

**ACCELERATING RESEARCH/
EMPOWERING PATIENTS**



Our investments in research rose 30 percent to \$2,930,291 in 2020.



Amy Gray
Chief Executive Officer



Gilles Bouchard
Chairman of the Board

Dear Friends,

Accelerating research and empowering patients are the polar stars of the CMTA's mission, guiding everything we do. Those guiding stars pointed our way through the pandemic so that we not only weathered the storm, we emerged—thanks to our community—in better shape than ever.

What makes our community so strong? Community members feel like they have a stake in the CMTA because they actually run much of it—leading branch meetings, organizing walks, signing up for Patients as Partners in Research and acting as camp counselors. There's a sense of ownership—and the pride that goes with it.

Community members are also proud of the research we are doing. Our Strategy to Accelerate Research (CMTA-STAR) continued to grow and expand in 2020. Our investments in research rose 30 percent to \$2,930,291 in 2020, up from \$2,245,091 in 2019. Those investments funded a multitude of projects with a plethora of partners, including big initiatives on gene therapy, small molecules and clinical trials.

The pandemic forced virtually all of the CMTA's community outreach and education activities online. Our intrepid branch leaders held meetings throughout the pandemic, inviting CMT experts to share educational talks on the many facets of CMT, from genetics and foot care to occupational therapy for CMT hands to breathing issues. The pandemic's silver lining was that we were able to reach more people with our online efforts than would have been possible IRL (in real life).

Our dedicated Board of Directors also stepped up as never before in 2020, moving their activities online without missing a beat and raising more than \$1 million to support our mission. Their efforts contributed to a record-setting total of \$5.32 million in revenue, breaking the \$5 million mark for the first time and earning Charity Navigator's coveted 4-star rating.

We think 2020 is best summed up by a quote from William Faulkner, who wrote that "man will not merely endure, he will prevail." The CMTA endured a very tough year with the pandemic, but with the help of our community, we prevailed.

With warm regards,

Amy Gray, Chief Executive Officer

Gilles Bouchard, Chairman of the Board

ACCELERATING RESEARCH

CMTA-STAR is dedicated to accelerating research into the development of new drugs and treatments for CMT by funding more grants than any other philanthropic organization. CMTA-STAR brings together the world's largest network of biotech research partners, research scientists and clinicians to work with the patient community, increasing the likelihood of finding a cure.

The CMTA has established a unique capability to develop new therapies directly with companies and to expertly test those potential therapy candidates. This allows a company interested in positioning a therapy for CMT to access the infrastructure needed to evaluate the therapy without committing significant time and money up front.

CROSS-TYPE INITIATIVES

Nerves are bundles of many nerve fibers, most of them wrapped in myelin.

Myelin is an insulating and protective coating, formed by Schwann cells, which also makes nerve impulses much faster (from 1 to >50 meters/second). Myelin problems cause demyelinating CMTs (CMT1). Problems with nerve fibers, or axons, cause axonal CMT (CMT2). Type 4s can be either.

Mutations in more than 100 different genes cause CMT neuropathies. The mutations have diverse cellular functions, resulting in many disease mechanisms.

Mutations in genes expressed by Schwann cells mostly cause demyelinating CMT, though eventually this damages the axons as well. Mutations in genes expressed in nerve cells and their axons mostly cause axonal types of CMT.

GENE THERAPY

Gene therapy involves the introduction of genetic material (DNA or RNA) into the cells and tissues of an individual instead of drugs or surgery.

There are several approaches to gene therapy: replacing a faulty (missing or mutated) gene that causes a disease with a healthy copy of the gene; deactivating or “silencing” a mutated gene that is functioning improperly; or editing part of a mutated toxic gene using a “cut and paste” method.

How does gene therapy work? Essentially, a virus, or “vector,” delivers the therapeutic gene to the target cell and inserts the genetic material. Once the healthy gene enters the cell, it restores proper functioning.

Gene therapies have to address the disease mechanism. That means that for CMT neuropathies caused by loss of function (mostly CMT 4 and X) hygiene to restore the function (gene replacement). For CMT neuropathies with a toxic gain of function (mostly CMT 1 and 2) mechanism, we can either silence (reduce) the toxic gene or try to repair (edit) the mutation.

The CMTA sponsors gene therapy development for many types of CMT, including CMT1A, CMT1X, CMT2A, CMT2D, CMT2E, CMT2F, CMT2K, CMT4A and CMT4C, utilizing a number of different technologies, including AAV delivery, gene silencing, gene replacement, genome editing using CRISPR-Cas9 and antisense oligonucleotides (ASOs).

EFFICIENT, EFFECTIVE BIOMARKERS CRITICAL FOR CLINICAL TRIALS

Because clinical trials involve a large investment of both time and funding, many conversations with CMT pharmaceutical partners about potential therapies focus on how to design clinical trials that will quickly address a new medication's efficacy.

These companies want to see measures that can evaluate signs of success, ideally within three to six months of starting the clinical trial. A measure that works only after a year or two simply takes too long for them to make that investment.

Consequently, one of the most urgent needs in the CMT field is to find better ways to assess the dysfunction of the peripheral nerves in patients with CMT.

The CMTA was an early supporter of the Inherited Neuropathy Consortium's development of neuropathy scores for adults. They went on to develop pediatric and infant neuropathy assessments. But since CMT is a slowly progressive disease, these neuropathy scores by themselves are not sensitive enough to changes and therefore not really adequate to serve in a clinical trial as a measure of whether the neuropathy has improved.

Biomarker efforts extend across types and include a number of different studies. In London, neurologist Dr. Mary Reilly developed a biomarker that uses magnetic resonance imaging (MRI) to measure the amount of muscle mass in calves. As CMT progresses, there is a gradual replacement of some of the muscle with fat. MRI was not identified with CMTA support, but we are supporting extension of 1A studies to other types.

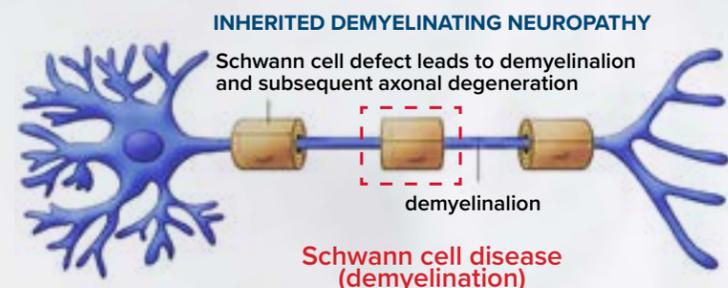
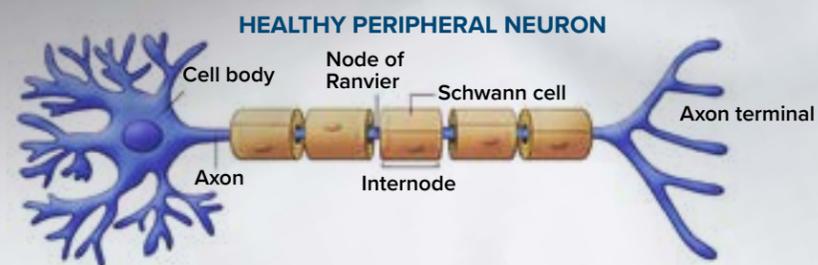
Dr. Reilly and Dr. Alexander Rossor also found that blood samples can be used to measure a protein called neurofilament light that is released from CMT nerves. Since the focus of several CMT1A therapies is reducing the expression of the PMP22 gene that causes neuropathy, the collaboration of Dr. Michael Shy at the University of Iowa and Dr. John Svaren at the University of Wisconsin has turned to the analysis of both blood samples and skin biopsies.

