

2019 ANNUAL REPORT



TREATMENT DRIVEN

COMMUNITY POWERED





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, Camp Footprint exclusively for kids with CMT, annual Patient/Family Conferences and online support communities.

*Eleven-year-old Ava Greeves
enjoying Camp Footprint*

TREATMENT DRIVEN

COMMUNITY POWERED



Amy Gray
Chief Executive Officer



Gilles Bouchard
Chairman of the Board

Treatment driven. Community powered. Those four simple words embody the CMTA's work in 2019. As always, our work focused on the search for treatments and a cure for CMT. And, as always, that search was powered by a strong, resourceful and committed community.

Led by the world's foremost researchers and clinicians, the CMTA's Strategy to Accelerate Research (STAR) gathered even more momentum in 2019. We have made so much progress from just three years ago. Today, we have more than 32 STAR alliance partners, testimony to the acceleration promised in STAR's name.

Our integrated team of top scientists and industry partners worked on a host of research projects in 2019, encompassing everything from gene therapy to small molecule therapies to drugs that promote axon survival and preserve nerve function.

The sophistication of the CMTA's research strategy is complemented by the patient voices the CMTA is able to harness in support of it. Community members continued to enroll in our Patients as Partners in Research (PPR) initiative in 2019, 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, signing up for CMTA-funded research studies with our clinical and scientific partners and joining clinical trials.

Community members don't just participate in research, they fund a substantial portion of it. Volunteers held 32 Walks 4 CMT in 2019, bringing in \$332,500 for STAR projects. The Board of Directors showed their commitment by raising \$916,701 to support the CMTA's mission and major donors challenged the community with \$2 million in matching donations to support Types 1 and 2 research.

The CMTA's stewardship of those donations earned it the highest possible rating, 4 stars, from Charity Navigator, meaning that we exceed industry standards and we are one of the most trusted and respected health care organizations.

All segments of our community worked together in 2019 to drive the search for a treatment forward. So long as our community remains engaged and inspired, we believe that search will be successful.

Amy Gray, Chief Executive Officer

Gilles Bouchard, Chairman of the Board

TREATMENT DRIVEN

STAR ALLIANCE

The CMTA's vision is a world where patients living with CMT have therapies available to them to manage, modify and ultimately cure their disease. That vision is poised to become reality as our Strategy to Accelerate Research (STAR) drives research forward at an ever-increasing pace. Since 2008, STAR has been a beacon of light and hope for the CMT community. STAR's unique approach is built on collaboration: It connects our world-renowned STAR Advisory Board to pharmaceutical, biotechnology and research service companies that are pursuing therapy development. These STAR alliance members, along with the CMT patients who are the real experts, complement a network of sponsored university laboratories and biotech companies positioned to deliver therapies to the market in the near future. The CMTA's funding and operations focus is on translational research that will lead as directly as possible to therapeutic treatments for CMT.



