35th Anniversary Edition

THE CMTA Report

www.cmtausa.org

CMTA
Charcot-Marie-Tooth Association

35 Years

Celebrating 35 Years of Growth, Discovery and Service to the CMT Community

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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.*

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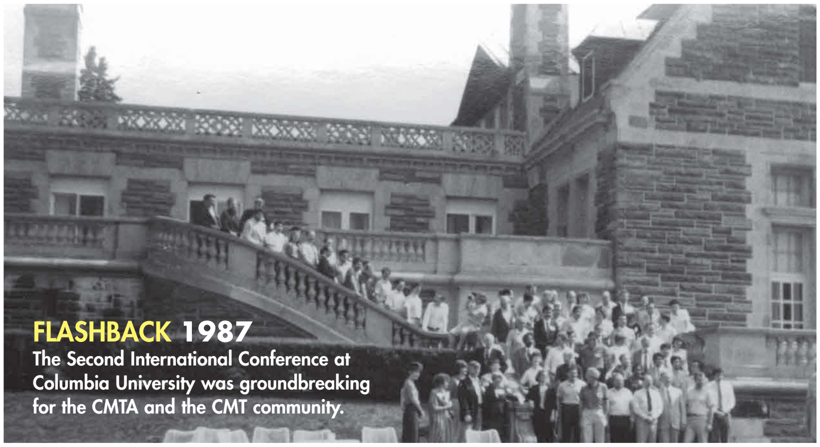
FLASHBACK 1987
The Second International Conference at Columbia University was groundbreaking for the CMTA and the CMT community.

THE CMTA Report

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DEAR FRIENDS,

35 years ago, a group of passionate individuals with CMT founded the Charcot-Marie-Tooth Association (CMTA). Like you, they wanted to ensure a better future for themselves, their families and all people living with CMT.

This year, we're celebrating our 35th anniversary. In this special edition of *The CMTA Report*, we've gone back in time and highlighted milestones that have shaped the CMTA. We've even dug into the archives of *The CMTA Report* and adapted articles that have appeared in past issues so that you can learn about our history and celebrate our bright future.

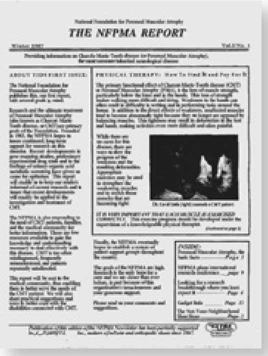
We are immensely grateful that along the way, friends like you have supported the CMTA to emerge as the leader in CMT research through STAR, our Strategy to Accelerate Research. Together, our 26 expert scientific advisors have advanced 25 cutting-edge projects just this past year alone, addressing virtually all types of CMT. We've also increased our research alliance network with pharmaceutical, biotechnology, and research service companies from two to 20 over a two-year period. We're proud to report that the CMTA has spent more than $8 million on translational or treatment-focused research through STAR in recent years, but the need remains to raise at least $10 million more to support our strategy to deliver treatments to our community.

It's clear that together, we have made significant strides in CMT awareness, patient advocacy and research through STAR—but we believe we're just getting started. We have more hope now than ever before that a treatment—a cure—for CMT is on the horizon. That's why we hope you will make your urgently needed year-end gift today. All donations designated for this year-end appeal will be matched dollar-for-dollar, up to $150,000 between now and December 31, 2018, so the time is right to give.

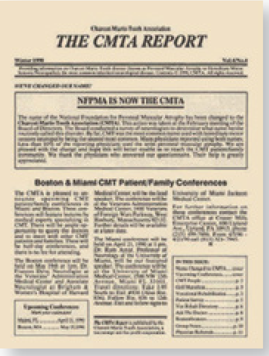
As 2018 draws to a close and the new year approaches, we're incredibly grateful to you for your support.

Amy Gray

AMY GRAY, Chief Executive Officer



THE NFPMA REPORT



THE CMTA REPORT

The CMTA Report

Our Year in Review

THE CMTA Report

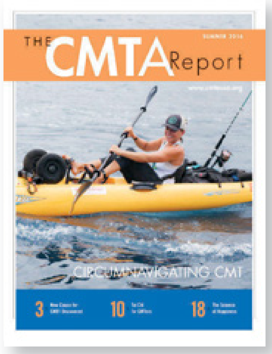
Two New Grant Recipients Announced

THE CMTA Report

ONE STEP CLOSER

THE CMTA Report

A New Year, A New Look, A New Website, A New Opportunity



THE CMTA Report

CIRCUMNAVIGATING CMT

WE DIG INTO OUR ARCHIVES…

When Howard Shapiro, PhD, convened a small group in 1982, he believed that a newsletter would be an essential component of connecting people, sharing information and raising awareness of CMT. The first NFPMA Report, now *The CMTA Report*, was published in 1987. It has had several makeovers through the years, and the current magazine format, which reaches thousands of people each year, was rolled out in spring 2016.

