and ultimately a cure, for all types of CMT. We are investing in projects that will benefit virtually all people with CMT. The chart below shows the progress made along the path to clinical trials.



\$2.021M
SPENT ON STAR



\$292,198
RAISED BY WALKS 4 CMT



31 WALKS



85%

OF EVERY DOLLAR
SPENT ON MISSION

50
RESEARCH PROJECTS



20
RESEARCH
PARTNERS

99% OF CMT PATIENTS
COVERED BY AN ACTIVE
RESEARCH PROJECT



73 BRANCHES



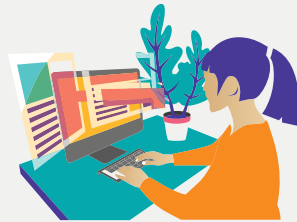
20
BY
NUM



74 CAMPERS AT CMTA
CAMP FOOTPRINT



52 CAMP COUNSELORS



821,230
PAGE VIEWS ON CMTAUSA.ORG

18
THE
BERS

41,592
SOCIAL MEDIA FOLLOWERS



17 ATTENDEES AT
SURGERY SUMMIT



275 ATTENDEES AT
PATIENT/FAMILY
CONFERENCES



2 PATIENT/FAMILY
CONFERENCES



292 BRANCH MEETINGS

goal:

increase and invigorate our community



While the CMTA leads the way in CMT research aimed at improving our future, it also recognizes that constituents need services and support today. As an organization, we continually strive to create a community in which people feel embraced and engaged. CMTA branches are the main touchpoint between the national organization and its members: The CMTA provides members education and support and members raise funds and awareness for the parent organization. The CMTA added three new branches in 2018—in South Louisiana; Jacksonville, Florida and El Paso, Texas.

The CMTA also has an active online community, with more than 35,000 Facebook followers who share information, resources and life experiences on a daily basis. In addition, the CMTA has more than 3,000 Twitter followers; more than 2,700 Instagram followers; and more than 700 LinkedIn professionals. The CMTA's online community raised \$66,368 for STAR research on Giving Tuesday (the Tuesday after Thanksgiving), besting the previous record high for the day.



One community member deserves special mention for his 2018 contributions to the CMTA. James McKenzie (Jim) Lea died in December 2016, but at the CMTA his memory will live forever. Jim, the inventor of the Therm-a-Rest self-inflating camping mattress, left \$2.1 million to the CMTA in 2018, ensuring that children with CMT will grow up with the hope of a world without the disease. You could almost say that he created a cushion against the hard ground for the CMTers who come after him.

goal:

increase access to CMTA Centers of Excellence



One of the clearest indications of the CMTA's growth in 2018 was the explosion in the number of CMTA Centers of Excellence, which skyrocketed from 22 to 32. These patient-centric, multidisciplinary clinics are staffed by some of the highest quality CMT clinicians and researchers in the world, providing comprehensive care to children, adults and families affected by CMT.

The CMTA Centers of Excellence also provide invaluable information to researchers searching for a cure for CMT, such as how different types of CMT progress over time and whether novel medications are slowing the course of the disease. This information is critical as the CMTA begins clinical trials for candidate therapies. The information is shared through the Inherited Neuropathies Consortium, a group of academic medical centers, patient support organizations and clinical research resources sponsored in part by the CMTA.

The 10 Centers of Excellence that came onboard in 2018 are:

Hospital for Special Care (New Britain, CT)*
University of Missouri (Columbia)
Northwestern Memorial Hospital (Chicago)*
Ohio State University (Columbus)
Oregon Health & Science University (Portland)*

University of Illinois at Chicago (Chicago)*
Children's Hospital of Pittsburgh (Pittsburgh)*
University of North Carolina (Chapel Hill)*
University of Pittsburgh Medical Center (Pittsburgh)*
Wayne State University (Detroit)

*These Centers of Excellence are not part of the Inherited Neuropathies Consortium.

goal:

increase awareness and events





The CMTA relies on personal donations and community fundraising to fund our mission, and the branches play a key role in making that happen. Large or small, everything our community does to generate dollars powers the CMTA's ability to invest in treatments and therapies.

The branches also play a key role in expanding CMT awareness. They do so in two key ways: by holding Walk 4 CMT events throughout the year and by holding a multitude of events and fundraisers during CMT Awareness Month in September. Walk 4 CMT events were held in 31 different locations nationwide in 2018, bringing together more than 1,500 people who care about finding a cure for CMT and raising \$292,198 for research. "Walks and runs can be intimidating for people with CMT, but a Walk 4 CMT is something special for us," Central New Jersey Branch Co-Leader Mark Willis said. "The majority of the people walk just like you! Some don't even walk," he added, "They ride their scooters. This was so much more of a community than any other fundraising event I've attended."