| STAR ALLIANCE PARTNERS | | THERAPY TYPE | DRUG DEVELOPMENT STAGE | | | | | |
|------------------------|---|--------------------------------|------------------------|----------------|-------------|--------|--------|--------|
| | | | Discovery | Research Tools | Preclinical | Phase1 | Phase2 | Phase3 |
| 1A | Pharnext | Small Molecule | | | | | | P3 |
| | Acceleron* | Biological | | | | | P2 | |
| | InFlectis BioScience | Small Molecule | | | | P1/P2 | | |
| | Cyprus + University of Wisconsin + University of Texas, SW | Gene Therapy | | | Preclinical | | | |
| | Ionis | Gene Therapy | | | Preclinical | | | |
| | National Institutes of Health* | Assays/Small Molecule | | | Preclinical | | | |
| | Regenacy Pharmaceuticals | Small Molecule | | | Preclinical | | | |
| | Sanofi* | Small Molecule/Biomarkers | | | Preclinical | | | |
| | Star Biotech Partner A | Small Molecule | | | Preclinical | | | |
| | Star Biotech Partner B* | Small Molecule | | | Preclinical | | | |
| | Star Biotech Partner C | Gene Therapy | | | Preclinical | | | |
| | Star Biotech Partner D | Biological | | | Preclinical | | | |
| | University of Rochester, NY | Biomarkers (Wearables) | | | Preclinical | | | |
| | University of Wisconsin + Memorial Sloan Kettering | Target Discovery | Discovery | | | | | |
| 1B | Acceleron* | Biological | | | | | P2 | |
| | InFlectis BioScience | Small Molecule | | | | P1/P2 | | |
| | National Institutes of Health* | Assays/Small Molecule | | | Preclinical | | | |
| | San Raffaele Scientific Institute | Small Molecule | | | Preclinical | | | |
| | Universities of Iowa and Wisconsin | Biomarkers | | | Preclinical | | | |
| | University of Iowa | Target Validation | | | Preclinical | | | |
| | University at Buffalo, NY | Animal Model/Target Validation | | Laboratory | | | | |
| | University at Buffalo, NY | Target Discovery | Discovery | | | | | |
| 1X | Acceleron* | Biological | | | | | P2 | |
| | Cyprus Institute | Gene Therapy | | | Preclinical | | | |
| | University of Pennsylvania | Target Validation | | | Preclinical | | | |
| | University of Illinois (Chicago) | Animal Model | | Laboratory | | | | |
| | The Jackson Laboratory | Animal Model | | Laboratory | | | | |
| 2A | Passage Bio + University of Pennsylvania | Gene Therapy | | | Preclinical | | | |
| | Regenacy Pharmaceuticals | Small Molecule | | | Preclinical | | | |
| | Star Biotech Partner E | Small Molecule | | | Preclinical | | | |
| | Star Biotech Partner A | Small Molecule | | | Preclinical | | | |
| | Star Biotech Partner F | Peptide | | | Preclinical | | | |
| | University of Iowa | Respiratory Study | | | | | P2 | |
| | University of Pennsylvania | Animal Models | | Laboratory | | | | |
| | Star Partner G | Small Molecule | Discovery | | | | | |
| | Gladstone Institute (University of California, San Francisco) | Gene Therapy (CRISPR) | Discovery | | | | | |
| | Washington University | Small Molecule | Research Interest | | | | | |
| 2E | Regenacy Pharmaceuticals | Small Molecule | | | Preclinical | | | |
| | STAR Biotech Partner D | Biological | | | Preclinical | | | |
| | Nationwide Children's Hospital + Ohio State University | Gene Therapy | | | Preclinical | | | |
| | Columbia University | Small Molecule | | | Preclinical | | | |
| | University of Miami | Small Molecule | | | Preclinical | | | |
| | Gladstone Institute (University of California, San Francisco) | Gene Therapy (CRISPR) | | | Preclinical | | | |
| | Columbia University | Animal Model | | Laboratory | | | | |
| 4 | 4C - Cyprus Institute | Gene Therapy | | | Preclinical | | | |
| | 4J - Neurogene | Gene Therapy | | | Preclinical | | | |
| ALL TYPES | Psychogenics + Cleveland Clinic + Charles River | Preclinical Testing Partners | | | Preclinical | | | |
| | Inherited Neuropathies Consortium | Research & Clinical Tools | | | Preclinical | | | |
| | New York Stem Cell Foundation | Stem Cell Lines | | Laboratory | | | | |
| | University of Miami | Gene Identification | Discovery | | | | | |
| | Jackson Laboratories | Animal Models | | Laboratory | | | | |