There are nerves present in the skin, so the affected Schwann cells—the cells in the peripheral nervous system that produce the myelin sheath around neuronal axons—can be assessed by sensitive gene detection methods to determine the level

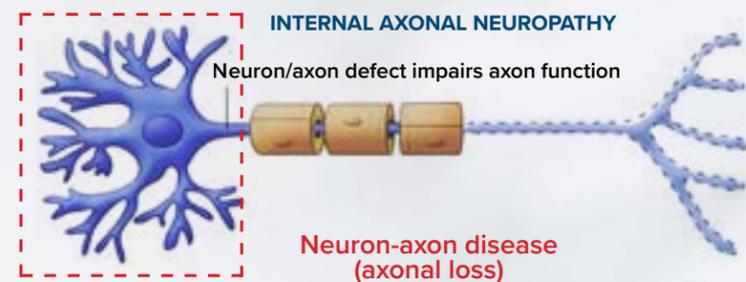
THE CAUSES OF CMT

While there are many genetic causes of CMT, certain advancements are common to virtually all types. Those commonalities include the development of gene therapies, improving genetic diagnostics and extending it to currently unclassified types of CMT, providing the biomarkers to measure the effects of treatments in clinical trials, preventing axon degeneration and developing inhibitors.

Mutations in more than 100 different genes cause CMT neuropathies. They have diverse cellular functions, resulting in many disease mechanisms.



Mutations in genes expressed by Schwann cells mostly cause demyelinating CMT - but eventually this destroys the axons as well.



Mutations in genes expressed in nerve cells and their axons mostly cause axonal types of CMT.

In some CMT types the mutation has a toxic effect (gain of function) and in other types the mutation results in loss of function.

GENE DISCOVERY

Gene discovery is another area the CMTA is pursuing. Fewer than 50 percent of CMT Type 2 patients know their gene. If the gene isn't known, there can be no therapy development and the patient is likely to be forced into an ongoing "diagnostic odyssey."

The CMTA supports the most important genomic initiative by the INC and the GENESIS project, which in 2020 discovered the most common recessive CMT2 gene—SORD neuropathy, which may be treatable with already-approved drugs. The majority of CMT genes have been discovered in the past decade in this effort

AXON DEGENERATION

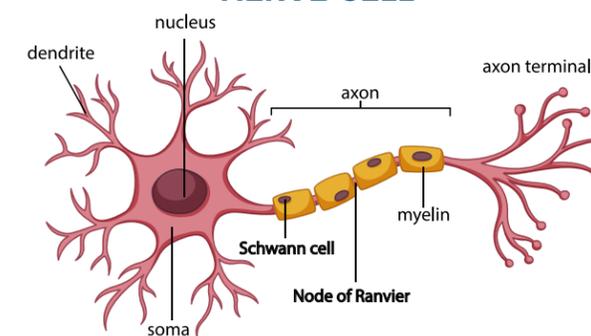
There are several genes involved in axon degeneration. Most notable is one called SARM1. The SARM1 gene codes for a protein that functions as an enzyme, affecting the levels of an important metabolite (NAD+) necessary for certain chemical processes in the body. So, what does this mean for a patient dealing with loss of neuromuscular functioning?

All nerve cells have axons whose proper functioning is essential in signaling muscles to contract. Axons are vulnerable to degeneration due to several destructive injury-induced triggers. In some types of neuropathy, a disease-induced (CMT) injury to the nerves causes inflammation, activating SARM1, which reduces the levels of axonal NAD+ and causes axonal degeneration. Inhibiting the activation of SARM1 has the potential of preventing this cascade of events from happening. Several companies are working to develop compounds that inhibit SARM1, and it is thought that this will prove to be a successful therapeutic for blocking injury-induced axonal degeneration pathways.

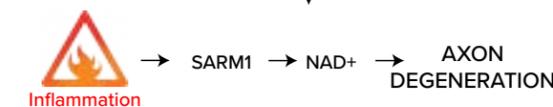
HDAC6 INHIBITORS

While the many genes associated with CMT make it unlikely that a single treatment will work for all forms of the disease, preclinical studies with HDAC6 inhibitors, which have been shown to reduce motor and sensory deficits, have demonstrated promising results in several mouse models of CMT. Based on these promising results, scientists believe that HDAC6 inhibitors might be beneficial in treating a wide array of neurodegenerative conditions including demyelinating (Type 1 and 4) and axonal (Type 2) CMTs. The CMTA recently granted Dr. Robert Burgess, a member of the CMTA's Scientific Advisory Board, \$45,000 for a study using mouse models of several forms of CMT to determine which types may be candidates for treatment with HDAC6 inhibitors and whether HDAC6 inhibitors may be of therapeutic benefit across a variety of CMT types. The latter will help determine whether patients with genetically undiagnosed cases of CMT are likely to benefit from this therapeutic strategy, or whether only select forms of CMT may respond to this treatment.

NERVE CELL



CMT INDUCED INJURY



CMTA-STAR'S PORTFOLIO FOR DEMYELINATING CMTs: 1A, 1B, 1X (AND SOME 4s)

Demyelinating forms of CMT affect the protective coating, or myelin, that insulates the “electrical wire” that is the nerve. Research specific to demyelinating forms of CMT—Types 1A, 1B, 1X and some Type 4s—includes some two dozen projects:

GENE THERAPY AND GENE EDITING PROJECTS

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 the CMTA is actively supporting the efforts of several gene therapy companies to develop new CMT gene therapies. Pioneering CMTA-sponsored preclinical gene therapy studies have shown great promise in models of demyelinating CMT. 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.

As the CMTA engages various partners to initiate gene therapy projects, it is important to keep in mind that we have a clear pathway for treatment of CMT2

type neuropathies that affect motor neurons. The recently approved AAV treatment for spinal muscular atrophy lays the technological groundwork for similar treatments for CMT2A and others. However, for CMT1A, it is not yet clear if AAV9 will be the optimal vehicle for Schwann cell delivery. Because of this, we are supporting a collaborative relationship to build on Dr. Kleopa's success in fixing Schwann cell deficits in preclinical trials for CMT1X and CMT4C. The partnership of Dr. Kleopa with an eminent gene therapy expert, Dr. Steven Gray, and an expert in Schwann cell specific gene expression, Dr. John Svaren, will address these challenges by trying multiple AAV subtypes and optimizing the vector engineering to build in the necessary safety factors and optimal administration of the AAV vector. This will allow us to target Schwann cells more precisely and move to clinical trials for CMT1A. These collaborative efforts will provide the basis of future partnerships as we engage in parallel testing of several strategies to determine which vector designs are most effective.

We are currently collaborating with one company to use CRISPR (genome editing) to treat demyelinating CMT, and additional collaborations with leading labs are underway.

SMALL MOLECULE AND BIOLOGICAL THERAPY PROJECTS

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 molecules that regulate the triggers of axon degeneration. We are currently testing the applicability of this approach in multiple models of CMT, collaborating with a number of companies to show that candidate drugs can promote axon survival, preserve nerve function and prolong patient mobility in demyelinating Type 1 CMT disorders.



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.

The CMTA has just approved two new projects to test small molecule therapies in preclinical models of CMT1A.

Most recently, the Board of Directors awarded the Feltri/Wrabetz Lab at the University at Buffalo \$138,110 for a 12-month study that will test whether drugs already approved for hypertension and erectile dysfunction can stifle the unfolded protein response of CMT1B.

PREPARING FOR CLINICAL TRIALS

In partnership with the Inherited Neuropathy Consortium, we are building on their recent successes in development of novel biomarkers and outcome measures in CMT1A and supporting major efforts to extend development and testing of critical biomarkers for CMT1B and CMT1X to support upcoming clinical trials. Toward that end, the CMTA Board of Directors awarded \$601,407 in January for a CMT1X biomarkers project that will

evaluate 60 patients over two years, measuring progression using outcome measures and biomarkers.

Clinical outcome assessments (COAs) are measures that have been developed to evaluate the clinical severity and progression of CMT over time. Biomarkers are chemicals in the body that reside in fluids like blood and tissues. Biomarkers are more sensitive than COAs, meaning that they measure changes over shorter periods of time, and therefore can more quickly and precisely measure whether a treatment or drug had a positive impact on the neuropathy.

COAs used in the CMT1X study will include:

The CMT Neuropathy Score (CMTNS) and the Examination Score (CMTES)—These measures are based on patients' symptoms, physical findings and electrophysiology. Measures include assessment of sensory symptoms as well as motor skills and strength of the arms, hands and legs.

The CMT Health Index (CMT-HI)—This is a patient-reported measure to assess therapeutic benefit in CMT clinical trials, but it may also be used to measure overall CMT patient

health. This measure is unique because it measures patients' perspectives on their mobility, foot and ankle strength, hand and finger function, and a series of related symptoms (pain, fatigue, numbness, hearing, etc.).