In addition to the numerous Walks 4 CMT, members and branches held a number of innovative fundraisers throughout 2018, including fishing tournaments, wine and chocolate tastings, bingo nights, yard and bake sales and car washes.

"Walks and runs can be intimidating for people with CMT, but a Walk 4 CMT is something special for us. The majority of the people walk just like you! Some don't even walk. They ride their scooters. This was so much more of a community than any other fundraising event I've attended."—Central New Jersey Branch Co-Leader Mark Willis.

goal:

create educational opportunities



Some 275 people attended the CMTA's two Patient/Family Conferences in 2018, one covering New York and New Jersey and one in Seattle.





In October 2018, the CMTA convened a group of 17 of the leading foot and ankle orthopedic surgeons in the United States for a summit on the surgical treatment of cavo-varus deformity from CMT.

Another key component of the CMTA's mission is education. Patients need education and information, as do specialists who practice in the CMT field. The CMTA provides educational opportunities for both groups. Daylong Patient/Family Conferences allow patients to learn from and network with the clinicians who treat them and the researchers dedicated to finding treatments and a cure for CMT. They also feature presentations from local clinicians and health care providers on topics such as 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. Some 275 people attended the CMTA's two Patient/Family Conferences in 2018, one covering New York and New Jersey and one in Seattle.

For those unable to attend a Patient/Family Conference, the CMTA made valuable information available online in two webinars in 2018: In November, CMTA Board Chairman Gilles Bouchard presented "STAR: The Webinar," an informative and exciting overview of what the CMTA is doing to advance treatments for CMT. In August, physiatrist Dr. Sabrina Paganoni and physical therapists Katey Burke and Amy Swartz from the Massachusetts General Hospital's CMT Center of Excellence teamed up to deliver best-in-class exercise recommendations for patients with CMT.

The CMTA is also dedicated to educating those who treat CMT patients. In October 2018, the CMTA convened a group of 17 of the leading foot and ankle orthopedic surgeons in the United States for a summit on the surgical treatment of cavo-varus deformity from CMT. Led by CMTA Advisory Board Member Dr. Glenn Pfeffer—director of the Foot and Ankle Center at Cedars-Sinai Medical Center in Los Angeles—the goal of the one-day meeting in Chicago was to develop a consensus opinion on the best surgical approach to these disabling deformities. A white paper memorializing the summit's findings is scheduled for publication in a highly respected orthopedic journal by the end of 2019.

goal:

expand camp footprint



The CMTA's Camp Footprint is the only camp in the United States just for kids with CMT.



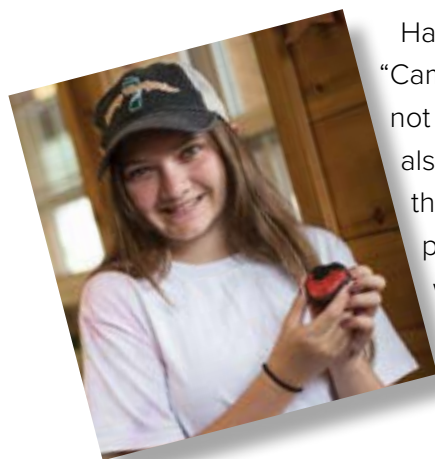
The CMTA continued to expand Camp Footprint in 2018, just as it has every year since starting the only U.S. camp for kids with CMT in 2016. The number of campers increased from 64 to 74 and the number of counselors from 35 to 52.

Children with CMT face physical challenges daily. For many, walking is difficult and running impossible. Even something as mundane as picking a coin up off a table can be challenging. Camp Footprint gives campers the rare opportunity to master their environment, to participate in never-before-tried activities like zip-lining, horseback riding and canoeing and to celebrate their abilities. Children at Camp Footprint enjoy all the benefits that campers without physical challenges do: increased independence and self-confidence; the chance to take part in fun activities; interaction with other campers and lasting friendships. In addition, Camp Footprint campers benefit from the positive role models offered by adult camp counselors and staffers who all have CMT, the chance to be independent of their parents and doctors for a while, increased physical activity and increased time outdoors.



Hannah Miles, 15, described her experience this way:

“Camp Footprint gives those of us with CMT the opportunity to not only make great friends and have an amazing experience but also to learn from one another. All of us there understand more than anyone else the struggles in our everyday lives—bullying, pain, lack of balance, or even opening bottles. But, as a family, we can encourage and help one another.”



board of directors



STAR honorees celebrate the 10th anniversary of STAR at the 9th Annual New York Gala and Auction.