STAR ALLIANCE PARTNERS

32 Research Partners
50+ Research Projects
Over \$16M in Research Funding

* Project concluded



DEMYELINATING CMT: TYPE 1, TYPE X AND TYPE 4 STAR RESEARCH DEVELOPMENTS

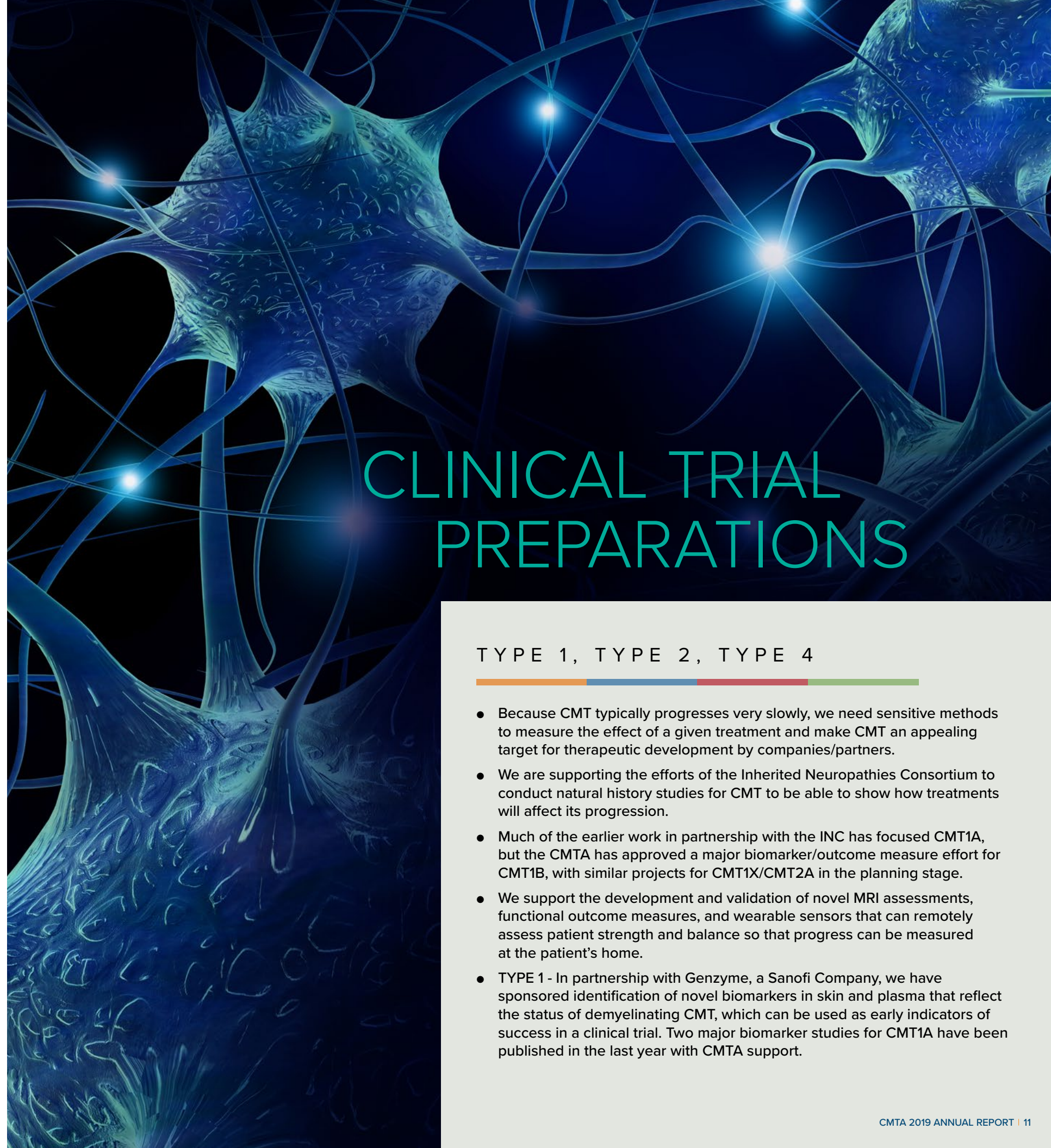
As part of this multi-pronged approach to identifying novel treatments for demyelinating CMT, the CMTA is supporting several promising therapeutic opportunities in collaboration with more than a dozen key partners:

GENETIC THERAPIES

- In collaboration with Ionis Pharmaceuticals, we are developing antisense oligonucleotides (ASOs), which have shown dramatic results in two rodent models of CMT1A.
- CMTA-funded studies by Dr. Kleopas Kleopa of the Cyprus Institute of Neurology and Genetics have shown that gene therapy is feasible in rodent models of CMT1X and CMT4C and there have been several interactions with gene therapy companies that are working with adeno-associated viruses (AAV), which can be engineered to deliver DNA to target cells. This approach is now being extended to use RNA interference to decrease the PMP22 levels found in CMT1A and to optimize delivery to the affected Schwann cells in demyelinating CMT.
- We are currently collaborating with one company to use CRISPR (genome editing) to treat demyelinating CMT, and additional collaborations with leading labs are underway.

DRUG DEVELOPMENT

- In partnership with InFlectis BioScience, we are developing agents to restore myelin protein balance for CMT1A and CMT1B. Phase 1 clinical trials have concluded and InFlectis is gearing up for Phase 2 trials.
- The progression of all types of CMT occurs as the longest axons are compromised in a process called axon degeneration. We are working with partners to develop chemical inhibitors of the triggers of axon degeneration. We are currently testing the applicability of this approach to multiple models of CMT, collaborating with several companies, including Regenacy Pharmaceuticals, on candidate drugs to promote axon survival and preserve nerve function.
- We are supporting work done by Dr. Maurizio D'Antonio of the San Raffaele Scientific Institute to test new drug classes for CMT1B, which are being developed for stress-related disorders such as stroke, Alzheimer's and retinal degeneration.
- We are supporting work at the University of Wisconsin and Memorial Sloan Kettering to identify new targets for drug development in CMT1A.
- We supported Acceleron Pharmaceuticals' efforts (since ended) to strengthen affected muscles in individuals with demyelinating CMT.