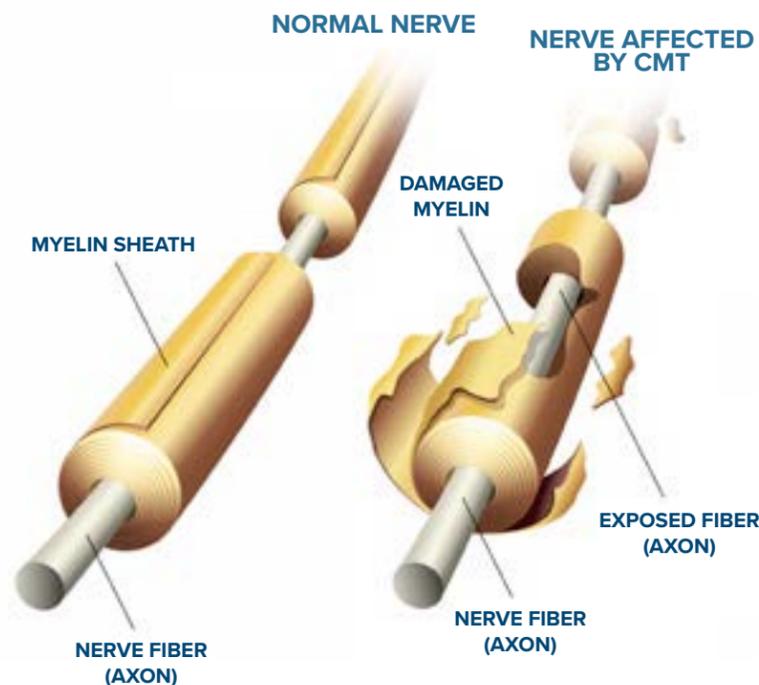
The CMT Functional Outcome Measure—This is a newer, performance-based measure that assesses the functional ability of adults with CMT. Performance measures include strength in hands and feet, lower and upper limb functioning (hand and finger dexterity), balance and mobility.

Biomarkers used in the CMT1X study will include:

MRI—the assessment of muscle, has been shown to a sensitive measure of progression in CMT1A, and this will be extended to CMT1X.

Skin Biopsies—Research shows that CMT affects expression of genes in the nerves found in skin, which can be used to measure response to treatment.

Neurofilament Light Chain—Blood plasma levels of the protein neurofilament light chain have been shown to be a marker of axonal damage as levels are elevated in CMT patients and correlate with disease severity.



Nerves are bundles of many nerve fibers, most of them wrapped in myelin.

Myelin is an insulating and protective coating formed by Schwann cells that make nerve impulses much faster (from 1 to >50 meters/second).

Myelin problems cause demyelinating CMT (CMT1). Problems with nerve fibers (i.e., axons, long extensions of nerve cells transmitting signals) cause axonal CMT (CMT2).

DEMYELINATING TYPES

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
1A	Pharnext	Small Molecule					P3
	Accelaron*	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 Partner D	Biological			Preclinical		
	University of Wisconsin	Small Molecule			Preclinical		
	University of Wisconsin	Target Validation			Preclinical		
	Jackson Laboratory	SARM1/Target Validation			Preclinical		
	Jackson Laboratory	HDAC6/Target Validation			Preclinical		
	University of Rochester, NY	Biomarkers (Wearables)			Preclinical		
	University of Wisconsin + Memorial Sloan Kettering	Target Discovery	Discovery				

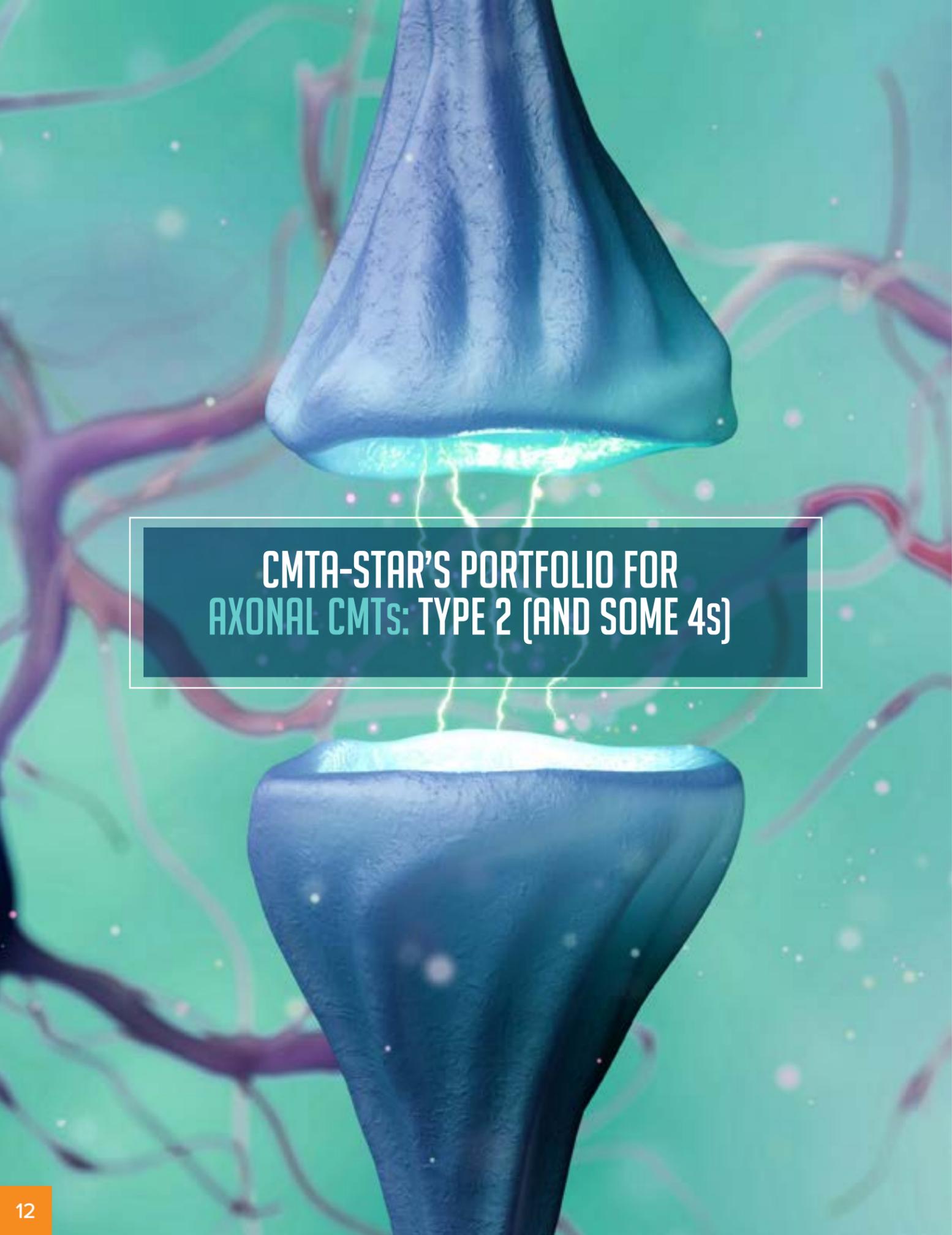
STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
1B	Accelaron*	Biological				P2	
	InFlectis BioScience	Small Molecule			P1/P2		
	National Institutes of Health*	Assays/Small Molecule			Preclinical		
	San Raffaele Scientific Institute	Small Molecule			Preclinical		
	University at Buffalo, NY	Small Molecule			Preclinical		
	Universities of Iowa and Wisconsin	Biomarkers			Preclinical		
	University of Wisconsin	Target Validation			Preclinical		
	University of Iowa	Target Validation			Preclinical		
	University at Buffalo, NY	Animal Model/Target Validation		Laboratory			
	University at Buffalo, NY	Target Discovery	Discovery				

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
1X	Accelaron*	Biological				P2	
	Cyprus Institute	Gene Therapy			Preclinical		
	University of Pennsylvania	Target Validation			Preclinical		
	Jackson Laboratory	SARM1/Target Validation			Preclinical		
	Universities of Iowa & Wisconsin	Biomarkers			Preclinical		
	University of Illinois (Chicago)	Animal Model		Laboratory			
	The Jackson Laboratory	Animal Model		Laboratory			



STAR ALLIANCE PARTNERS

32+ Research Partners
50+ Research Projects
\$17M+ in Research Funding



CMTA-STAR'S PORTFOLIO FOR AXONAL CMTs: TYPE 2 (AND SOME 4s)

Axonal forms of CMT affect the “electrical wire” that is the nerve, which is surrounded by the protective insulator coating known as myelin. Research specific to axonal CMT—Types 2 and 4—includes some two dozen projects and the CMTA Board of Directors recently approved two new ones: a gene therapy study for CMT4A, which researchers have identified as an ideal candidate for this approach, and a study on CMT2A biomarkers.