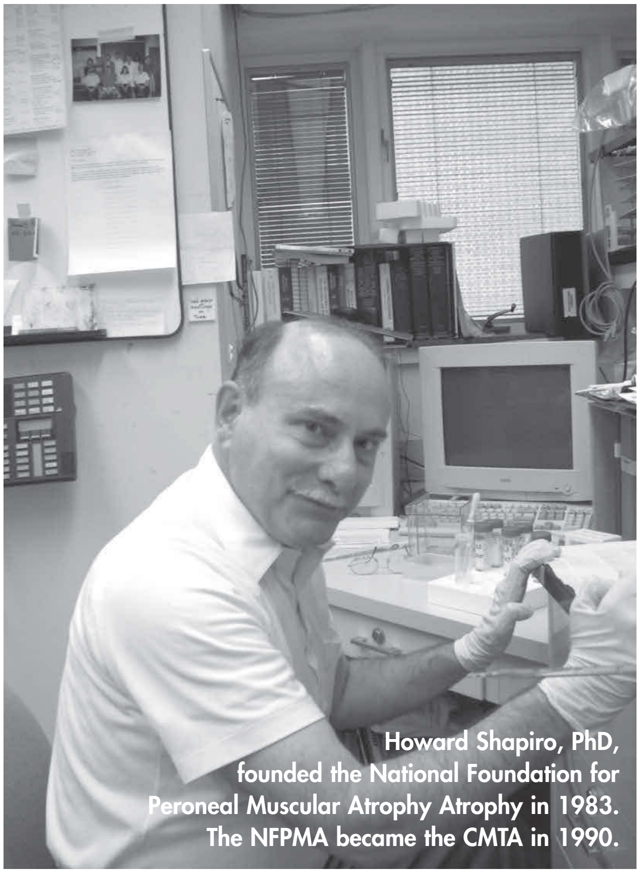
ADAPTED FROM THE CMTA REPORT – WINTER 2003

The First Gathering for People with CMT Took Place in 1983

Historic meeting paves the way for the CMTA.

It all began in an apartment in New York City. Five people with a common cause came together and laid plans for the National Foundation for Peroneal Muscular Atrophy (NFPMA), which would become the Charcot-Marie-Tooth Association (CMTA). Here's the story:

In 1982, Carolyn Redell organized the first-ever support group meeting for people with CMT. Robert Lovelace, MD, Howard Shapiro, PhD, and Redell's nephews, George and Frank Crohn, attended the meeting in Carolyn's New York City apartment. Together, they formulated plans for an organization which they hoped would raise money for CMT research while also meeting the existing need for patient self-help and education.



Howard Shapiro, PhD, founded the National Foundation for Peroneal Muscular Atrophy Atrophy in 1983. The NFPMA became the CMTA in 1990.

Carolyn Redell organized the first-ever support group meeting for people with CMT.

Dr. Shapiro had CMT, as did several members of his family. His work was instrumental in transforming the hopes of the original meeting attendees into a full-fledged national non-profit organization, the National Foundation for Peroneal Muscular Atrophy, as it was known until 1990 when it became the CMTA.

One of Dr. Shapiro's most important contributions was his organization of the Second International CMT Conference with Dr. Robert Lovelace, who went on to become the first chair of the CMTA's Medical Advisory Board. Held in 1987 at Columbia University, the conference engendered significant advancements in CMT research. Following the conference, a textbook based on the lectures given at the meeting was published, and the publication of significant research and journal articles on CMT increased dramatically. This momentum led to a number of significant breakthroughs in the CMT field, the majority of them in CMT Type 1 research.

The programs initially established by Dr. Shapiro to help patients and advance research—support groups, a newsletter (first published in 1987), patient/family seminars and research symposia—have remained vital to the misson of the CMTA to this day. In celebration of its 20th anniversary, the CMTA proudly named a grant in honor of Dr. Shapiro in recognition of his lasting influence.

ADAPTED FROM THE CMTA REPORT – FALL 1991

CMTA Advisory Board Member James Lupski, MD, PhD, Identifies Gene That Causes CMT1A

Early commitment to research drives the CMTA's progress.

Since its inception in 1983, laying the groundwork to find a cure for CMT has been at the forefront of the CMTA's mission. Fortunately, early breakthroughs by scientists involved with the CMTA provided the foundation the CMTA needed to move forward with further research.