CLINICAL TRIAL PREPARATIONS

TYPE 1, TYPE 2, TYPE 4

- Because CMT typically progresses very slowly, we need sensitive methods to measure the effect of a given treatment and make CMT an appealing target for therapeutic development by companies/partners.
- We are supporting the efforts of the Inherited Neuropathies Consortium to conduct natural history studies for CMT to be able to show how treatments will affect its progression.
- Much of the earlier work in partnership with the INC has focused CMT1A, but the CMTA has approved a major biomarker/outcome measure effort for CMT1B, with similar projects for CMT1X/CMT2A in the planning stage.
- We support the development and validation of novel MRI assessments, functional outcome measures, and wearable sensors that can remotely assess patient strength and balance so that progress can be measured at the patient's home.
- TYPE 1 - In partnership with Genzyme, a Sanofi Company, we have sponsored identification of novel biomarkers in skin and plasma that reflect the status of demyelinating CMT, which can be used as early indicators of success in a clinical trial. Two major biomarker studies for CMT1A have been published in the last year with CMTA support.



AXONAL CMT: TYPE 2 STAR RESEARCH

One of the most exciting areas of progress in Type 2 research has been the development of gene therapy for spinal muscular atrophy (SMA), which affects the same motor neurons that are affected in CMT2.

Scarlett smiles for the camera with her mom

GENE THERAPY

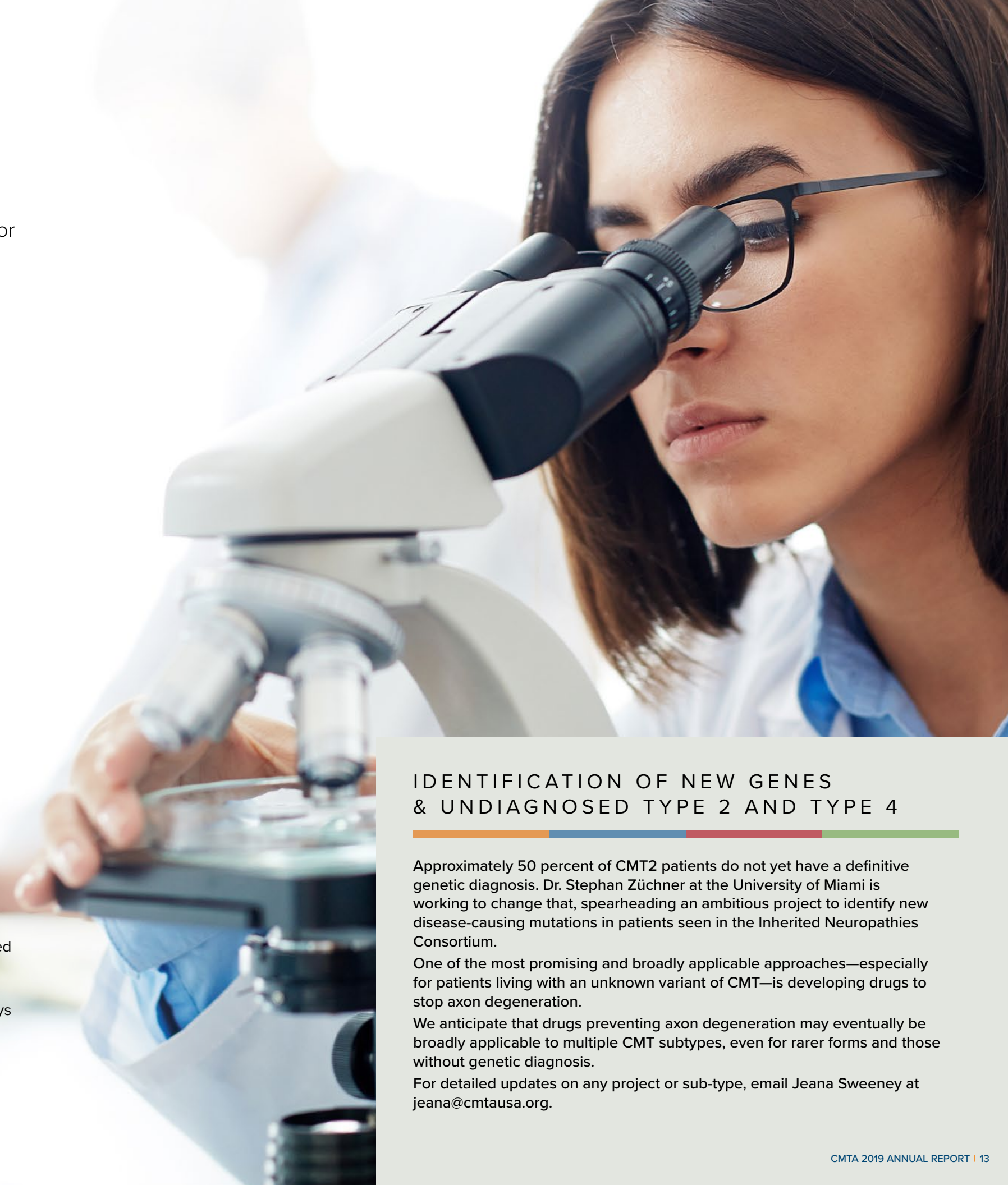
The CMTA is:

- Supporting pilot studies of gene therapy in CMT mouse models after convening a meeting of gene therapy experts to outline the next steps in bringing this therapeutic strategy to CMT2.
- Recruiting leaders in the gene therapy field to our Scientific Advisory Board to guide our efforts in this area.
- Following a gene therapy trial for one peripheral neuropathy (GAN) at the National Institutes of Health. Parallel initiatives are also underway for CMT4J and CMT2S, and projects for several additional types of axonal CMT are in the planning stage.
- Partnering with Dr. James Wilson at the University of Pennsylvania and Passage Bio to use gene therapy to treat CMT2A.
- Funding work with two eminent experts, Drs. Bruce Conklin and Luke Judge of the Gladstone Institutes and UCSF Departments of Medicine and Pediatrics, to explore applying the new technology of genome editing (CRISPR) to CMT2A, CMT2E and CMT2F.

SMALL MOLECULE THERAPIES

As noted above, the progression of all types of CMT occurs as the longest axons are lost in a process called axon degeneration.

- CMTA partners are working on developing chemical inhibitors of recently identified biochemical triggers of axon degeneration. The CMTA plans to explore the applicability of this recent technology to multiple forms of CMT2 using the many models of CMT that have been generated within our network.
- The most common cause of CMT2A is mutation of the Mitofusin 2 gene. Researchers have recently identified custom-designed molecules that can stimulate the activity of mitofusin proteins and we are discussing with them the possibility of testing this therapy in our recently developed rat models of CMT2A.
- We are funding Dr. Mario Saporta's work at the University of Miami using human stem cells to develop assays and test additional libraries of drugs for treatment in CMT2E.
- We are supporting work in Dr. Ron Liem's lab at Columbia University to screen already FDA-approved compounds to treat CMT2E.



IDENTIFICATION OF NEW GENES & UNDIAGNOSED TYPE 2 AND TYPE 4

Approximately 50 percent of CMT2 patients do not yet have a definitive genetic diagnosis. Dr. Stephan Züchner at the University of Miami is working to change that, spearheading an ambitious project to identify new disease-causing mutations in patients seen in the Inherited Neuropathies Consortium.

One of the most promising and broadly applicable approaches—especially for patients living with an unknown variant of CMT—is developing drugs to stop axon degeneration.

We anticipate that drugs preventing axon degeneration may eventually be broadly applicable to multiple CMT subtypes, even for rarer forms and those without genetic diagnosis.

For detailed updates on any project or sub-type, email Jeana Sweeney at jeana@cmtausa.org.



Whitney Wright meeting others with CMT for the first time at the Vermont Cycle 4 CMT

COMMUNITY POWERED

BRANCHES

The CMTA added five new branches in 2019, bringing the total to 70. The branches—volunteer-driven support and education groups—are a vital component of the CMTA’s education and awareness mission. Guest speakers provide education about the disease and best practices for therapeutic intervention to help manage disease symptoms.

The new branches are located in Jacksonville, Florida; Newbury, New Hampshire; Syracuse, New York; Tulsa, Oklahoma; and Southwestern Virginia. The new branch leaders hit the ground running by building their branches, planning fun and informative meetings and taking time to support the CMT community in their areas. Two of the new branch leaders—Tim Nightingale in Florida and Jacinta Gantz in New Hampshire—expressed surprise at the number of people (aka potential members) in their vicinity who have CMT. According to Tim, they share a thirst for knowledge and information about the disease.