GENE THERAPY AND GENE EDITING PROJECTS

The CMTA is supporting pilot studies of gene therapy in CMT mouse models following a gene therapy trial for one peripheral neuropathy (GAN) at the NIH.

We are partnering with Dr. James Wilson at the University of Pennsylvania and Passage Bio to use gene therapy to treat CMT2A.

We are 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 therapeutic application of genome editing technology (CRISPR) to CMT2A, CMT2E and CMT2F.

Most recently, the CMTA Board of Directors awarded \$227,170 to two researchers who believe that CMT4A is an ideal candidate for potential gene therapy approaches. Noting the investigators' extensive expertise in gene therapy and viral vectors, reviewers from the CMTA's Scientific Advisory Board said the innovative project may lead to the development of clinically translatable gene replacement therapy for patients with GDAP1 (ganglioside-induced differentiation associated protein 1) mutations.

The prevalence of CMT4A is estimated at 1,000 out of every 100,000 people living with CMT. Some of the patients already enrolled in the Inherited Neuropathy Consortium's natural history project have this type, making it a potentially attractive option for a biotech company to explore. One company has already expressed some interest.

Principal investigators Steven Gray, PhD, and Xin Chen, PhD, of the University of Texas Southwestern Medical Center hypothesize that broad central nervous system (CNS)-directed delivery of GDAP1 gene with adeno-associated viral 9 (AAV9) during early life can ameliorate CMT4A disease symptoms in GDAP1 mutant mice, using an approach amenable to human translation.

Reviewers from the CMTA's Scientific Advisory Board said the clear innovative aspect of the project may lead to the development of clinically translatable gene replacement therapy for patients with GDAP1 mutations, advancing the field of gene therapy for both CMT4A and CMT2K patients.

SMALL MOLECULE AND BIOLOGICAL THERAPY PROJECTS

CMTA partners are working on developing molecules that regulate recently identified biochemical triggers of axon degeneration.

CMT2E is caused by mutations in the neurofilament light (NEFL) gene. The CMTA has a diverse portfolio of approaches for 2E.

First, 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.

Second, we supported a screen of FDA-approved compounds in Dr. Ron Liem's lab at Columbia University.

Third, the CMTA is supporting a new project to bring gene therapy to CMT2E being conducted by Dr. Kathrin Meyer and a leading gene therapy group at Nationwide Children's Hospital in Cincinnati.

UNDIAGNOSED TYPE 2

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 COEs affiliated with the Inherited Neuropathy Consortium.

PREPARING FOR CLINICAL TRIALS

In partnership with the INC, we are building on their recent successes in development of novel biomarkers and outcome measures in CMT1A and supporting major efforts to extend development and testing of critical biomarkers for CMT2A in order to support the efficient design of upcoming clinical trials.

The CMTA Board of Directors recently awarded \$559,555 for a study on identifying disease biomarkers for CMT2A, complementing the Inherited Neuropathy Consortium's cross-sectional analysis and evaluation of impairment in those patients over time (longitudinal) in those patients. Several academic centers and companies have reached out to the INC to develop clinical trials for CMT2A, which will likely be instituted within the next two to three years.

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The CMTA Board of Directors recently awarded \$559,555 for a study on identifying disease biomarkers for CMT2A.

AXONAL TYPES

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
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		
	Universities of Iowa & Wisconsin	Biomarkers			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					

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
2D	Jackson Laboratory	SARM1/Target Validation			Preclinical		
	Jackson Laboratory	HDAC6/Target Validation			Preclinical		

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
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		
	Jackson Laboratory	SARM1/Target Validation			Preclinical		
	Jackson Laboratory	HDAC6/Target Validation			Preclinical		
	Columbia University	Animal Model		Laboratory			

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
2S	Jackson Laboratory	SARM1/Target Validation			Preclinical		
	Jackson Laboratory	HDAC6/Target Validation			Preclinical		

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
4	4A – University of Texas Southwestern	Gene Therapy			Preclinical		
	4B – San Raffaele Scientific Institute	Small Molecule			Preclinical		
	4C – Cyprus Institute	Gene Therapy			Preclinical		
	4J – Neurogene	Gene Therapy			Preclinical		
	4A – Envigo	Preclinical Testing Partner		Research Tools			

STAR ALLIANCE PARTNERS	THERAPY TYPE	DRUG DEVELOPMENT STAGE					
		Discovery	Research Tools	Preclinical	Phase1	Phase2	Phase3
ALL TYPES	PsychoGenics + Cleveland Clinic + Charles River	Preclinical Testing Partners			Preclinical		
	Inherited Neuropathy Consortium	Research & Clinical Tools			Preclinical		
	New York Stem Cell Foundation	Stem Cell Lines		Laboratory			
	University of Miami	Gene Identification	Discovery				
	Jackson Laboratory	Animal Models		Laboratory			



CMTA-STAR'S PORTFOLIO FOR AXONAL CMTs - TYPE 2 (AND SOME 4s)

(continued from page 13)

However, disease biomarkers for CMT2A are needed to demonstrate biological effects of candidate therapies and to provide additional sensitive natural history data of disease progression.

Led by CMTA Board Members Drs. Michael Shy of the University of Iowa and John Svaren of the University of Wisconsin, the study will examine a number of different biomarkers, including: protein biomarkers identified in blood samples, such as neurofilament light, which can be used to measure axonal damage; RNA biomarkers identified from skin biopsies and MRI imaging of patients' legs because the accumulation of fat within muscles damaged by neuropathy can be measured very precisely.

To bring this state-of-the-art program to CMT2A (as has already been done with CMT1A, and recently approved for 1B), study authors will evaluate 60 patients with CMT2A over two years to:

- Measure progression in a combination of clinical outcome assessments, including the Rasch modified CMT Examination Score (CMTES-R), CMT Functional Outcome Scale (CMT-FOM), and patient-reported CMT Health Index;
- Measure known biomarkers like neurofilament light and identify novel plasma biomarkers;
- Adapt a nanostring platform for skin biopsy analysis to help identify patients most able to benefit from a given therapy; and
- Take repeated MRI images over a 12-month period to identify increases in intramuscular fat accumulation (IMFA) of patients' lower limbs.



PSYCHOGENICS

A KEY STRATEGIC PARTNER

From the beginning, a crucial part of the CMTA's Strategy to Accelerate Research (CMTA-STAR) has been its formation of strategic partnerships with leaders in the drug development field. PsychoGenics is one such partner.

PsychoGenics is a contract research organization, or CRO, meaning simply that it contracts with other companies to manage their trials and complex medical testing. CROs reduce the cost of research and development. They typically work with larger volumes than academic labs. They also work with smaller companies that don't have the funds to put a vivarium together. Independence is a key factor in the decision to use a CRO and some research grants actually require an independent lab.

The CMTA's animal testing program uniquely allows companies to partner with expert pharmaceutical testing service companies on studies arranged and performed under CMTA's direction and expertise. The CMTA has acquired and characterized six best-in-class mouse and rat models representing four different types of CMT so we know when to test a drug, for how long and what signifiers of improvement need to be measured. Expert contract research organizations like PsychoGenics are performing the testing under CMTA direction.

Mark Scheideler, chairman of the CMTA's Therapy Expert Board, praised PsychoGenics for its unique capabilities in neurological testing and collaborative business practices. "If not for PsychoGenics and the protocols they have in place we wouldn't have been able to continue all the pre-clinical pre-testing for our biotech partners during the pandemic. Other partners were on lockdown but PsychoGenics kept

moving forward in a year when a lot of researchers shut down."

The company is very flexible, Scheideler continued, and "retains a resiliency I've not seen in other places. It requires the ability to adapt and stay flexible. Their business side backs up their expertise and they show both commitment and flexibility."