In August, more than 165 enthusiastic participants joined Gilles Bouchard, Elizabeth Ouellette and Chris Ouellette for the 5th Annual Cycle for CMT, biking through the breathtaking countryside around Vermont's Lake Champlain before an after-party and auction.



28 swimmers, 61 bikers and 32 walkers joined Steve O'Donnell on Maryland's Eastern Shore for the Fifth Annual Oxford "Fun"athlon.

The CMTA's Board of Directors is a dedicated cadre of business owners, executives, doctors and lawyers charged with overseeing the organization's operations and strategy. Because they are all personally affected by CMT, they are deeply committed to the organization and give generously of their time and talents. Board members collectively contributed or raised \$606,101 to support the CMTA's mission in 2018.

Board members organized three major fundraisers in 2018. In June, 28 swimmers, 61 bikers and 32 walkers joined Steve O'Donnell on Maryland's Eastern Shore for the Fifth Annual Oxford "Fun"athlon. In August, more than 165 enthusiastic participants joined Gilles Bouchard, Elizabeth Ouellette and Chris Ouellette for the 5th Annual Cycle for CMT, biking through the breathtaking countryside around Vermont's Lake Champlain before an after-party and auction. Finally, in October, members of the CMT community joined Phyllis Sanders and Alan Korowitz to celebrate the 10th anniversary of STAR at the 9th Annual New York Gala and Auction at the elegant Essex House. Collectively, the three events added \$483,244 to CMTA coffers.

CMTA BOARD OF DIRECTORS

Gilles Bouchard, Chairman
Gary Gasper, Treasurer
Herb Beron, Secretary
Thomas W. Dubensky, Jr., Ph.D.
Laura Fava
Alan Korowitz
Steve O'Donnell
Chris Ouellette
Elizabeth Ouellette
Phyllis Sanders, Esq.
Steven Scherer, M.D., Ph.D.
Michael Shy, M.D.
John Svaren, Ph.D.
Lawrence Wrabetz, M.D.
Special Advisor to the Board: Bruce Chizen

STAFF

Like the board, the staff is committed to the CMTA's vision—a world without CMT. Most staffers either have CMT, or are personally connected to it through family and friends. They won't rest until there is a cure.

Amy Gray, Chief Executive Officer
Andi Cosby, National Events Manager
Leslie Nagel, Marketing Coordinator
Kim Magee, Director of Finance and Administration
Laurel Richardson, Director of Community Outreach
Jeana Sweeney, Director of Development
Elizabeth Ouellette, Board Member, Full-Time Volunteer

cmta advisors

CMTA STAR ADVISORY BOARD

The CMTA's STAR Advisory Board includes 31 of the top CMT scientists from around the world, including the four gene therapy experts added in 2018. It comprises a Scientific Advisory Board, a Therapy Expert Board and a Clinical Expert Board.

John Svaren, Ph.D., Chair,
Scientific Advisory Board

Mark Scheideler, Ph.D., Chair,
Therapy Expert Board

Michael E. Shy, M.D., Co-Chair,
Clinical Expert Board

Mary Reilly, M.D., Co-Chair,
Clinical Expert Board

Frank Baas, M.D., Ph.D.

Robert H. Baloh, M.D., Ph.D.

Joshua Burns, Ph.D.

Beverly Davidson, Ph.D.

Maurizio D'Antonio, Ph.D.

M. Laura Feltri, M.D.

Richard Finkel, M.D.

Steven Gray, Ph.D.

Scott Harper, Ph.D.

David Herrmann, M.D.

Tage Honore, Ph.D.

Christopher Klein, M.D.

Kleopas Kleopa, M.D.

Lars J. S. Knutsen, Ph.D.

Jun Li, M.D., Ph.D.

Rudolph Martini, Ph.D.

Michael McDermott, Ph.D.,
Consultant

Albee Messing, VMD, Ph.D.

Klaus-Armin Nave, Ph.D.

Davide Pareyson, M.D.

Brian Popko, M.D.

Mario Saporta, M.D., Ph.D.

Steven S. Scherer, M.D., Ph.D.

Claes Wahlestedt, M.D., Ph.D.

Lawrence Wrabetz, M.D.

Stephan Züchner, M.D., Ph.D.

CMTA ADVISORY BOARD

Advisory Board members are experts in a wide array of fields and provide invaluable information on topics including bracing, genetic counseling, physical and occupational therapy, emotional well-being, resources for parents and kids, disability benefits and surgery.

Jonah Berger, M.Ed.

Gregory Carter, M.D., M.S.