Syracuse Branch Leader Mike Casey, who held both his first meeting and his first fundraiser in 2019, said he was most surprised by the turnout for his branch’s walk, which raised almost \$4,000 and made him feel that “We are NOT alone.” One of those people was a 6-year-old with purple braces who had never seen another human being wearing AFOs before meeting Mike.

Karen Brown from Southwestern Virginia said that while she was nervous about leading a new branch all by herself and imagined she’d be begging people to help her, the complete opposite was true, a “welcome and wonderful surprise.” Members were “all-in” with helping grow the branch and planning a fundraising event. “I did not anticipate such willing help and great ideas right away and from such small numbers, but my people are as excited as I am to have a regional presence, and I have a real team already,” she says.



*Jasmine and Vanessa Gallion
at the Oxford Funathlon*



The CMTA is one of the few national non-profit organizations with a walk campaign organized and led solely by volunteers. The CMTA's Walk 4 CMT campaign has grown exponentially since the first walk took place 11 years ago. In 2019, the CMTA's amazing volunteers stepped up to organize walks in 32 cities in 22 states and two countries, raising \$332,500 for STAR research.

Walk leaders not only make a personal difference, they give others the opportunity to join a community and reach out to their networks to share their CMT stories, spread awareness about CMT and raise funds for research. The CMTA makes it simple to host a walk with a Walk4CMT.org website, turnkey templates for sponsorship and marketing and support from our dedicated staff.

Boston Branch Leader Jill Ricci, who also organized the Boston Walk 4 CMT in 2019, said, "The CMTA makes it very easy ... providing T-shirts and goodies for everyone to enjoy." Walk 4 CMT events are not just fundraisers, they empower local CMT communities to be a part of something bigger—moving CMT research forward. Jill affirms, "A Walk 4 CMT gives everyone a chance to be part of a bigger picture—to have a hand in helping in the fight to END CMT."

2019 WALK 4 CMT HIGHLIGHTS

HIGHEST ATTENDANCE AT A WALK 4 CMT: 311 people at the Parkland, Florida

MOST RAISED BY A SINGLE WALK 4 CMT: \$41,061 by Washington, D.C.

MOST RAISED BY A WALK 4 CMT TEAM: \$11,536 by Team Rustici

TOP FUNDRAISER: Molly Fernandes (\$9,023)

8 NEW WALK 4 CMT EVENTS: San Diego, CA; Denver, CO; Miami, FL; Long Island, NY; Syracuse, NY; Columbus, OH; Greenville, SC; Houston, TX

STATES WITH MOST WALKS: Florida and California, with three each

1,836 PARTICIPANTS NATIONWIDE

22 WALKS RAISED OVER \$5,000 and 14 WALKS RAISED OVER \$10,000



CMTA ONLINE COMMUNITY

By the end of 2019, the CMTA had a total of 44,836 community members connecting with each other and the CMTA through social media. During the year, 1,510 new people became fans of the CMTA family, bringing the total number of our Facebook followers to 36,611. On Instagram we gained 1,338 followers in 2019, bringing the total to 4,119.

CMT Awareness Month was an exciting and memorable celebration of community in 2019, as well as a successful educational event that made a big impact on the CMTA's social networks and beyond. The 2019 theme was "See CMT!" illustrated with a pair of orange CMTA glasses symbolizing that many of the symptoms and challenges that come with having CMT are invisible from the outside.

The glasses were featured in the Facebook frame supporters used to deck out their profile pictures and get in the Awareness Month spirit. During the month, the CMTA issued weekly challenges, including uploading the Facebook frame, sharing a "What is CMT?" infographic, participating in a Walk 4 CMT and supporting CMT research. Community members participated in all of the challenges and made a huge wave on social media. On Facebook alone, CMT Awareness Month posts received 37,686 hits and 3,448 shares.

Rare Disease Day – February 28 – also drew throngs of supporters to Facebook in 2019: our Rare Disease Day-related Facebook posts reached a total of 169,873 people, and the "Charcot-Marie-Tooth disease is...." infographic was shared on Facebook 2,591 times.

Capping off its 2019 social media efforts, the CMTA launched a newly designed website in June with enhanced content that allows patients, families and researchers to navigate with ease through the information provided. Major topics include crucial areas like Understanding CMT, Living with CMT, Research, Getting Involved and Why to Give. The new website garnered nearly 353,000 page views in just six months.