Taleen Hanania, PhD, is the executive vice president for external scientific affairs at PsychoGenics and Mark's point person there. The company started out doing behavioral testing in 1999, then over the years added electrophysiology, translational EEG, quantitative histology, molecular biology and microdialysis. Today they do mostly preclinical testing, but they also have an internal drug discovery platform.

PsychoGenics began working with the CMTA in 2014. When a company approaches the CMTA about a drug candidate, Mark is the first point of contact. If the board makes the decision to go ahead, PsychoGenics puts together a study protocol and takes it from there. "It's a nice collaboration," Hanania said, adding "It's encouraging to see it all put together." When the pandemic hit, the company was breeding a lot of animals and had started a number of longitudinal studies that couldn't be paused. "These are very precious animals," Hanania said, and the company didn't want to waste any of them. PsychoGenics was able to go on when other companies couldn't, she said, by reducing staffing levels and housing key staff at hotels close to their New Jersey facility. They eliminated all offsite visitors, moved to shift work and tested in-house employees regularly.

CMTA-STAR ALLIANCE PARTNERS

Accelaron Pharma
ARQ Genetics
Charles River
Cleveland Clinic
Envigo
Genzyme, A Sanofi Company
HumanFirst Therapeutics LLC

Ionis Pharmaceuticals
InFlectis BioScience
NCATS (NIH)
Neurogene
New York Stem Cell Foundation
Passage Bio
Pharnext

PsychoGenics
Regency Pharmaceuticals
The Jackson Laboratory
7 Non-Publicly Disclosed STAR
Biotech Partners

CMTA-STAR ADVISORY BOARD

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University of Wisconsin

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University of Amsterdam, The Netherlands

Robert Burgess, PhD, The Jackson Laboratory

Maurizio D'Antonio, PhD, San Raffaele Scientific Institute, DIBIT, Milan, Italy

M. Laura Feltri, MD, University at Buffalo

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Tage Honore, PhD
Aestus Therapeutics Inc.

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University of Miami

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Mary Reilly, MD, CEB CO-CHAIR
National Hospital, London, England

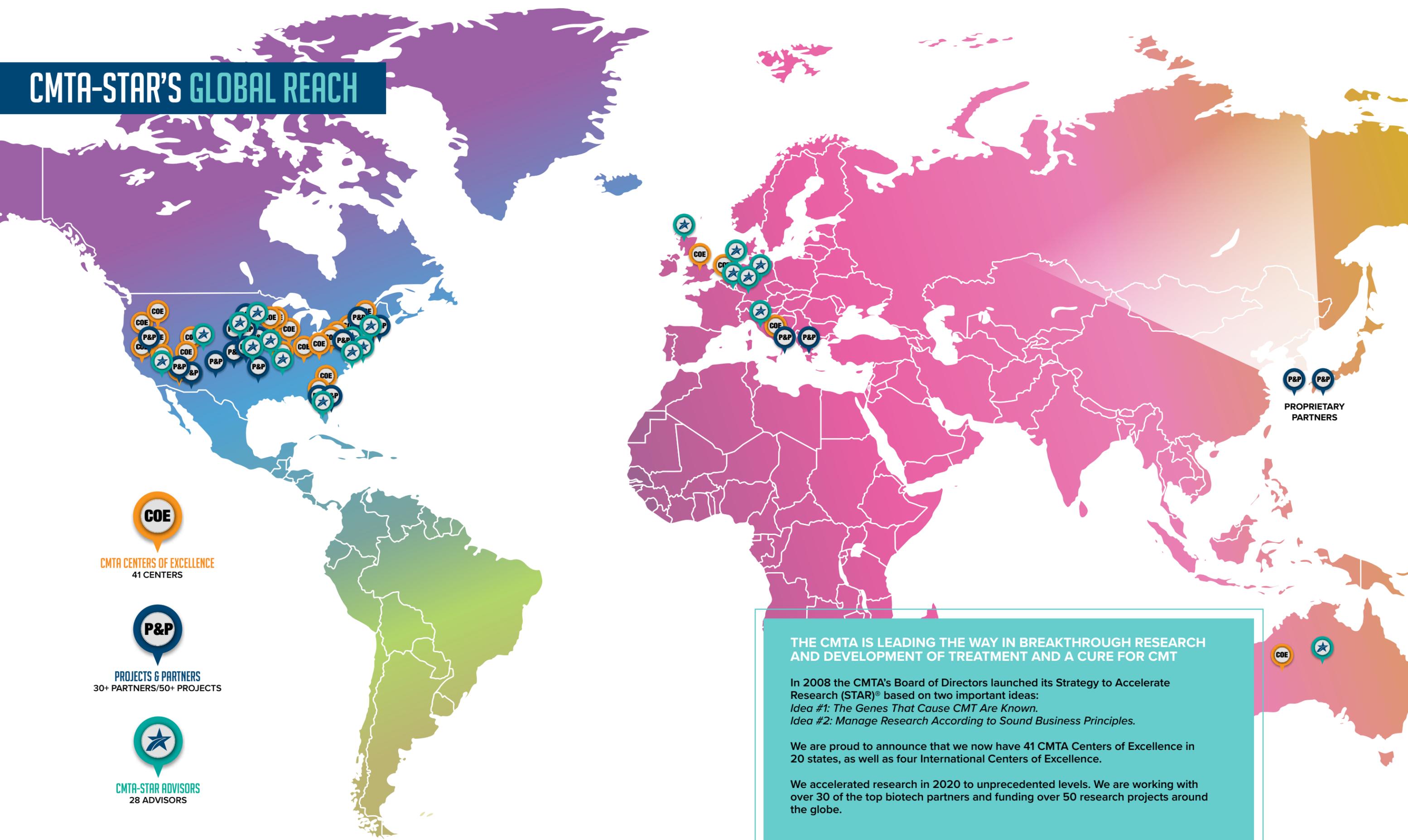
Richard Finkel, MD, Nemour's Children's Hospital, Orlando, Florida

Davide Pareyson, MD
Besta Institute, Milan, Italy

Joshua Burns, PhD
University of Sydney, Australia

Michael McDermott, PhD, CONSULTANT
University of Rochester Medical Center

CMTA-STAR'S GLOBAL REACH



CMTA CENTERS OF EXCELLENCE
41 CENTERS



PROJECTS & PARTNERS
30+ PARTNERS/50+ PROJECTS



CMTA-STAR ADVISORS
28 ADVISORS

THE CMTA IS LEADING THE WAY IN BREAKTHROUGH RESEARCH AND DEVELOPMENT OF TREATMENT AND A CURE FOR CMT

In 2008 the CMTA's Board of Directors launched its Strategy to Accelerate Research (STAR)[®] based on two important ideas:

Idea #1: The Genes That Cause CMT Are Known.

Idea #2: Manage Research According to Sound Business Principles.

We are proud to announce that we now have 41 CMTA Centers of Excellence in 20 states, as well as four International Centers of Excellence.

We accelerated research in 2020 to unprecedented levels. We are working with over 30 of the top biotech partners and funding over 50 research projects around the globe.

P&P **P&P**
PROPRIETARY PARTNERS



EMPOWERING PATIENTS

BUILDING OUT OUR ONLINE COMMUNITY



I sat down at my computer to see if crowdsourcing could help me find a knowledgeable neurologist.

- Jane Bauer -



The information I get from the CMTA Discussion Group is better than what I have found with most doctors.

- Mark Easter -



Community members responded generously to our two online fundraising campaigns: On Giving Tuesday in May, we garnered more than 18,891 video views and 2,274 engagements, raising more than \$39,098.

Community members responded to our online efforts enthusiastically in 2020, when the CMTA's social media reached—and brought together—more people than ever before. **The number of impressions—times social media posts were displayed to a viewer—increased by 72 percent, or 3.8 million impressions.** We also reached a record post engagement level: Our social media users engaged with our posts 378,302 times in 2020—an increase of 125,879 engagements over the previous year.

At the close of 2020, the CMTA had a total of 48,456 fans across our various social platforms. During the year, we gained 1,586 fans on Facebook, bringing our total to 38,197. And on Instagram, we gained 2,511 followers, raising that audience to 5,745 people.