Bob DeRosa

Katy Eichinger, Ph.D., PT, DPT, NCS

Ashraf Elsayegh, M.D., FCCP

Tim Estilow, OTR/L

Shawna Feely, M.S., CGC

Valery Hanks, OTR/L, C/NDT

Sarah Kesty, M.A.

Kate Lair, M.A., ALHC

Sean McKale, C.O., L.O.

Bethany Noelle Meloche

Tom Meloche

David Misener, B.Sc. (HK), CPO, MBA

Elizabeth Misener, Ph.D., LMSW

James Nussbaum, PT, Ph.D., SCS, EMT

Sabrina Paganoni, M.D., Ph.D.

Glenn Pfeffer, M.D.

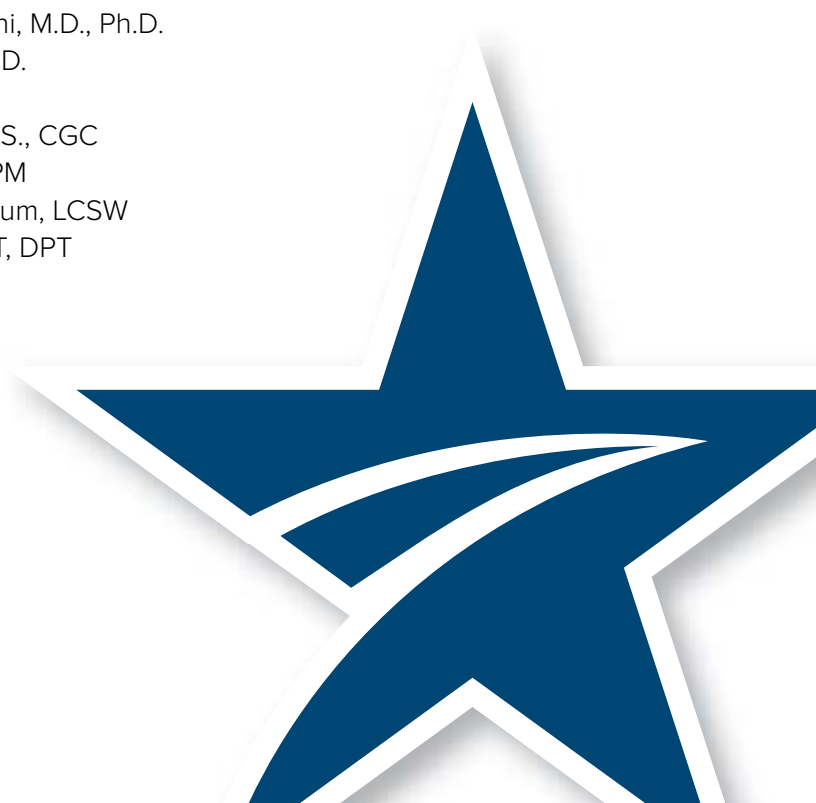
Clark Semmes

Carly Siskind, M.S., CGC

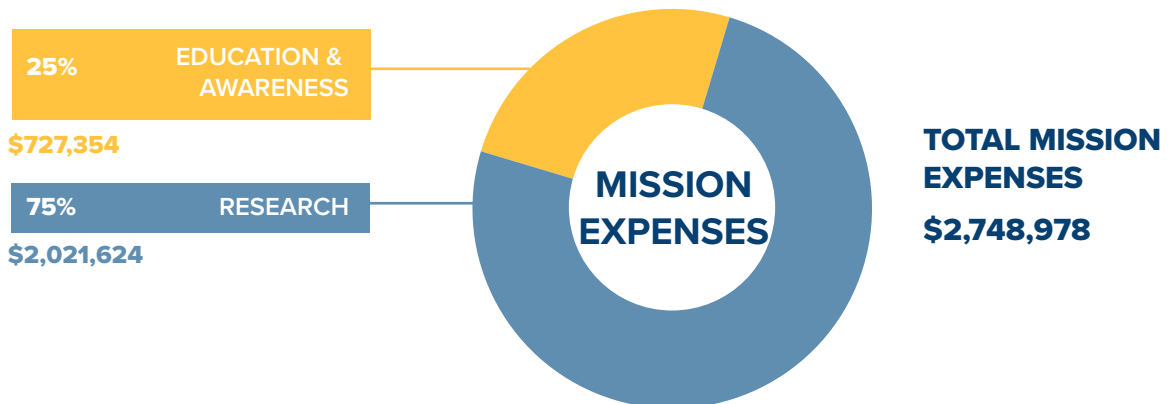
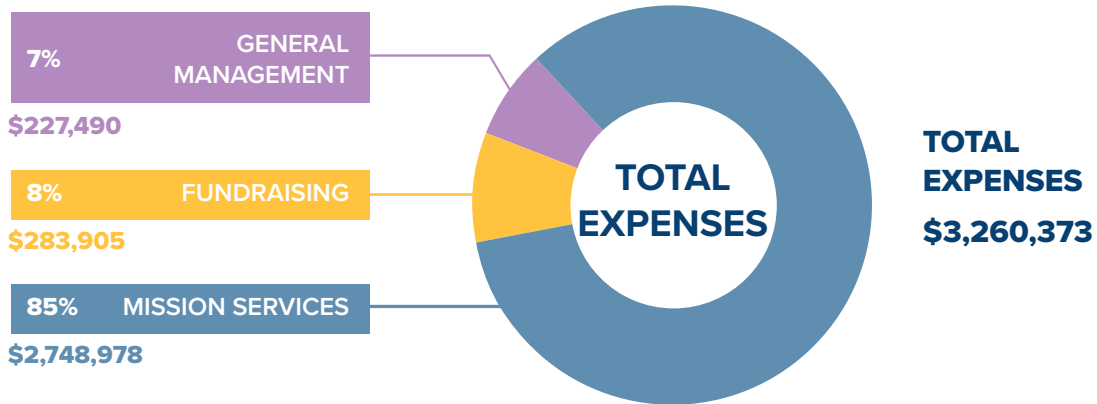
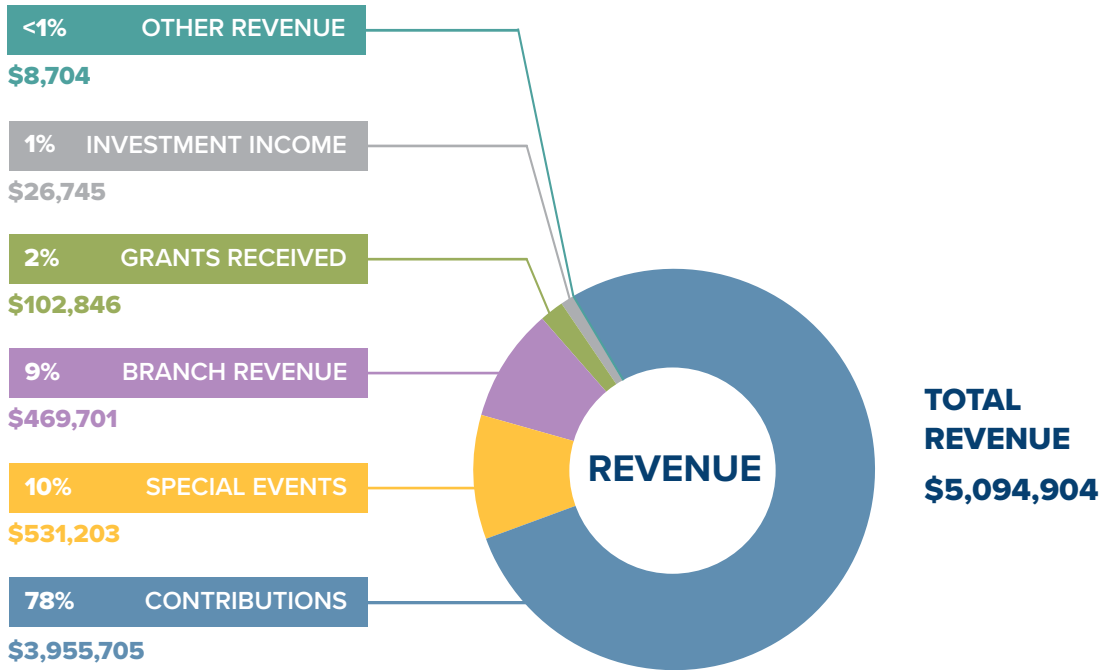
Greg Stilwell, DPM

David Tannenbaum, LCSW

Amy Warfield, PT, DPT



CONSOLIDATED STATEMENT OF FINANCIAL POSITION
December 31, 2018



achieving our goals

With your help, the CMTA expanded on almost every front in 2018—research projects, research partners, branches, Centers of Excellence and Camp Footprint. We are so grateful for the support that powered the expansion. We reached or exceeded all our goals for the year, then set new ones.

Working together, we believe that we can accomplish our ultimate goal of a world without CMT.



Charcot-Marie-Tooth Association

TREATMENT DRIVEN • COMMUNITY POWERED

P.O. Box 105 • Glenolden, PA 19036 • 800-606-2682

CMTAUSA.ORG