ONLINE HIGHLIGHTS

NEW WEBSITE GARNERED NEARLY 353,000 PAGE VIEWS IN JUST SIX MONTHS

44,836 SOCIAL MEDIA FOLLOWERS

1,510 NEW FANS ON FACEBOOK

1,338 NEW FOLLOWERS ON INSTAGRAM

CMT AWARENESS MONTH POSTS DREW 37,686 ENGAGEMENTS

RARE DISEASE DAY-RELATED FACEBOOK POSTS REACHED A TOTAL OF 169,873 PEOPLE



YOUTH COUNCIL (left to right), Anthony Herrera, Evan Zeltsar, Sam Docker, Riley Williams, Emmily Stufflet, Erin Black, Meghan McClung, Paola Martinez, Sean Tyree and Jaden Ellman

YOUTH COUNCIL 2019

Recognizing that the youth of our community represent the future, the CMTA formalized their role in 2019 by appointing a Youth Council. The main goal of the CMTA Youth Program is two-fold: 1) to connect the youth of the CMT community with one another, providing them support, encouragement, understanding and inspiration from other youth and 2) to give the youth of the CMT community a voice—a chance to introduce the youth to the community in their own words and, in the process, nurture future leadership of the CMTA.

One of the Youth Council's first efforts was the establishment of a Facebook database, which quickly grew to 334 followers who averaged 161 profile visits per week. Three-quarters are female and 35 percent fall in the age range of 18 to 24.

Camp Footprint, the CMTA's premier youth program, continued to grow and thrive in 2019, increasing its numbers from 36 campers and 18 staffers in its inaugural year in 2016 to 89 campers and 60 staffers in 2019.

Camp Footprint is the place where kids who have never met another kid with CMT find their tribe. Given that just one in 2,500 people has CMT, chances are that if you're a kid in a small town, you've never met anyone outside of your family who shares your disease—as well as your hopes and fears and methods of coping.

Camp Footprint—the only U.S. sleepaway camp solely for kids with CMT—changes that equation, giving campers the rare opportunity to connect with their peers while mastering their environment, participating in activities planned just for them and celebrating their abilities.

Find out more about the CMTA Youth Program at: cmtausa.org/get-involved/cmta-youth-program

Jenna Hyman slip sliding away at Camp Footprint



2019 BY THE NUMBERS

**Charity Navigator - 4 Stars,
Highest Possible Rating**

\$2,245,207 spent on STAR

**87% of every dollar donated
spent on mission**

\$916,701 raised by the board

11,565 donations from community

50+ research projects

32 STAR alliance partners

**99% of CMT patients covered
by an active research project**

\$332,500 raised by Walks 4 CMT

32 Walks for CMT

70 branches

192 branch meetings

3 Patient/Family Conferences

331 attendees at Patient/Family Conference

89 campers

60 camp counselors

353,000+ page views on cmtausa.org

44,836 social media followers



CMTA CENTERS OF EXCELLENCE

The CMTA's Centers of Excellence are the link between the researchers and clinicians looking for a treatment and the patients who need it. In addition to testing and treating patients, the centers provide invaluable information to researchers, 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 FIVE CENTERS OF EXCELLENCE THAT CAME ON BOARD IN 2019 ARE:

- Nationwide Children's Hospital (Columbus, OH)
- Kane Hall Barry Neurology (Bedford, TX)
- Rush University (Chicago, IL)
- University of California San Francisco (San Francisco, CA)
- Washington University (St. Louis, MO)



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.
- Ken Cornell, C.O.
- 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
- 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
- Evan Zeltsar, Youth Council

STAR ADVISORY BOARD

The CMTA's STAR Advisory Board includes 29 of the top CMT scientists from around the world. 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. | Lars J. S. Knutsen, Ph.D. |
| Robert H. Baloh, M.D., Ph.D | Jun Li, M.D., Ph.D. |
| Robert Burgess, Ph.D. | Rudolph Martini, Ph.D. |
| Joshua Burns, Ph.D. | Michael McDermott, Ph.D., Consultant |
| Maurizio D'Antonio, Ph.D. | Klaus-Armin Nave, Ph.D. |
| M. Laura Feltri, M.D. | Davide Pareyson, M.D. |
| Richard Finkel, M.D. | Brian Popko, M.D. |
| Steven Gray, Ph.D. | Mario Saporta, M.D., Ph.D. |
| Scott Harper, Ph.D. | Steven S. Scherer, M.D., Ph.D. |
| David Herrmann, M.D. | Claes Wahlestedt, M.D., Ph.D. |
| Tage Honore, Ph.D. | Lawrence Wrabetz, M.D. |
| Christopher Klein, M.D. | Stephan Züchner, M.D., Ph.D. |
| Kleopas Kleopa, M.D. | |



CMTA BOARD OF DIRECTORS

The CMTA's Board of Directors is a dedicated group of professionals of diverse backgrounds, including business owners, executives, doctors and lawyers. Their backgrounds and experience give them the expertise to oversee the organization's operations and strategy, including the CMTA's research initiative, the Strategy to Accelerate Research.