The CMTA's Facebook groups provided a much-needed space for connection during the uncertainties of 2020. The CMTA Discussion Group, a Facebook group of more than 17,000 members, provided a welcoming and helpful community to CMTers. Jane Bauer for example, found the CMTA Discussion Group even before she was diagnosed. Knowing that her mother and nephew

had CMT, Jane thought she probably did, too. She sat down at her computer to see if crowdsourcing could help her find a knowledgeable neurologist. "That's how I found Dr. Clifton Gooch at the University of South Florida in Tampa," Jane explained. "If you go to a doctor, nobody has ever heard of CMT. You feel like you have to re-explain it every single time."

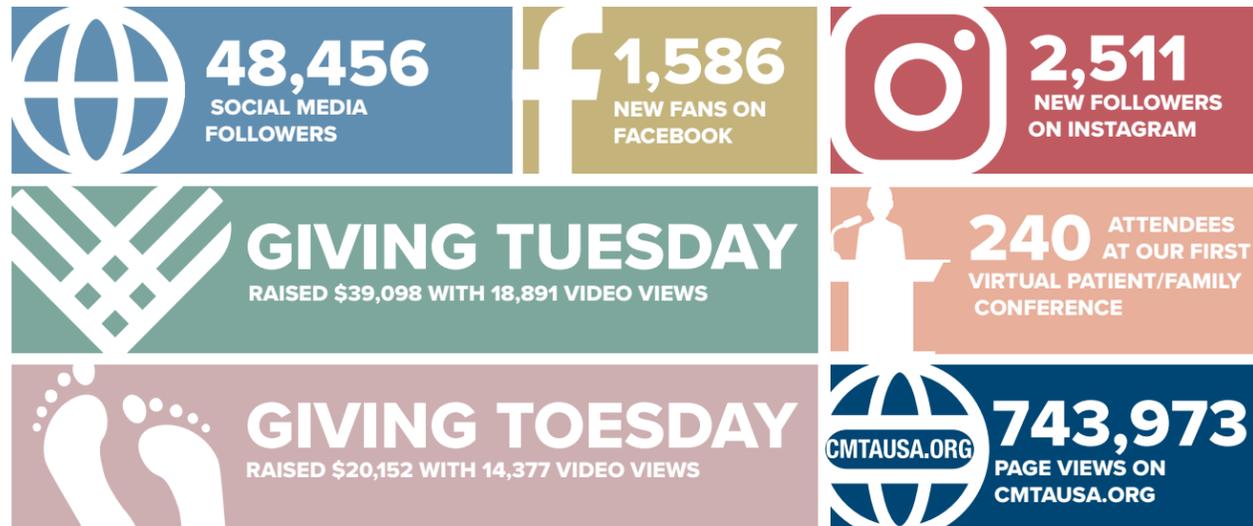
After receiving a confirmed diagnosis at age 47, Jane has continued to find the tips and advice of the CMTA Discussion Group invaluable. "People talk a lot about the things that help them—CBD oil, magnesium, etc. I'm hoping I've helped people, too" she said.

Even more importantly, the CMTA Discussion Group was where she found two things sorely missing in her life: some CMT humor and understanding. "My feet are so freezing cold at night that I put on two pairs of socks and tuck in my pajama pants, even in summer. The CMTA Discussion Group was the first time I was able to laugh about it." To Jane, the best part of being in the group is "just to know there's thousands of people who completely understand."

Mark Easter, 48, has found the CMTA Discussion Group's support and advice life changing. Diagnosed just last year with CMT Type 2, Mark knows how it feels to go most of your life thinking you might be the only one with your disease. "They call it a rare disease, and I keep seeing that one in every 2,500 people has CMT. But it feels rarer than that when you don't know what's going on, no one else can help you, and you don't know anyone with it."

Mark says the information he gets from the CMTA Discussion Group is better than what he found with most doctors. Like many people, he went to many doctors and neurologists before he was diagnosed, but never got the right answer or any answer at all. Now, in a community of others with similar issues, Mark has appreciated the opportunity to share and learn from others' experiences with everything from community that understands and "just knowing you're not crazy" makes a world of difference in his CMT journey.

2020 ONLINE HIGHLIGHTS



The CMTA put a spin on the typical Giving Tuesday, changing it up to Giving "Toesday," when talented volunteer Aron Taylor produced a rap video for the CMTA that attracted 14,377 video views and 2,661 engagements, raising more than \$20,152 in the process.



The CMTA added one new branch in 2020 (Destin, FL) bringing the total to 73 branches (including two in Canada). These support/education groups are fueled by our amazing volunteers, who went above and beyond the call of duty in 2020. When the pandemic started and quarantine left people feeling isolated, the CMTA pivoted quickly to bring the community together via Zoom.

Branch leaders didn't hesitate to move their meetings online, though they questioned, "If we build this, will they come?" The answer was a resounding "Yes." New faces, old friends, family members and even pets attended more than 80 virtual branch meetings in 2020. It was a wonderful way to stay connected during a time of isolation and a purposeful way to continue to learn more about living with and managing CMT. Plus, it was FUN!



Community member and Los Angeles Branch Co-leader John Ramos, who has participated in many virtual branch meetings, explained, "Since going virtual, I not only get to learn more about CMT, I learned more about the CMT

community and their experiences and thoughts around having CMT. Attending these meetings has taught me that none of us are alone; we just need to reach out to our CMT community."

Virtual branch meetings made it possible for anyone—anywhere—to join in the fun. Branch leaders planned meetings with CMT experts from CMTA Centers of Excellence as well as the CMTA Advisory Board. These experts updated members on the latest happenings in the CMTA-STAR research program, the best orthotics, meditation and mindfulness and exercise, to name just a few topics.

Roberta, who asked that her last name not be used, described how empowering a branch Zoom with guest speaker David Tannenbaum was for her. "As I listened to him explain how people deal with CMT all I was thinking about was he was describing me. I have never really accepted that I am disabled even though I need the help of a walker to manage everyday tasks."

Roberta says the branch meeting with David (a New York City psychotherapist and CMTA Advisory Board member) and the time with other CMT members "has taken me a step closer to finally accepting my disease. David's talk was very informative and uplifting and taught me to not allow fear to rob me of all the great things in my life."

EMPOWERING PATIENTS

VOLUNTEERS LEAD THE WAY



According to Chicago Branch Leader Doreen Pomykala, "One of the best things about being a branch leader is helping people in the community find the resources they need and also connecting branch members to each other to talk about shared experiences with CMT. Being able to have time together as a branch (on Zoom) has been invaluable." Connecting virtually has been so well received that even when it's safe to gather again, the CMTA will continue to offer a hybrid model of in-person and virtual meetings.



According to Branch Leader Mike Casey, "After a couple of weeks, I decided that if we were going to make a Virtual Walk successful, we had to keep a positive outlook. So, I used my email and was on Facebook on a very regular basis asking for donations. I got more exposure for our walk in the local newspaper, and I was featured on a local program on our ABC affiliate in Syracuse." Mike went on, "We should have a good time and gather, even during these rough times with the pandemic. I feel that a positive outlook helps with meetings and other activities. We were very fortunate this year to have the Bucks County, PA and Albany, NY Branches join us. I can't wait until next year when we can have a walk in person."

2020 ONLINE HIGHLIGHTS

Virtual Walks 4 CMT Raised \$183,496 for CMT Research

- 26 Walk 4 CMT events
- 459 participants
- 11 sites raised over \$5k, five sites raised over \$10k
- DC Walk raised a recordbreaking \$42,000
- Top Team "The Mighty Quinn" in Washington, DC Raised \$22,810



The CMTA is one of the few national non-profit organizations with a walk campaign organized and led solely by volunteers. Those volunteers hosted 26 virtual walks in 2020, raising nearly \$180,000 despite the disruptions and restrictions caused by the pandemic. Because the 2020 Walks 4 CMT were virtual, participants were not limited by time or location. Teams held smaller walks with their families and walk leaders held virtual events using Zoom and social media. Many kicked things off with a fun activity like a Bingo or trivia night, then walked for an entire month, some in smaller groups, all with proper social distancing.



The Syracuse, NY Branch kicked off its walk with a face mask fashion show on Zoom and went on to beat both their 2019 record and their goal for 2020.

Top Team "The Mighty Quinn" in Washington, DC Raised \$22,810

Any city or town can host a Walk 4 CMT—all it takes is one leader to step forward. 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.



NEW CENTERS OF EXCELLENCE

The CMTA designated three new Centers of Excellence (COEs) in 2020, bringing the total to 41. Adding to the CMTA's wide network of clinical support ensures that as many people as possible have access to expert advice and care. Like all of the centers, they are led by professionals with deep experience in treating CMT.

COEs are patient-centric, multidisciplinary CMT clinics where children, adults and families affected by CMT can be assured of receiving comprehensive care by a team of CMT experts. Many of the CMTA Centers of Excellence are affiliated with the international sites that make up the NIH Inherited Neuropathy Consortium (INC)—a group of academic medical centers, patient support organizations and clinical research resources sponsored in part by the CMTA. The centers will become even more important as the CMTA begins clinical trials, which will depend on how much we know about the “natural history” of CMT—how different types of CMT progress over time and whether novel medications are slowing the course of the disease. Much of that information will be supplied by the Centers of Excellence.

THE CMTA CENTERS OF EXCELLENCE

Many of the CMTA Centers of Excellence are affiliated with the international sites that make up the NIH Inherited Neuropathy Consortium (INC).



THE CMTA YOUTH COMMUNITY



Paola



Evan

Members of the CMTA's youth community raised more than \$17,000 for the CMTA in 2020 and look forward to keeping the momentum going in 2021. “This is the year we get the whole community of youth involved because together there is no stopping us,” the Youth Council's Fundraising Committee—Elijah Tolz, Paola Martinez and Evan Zeltsar—said in a statement.

The three are spread out on opposite coasts but share a passion for fundraising to cure CMT and are “determined and ready to encourage other youth in the community to make a difference.” Plans for 2021 include a virtual Bingo game, publication of “Walk a Mile in Our Braces,” a book charting the experiences of 75 youth with CMT, and the first-ever Global Online Dance-a-thon 4 CMT.

The council has also developed a “Fundraising E-Kit,” a presentation filled with information on the different ways to fundraise and get involved. The E-kits are downloadable and tailored specifically for situations like school fundraisers, Walks 4 CMT and birthday celebrations.



Elijah





100 campers and 59 counselors attended the 2020 Virtual Camp Footprint

For kids with CMT, Camp Footprint is the one week a year they can connect with their peers, an irreplaceable opportunity to share the hopes and fears of living with a rare neuromuscular disorder. When COVID-19 made real-life camp impossible, the CMTA decided to recreate the camp experience online. It worked better than anyone dreamed possible.

The CMTA started the only U.S. sleepaway camp solely for kids with CMT in a beautiful wooded setting outside of Pittsburgh in 2016. For four years, it was a place where kids who had never met another kid with CMT found each other in what they soon came to call The Tribe of the Funky Feet. Camp Footprint (motto: One Step at a Time) gave campers the chance to master their environment, participate in activities planned just for them and celebrate their abilities.

One hundred campers experienced all the same benefits when the fifth year of Camp Footprint migrated onto Zoom in August 2020. To an amazing extent, planners were able to replicate or find workarounds for all the campers' favorite activities. Take the traditional first-night campfire, for example. The "Camp in a Box" kit mailed to each camper contained everything necessary for a virtual bonfire: portable lanterns with a campfire setting that created a flickering light in their own personal "forts," a camp T-shirt, shaker eggs for the drum circle and a campfire mug. While planners wanted to include graham crackers, Hershey bars and marshmallows for the traditional campfire treat of s'mores, they were concerned about the items melting during shipment. Their solution? S'more Pop-Tarts.

Camp Footprint empowers everyone who participates. It empowered Reagan Leigh Breeding, 10, who was diagnosed with CMT1A at 18 months old. Back then, her mother says, there were very few opportunities to meet other children with the same symptoms and life challenges. When Reagan turned 5, she attended a week-long Muscular Dystrophy Association (MDA) summer camp. She loved it, but she was the only one there with CMT.

Reagan's mom, B. Alexandra Breeding, found Camp Footprint in 2020 and signed her daughter up. She was immediately welcomed into the Tribe of the Funky Feet by Youth Council members and CMTA leaders. When it was announced that Camp Footprint was going "virtual" due to the COVID-19 pandemic, Reagan was extremely upset. And yet, the day UPS dropped off Reagan's Camp Footprint's Camp in a Box was a day filled with excitement and wonder. Reagan was overjoyed with the items, which made her feel like she belonged because "CMT was written on everything, and that's what I have."

Breeding says Camp Footprint "left a positive, long-lasting impression with our family. Its impact on Reagan has been remarkable. I am blessed to have witnessed Reagan develop a sense of independence and self-confidence and experience belonging and acceptance among the Tribe of the Funky Feet. I saw those newfound skills carry over into the school setting and other areas in Reagan's life."

Building on the success of Virtual Camp Footprint, the youth program launched quarterly Youth Zoom Hangouts in October 2020. The Zoom hangouts give participants the chance to catch up with old friends, meet and welcome new ones and check in on life, a truly invaluable way for the youth of the CMTA to connect during the pandemic. The first three hangouts averaged 75 youth participants each.



Camper Reagan Breeding



Camper Mika Perez



Counselor Cassidy Miller

CMTA BOARD OF DIRECTORS

The CMTA's Board of Directors includes business owners, executives, doctors and lawyers, all of them dedicated to making the CMTA's vision of a world without CMT a reality. 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.

Our dedicated Board of Directors stepped up as never before in 2020, moving their activities online without missing a beat. Some board members swam for CMT. Herb Beron and Team Julia held a virtual swim, raising \$71,000 for a total of more than \$1 million in the

last 14 years. Steve O'Donnell also went virtual for his Sixth Annual Funathlon in June, swimming with his son across the Tred Avon River, then biking 20 miles to raise more than \$80,000, for a lifetime total of more than \$1 million. Steve also held a golf tournament to benefit the CMTA in September, raising an estimated \$120,000.

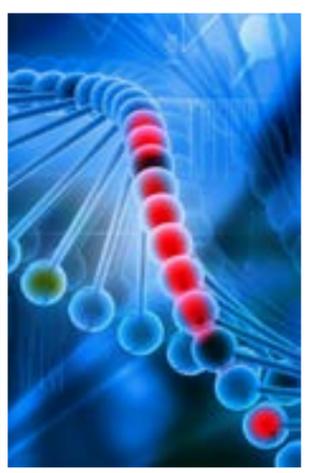
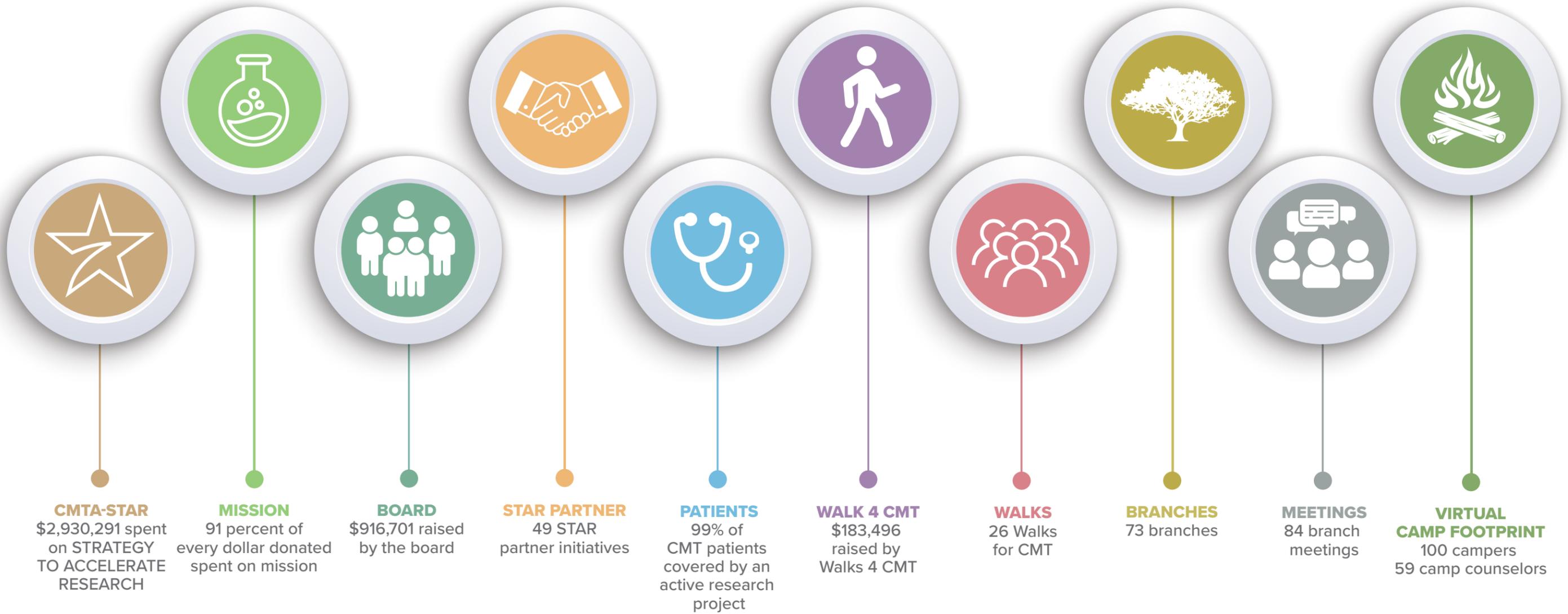
Some board members biked and walked for CMT. Chris and Elizabeth Ouellette organized the virtual Seventh Annual Vermont Cycle (and Walk!) 4 CMT, which raised over \$200,000, for a seven-year total of more than \$1.2 million. Event founder Chris also rode 300 miles in three days for the 3 million people worldwide who have CMT. Board Chair Gilles Bouchard and Thomas Dubensky biked across the Golden Gate Bridge in support of the virtual event.