What makes the CMTA board special, though, is that each member is directly impacted by the disease. This gives them an intimate understanding of what will enhance the lives of people with CMT, the ultimate goal of every decision they make.

Collectively, board members contributed or raised **\$916,701** to support the CMTA's mission in 2019.

Gilles Bouchard, Chairman
 Gary Gasper, Treasurer
 Herb Beron, Secretary
 Dan Chamby
 David Coldiron
 Thomas W. Dubensky, Jr., Ph.D.
 Laura Fava
 Alan Korowitz
 David Norcom

Steve O'Donnell
 Chris Ouellette
 Elizabeth Ouellette
 Kevin Sami
 Phyllis Sanders, Esq.
 Steven Scherer, M.D., Ph.D.
 Michael Shy, M.D.
 John Svaren, Ph.D.
 Special Advisor to the Board Bruce Chizen

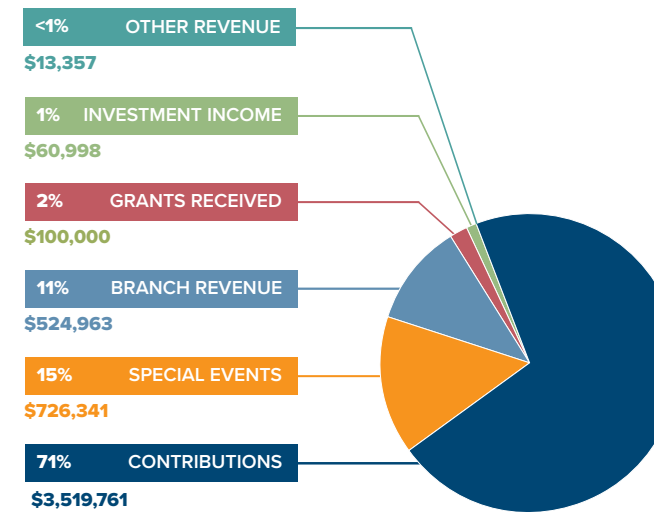
CMTA STAFF

At every level, the CMTA is lucky to have the best and the brightest working together to advance its mission. 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.

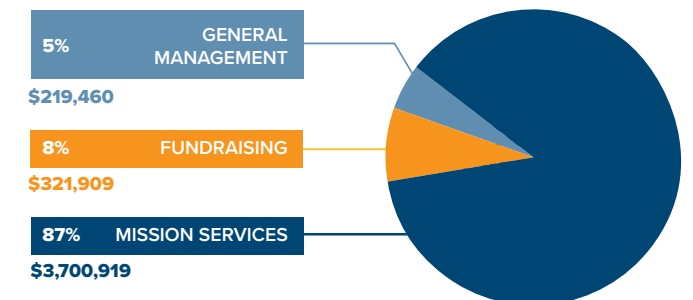
Amy Gray, *Chief Executive Officer*
 Andi Cosby, *National Events Manager*
 Leslie Nagel, *Marketing Coordinator*
 Kim Magee, *Director of Finance and Administration*
 Laurel Richardson, *Director of Community Outreach*
 Jeana Sweeney, *Director of Development*
 Jonah Berger, *National Youth Programs Manager*
 Elizabeth Ouellette, *Board Member and Staff Volunteer*

CONSOLIDATED STATEMENT OF FINANCIAL POSITION DECEMBER 31, 2019

TOTAL REVENUE
\$4,945,420



TOTAL EXPENSES
\$4,242,288



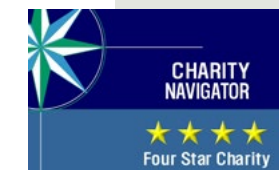
MISSION EXPENSES

TOTAL MISSION EXPENSES - \$3,700,919



RESEARCH
 74% - \$2,741,010

PATIENT & FAMILY PROGRAMS
 26% - \$959,909



Charity Navigator is the nation's largest and most-utilized evaluator of charities. A four-star rating is the best a charity can attain, indicating exceptional work that exceeds industry standards and outperforms most charities in its cause.

Fitness instructor Tara Lyn Emerson (CMT2A) celebrates the Los Angeles Walk 4 CMT with family and friends.



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

CMTAUSA.ORG