Some board members held virtual fundraisers: New board member Dave Coldiron and his family held a first-year fundraiser that brought in \$32,151, and in New York, Alan Korowitz and Phyllis Sanders pivoted from their usual New York City gala to raise money through a friends and family fundraising campaign. Other board members pledged their own funds. New board member Dan Chamby and his family pledged \$1 million, including a \$200,000 match for Awareness Month. Kevin Sami, Tom Dubensky, Gary Gasper, Laura Fava and David Norcom all made personal commitments to support our mission through their generous donations.

Collectively, board members contributed or raised \$982,008 to support the CMTA's mission in 2020.



2020 BY THE NUMBERS



YOUR TEAM

BOARD OF DIRECTORS

Gilles Bouchard, *Chairman*
 Gary Gasper, *Treasurer*
 Herb Beron, *Secretary*
 Dan Chamby
 David Coldiron
 Thomas W. Dubensky, Jr., PhD
 Laura Fava
 Alan Korowitz
 David Norcom
 Steve O'Donnell
 Chris Ouellette
 Elizabeth Ouellette
 Kevin Sami
 Phyllis Sanders, Esq.
 Steven Scherer, MD, PhD
 Michael Shy, MD
 John Svaren, PhD
 Special Advisor to the Board
 Bruce Chizen

STAFF

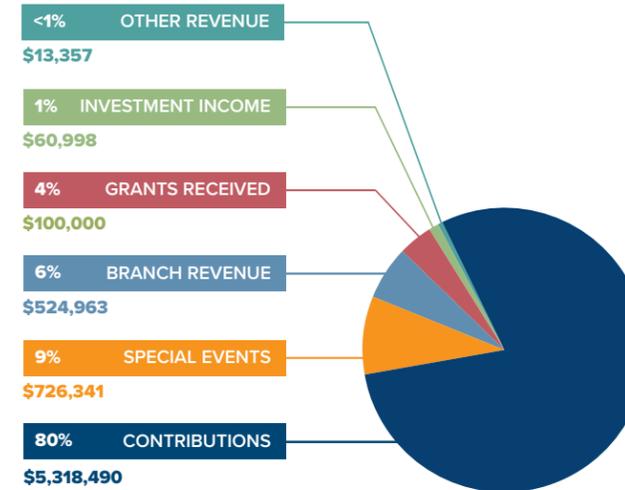
Amy Gray,
 Chief Executive Officer
 Jonah Berger
*National Youth Programs
 Manager*
 Kim Magee
*Director of Finance and
 Administration*
 Laurel Richardson
*Director of Community
 Outreach*
 Jeana Sweeney
Director of Development
 Elizabeth Ouellette
*CMTA Board Member/
 Staff Volunteer*

ADVISORY BOARD

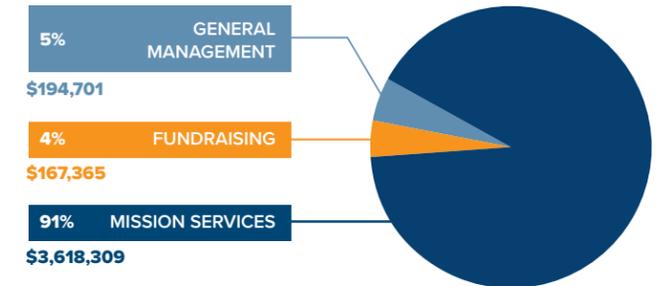
Teresa Carroll, MS, PhD
 Gregory Carter, MD, MS
 Ken Cornell, CO
 Bob DeRosa
 Katy Eichinger, PT, DPT, NCS
 Ashraf Elsayegh, MD, FCCP
 Tim Estilow, OTR/L
 Shawna Feely, MS, CGC
 Valery Hanks, OTR/L, C/NDT
 Sarah Kesty
 Kate Lair
 Bethany Noelle Meloche
 Tom Meloche
 David Misener, BSc (HK), CPO, MBA
 Elizabeth Misener, PhD, LMSW
 Christine Murray, MD
 James Nussbaum, PT, PhD, SCS, EMT
 Sabrina Paganoni, MD, PhD
 Glenn Pfeffer, MD
 Kenneth Raymond
 Clark Semmes
 Carly Siskind, MS, CGC
 Greg Stilwell, DPM
 David Tannenbaum, LCSW
 Amy Warfield, PT, DPT
 Evan Zeltsar

CONSOLIDATED STATEMENT OF FINANCIAL POSITION DECEMBER 31, 2020

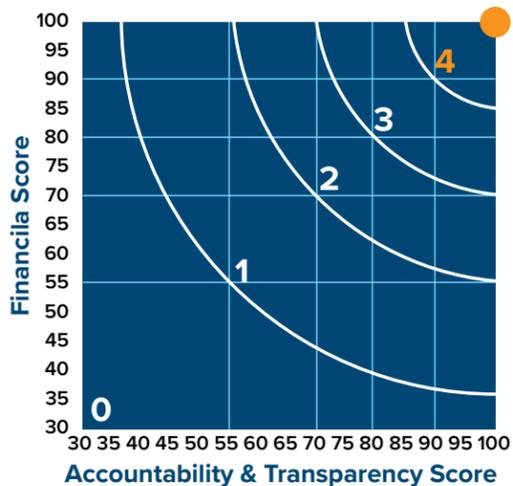
**TOTAL
 REVENUE**
\$5,318,490



**TOTAL
 EXPENSES**
\$3,980,375



Charity Navigator - 4 Stars, Highest Possible Rating



100%
 Score for Accountability
 and Transparency
99%+
 Score for Financial
 Accountability

The CMTA was the proud recipient of a 4-star, or "Exceptional" rating from Charity Navigator, which awards this ranking to organizations with cumulative scores of 90 percent. The CMTA's cumulative score was 99.51 percent, indicating that we "exceed industry standards and outperform most charities" in our space. Breaking down that score, the CMTA received a 100 percent in the category of Accountability and Transparency and a 99.31 in the Financial Category.

MISSION EXPENSES

TOTAL MISSION EXPENSES - \$3,618,309



RESEARCH
 81% - \$2,930,291

**EDUCATION
 & AWARENESS**
 19% - \$688,018



HARNESSING THE POWER OF STAR 2021 AND BEYOND

- Continue to bring new technologies, labs and companies into the CMT space
- Transition preclinical studies to clinical trial planning
- Expand new technologies to include RNAi/ASO, gene replacement, CRISPR/Cas9, etc.
- Test initiatives for axon degeneration as a potential pan-CMT treatment
- Provide support for genetic diagnostics
- Identify and validate biomarkers for fast and reliable FDA-approved trials

A FUTURE WORTH FIGHTING FOR



CMTA
Charcot-Marie-Tooth Association

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

CMTAUSA.ORG