In 1991, James Lupski, MD, PhD, a member of the CMTA Medical Advisory Board and the principal author of the research paper "DNA duplication associated with Charcot-Marie-Tooth disease type 1A," announced the identification of the gene causing CMT1A.

Historically, diagnosis of CMT had been done through family history, clinical examination of nerve and muscle function, electrodiagnostic testing and sometimes by nerve biopsy. Now the identification of a disease-causing gene allowed for a simple blood test to be used in some CMT patients and families for diagnosis.

James Lupski, MD, PhD, identified the gene that causes CMT1A, which allowed for a simple blood test to be used in some CMT patients and families for diagnosis.

Dr. Lupski and others developed the test at Baylor College of Medicine. In the patients studied, researchers found CMT is caused by a duplication of DNA on chromosome 17. According to Dr. Lupski, who has CMT, "we now have a direct diagnostic tool for one form of CMT."

In addition to providing clinicians with an important diagnostic tool, the discovery led to greater understanding of the underlying causes of CMT and promoted further investigation into the genetic causes of CMT, work that continues to help researchers develop therapies to address CMT.

Researchers first identified a gene that causes CMT in 1991, and since then, more than 100 more. Knowing which genes cause CMT helps researchers study therapies and patients better understand their disorder.

ADAPTED FROM THE CMTA REPORT – APRIL 2001

National Institutes of Health Establishes Rare Disease Clinical Research Network

Database created to address challenges in diagnosing and treating rare diseases like CMT.

In 2003, the National Institutes of Health established the Rare Diseases Clinical Research Network (RDCRN) to advance medical research on more than 190 rare diseases, including CMT, by providing support for clinical studies and facilitating collaboration, study enrollment and data sharing.

Through the RDCRN, researchers from multiple universities and organizations collaborated to create the largest repository of information on people with CMT. By conducting evaluations of CMT patients using standard procedures, many of the CMTA's Centers of Excellence now help ensure the validity of information contributed to the RDCRN by people with CMT. This benefits people with CMT in several ways.

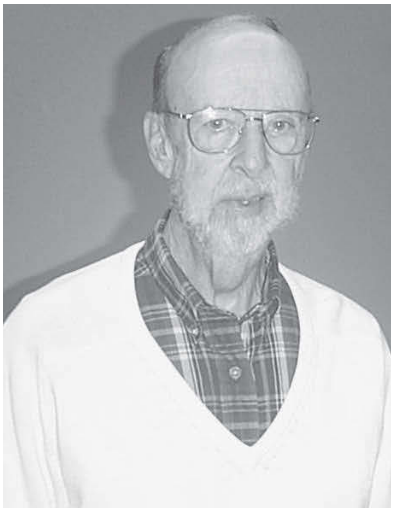
First, the information compiled in the database gives physicians and researchers a more thorough understanding of all the clinical symptoms that accompany the various types and subtypes of CMT, allowing them to say with certainty, "This is what CMT actually is."

Michael Shy, MD, is a neurologist at the CMTA Center of Excellence at the University of Iowa which contributes to the RDCRN.

Second, it provides clinicians and researchers with a better picture of the "natural history"—the severity and progression—of the various types of CMT. This knowledge also:

- Makes CMT easier to diagnose.
- Is a blessing for those who have lived—sometimes for years—with the uncertainty of whether or not they have CMT.
- Provides a better picture of how prevalent CMT actually is.
- Influences and perhaps changes the direction CMT research will take.
- Stimulates research interest and attracts more research funds.
- Speeds up the pace of CMT research by making the information in the database available to CMT researchers.

The more people who enroll in the database, the more information will be available to researchers—information that will be critically important as drug development for CMT reaches the stage where participants will be needed for clinical trials.



LEFT TO RIGHT: CMTA support group liaison Bob Budde, and his successors, Elizabeth Ouellette (current Board member and volunteer) and Jeana Sweeney (current Director of Development).

ADAPTED FROM THE CMTA REPORT – WINTER 2003 AND MARCH 2010

Community Leaders Form Support Groups for CMT Patients and Families

Groups grow into a coordinated nationwide network.

The entire CMTA grew from a single support group started in 1982. The members of that group formulated a plan for a larger organization, initially hoping to raise money for research, but also recognizing the need for a forum for patient self-help, discussions and resources. These support groups have evolved into more than 70 CMTA branches while staying true to their original mission of providing people with CMT and their families with a place where they know they are not alone in facing the challenges of living with CMT.

Laurel Richardson, Director of Community Outreach, currently coordinates the CMTA Branches.

While much has changed within the CMTA through the years, the important thing the CMTA learned from that very first support group was that allowing people with CMT to come together face-to-face can lead to great things.

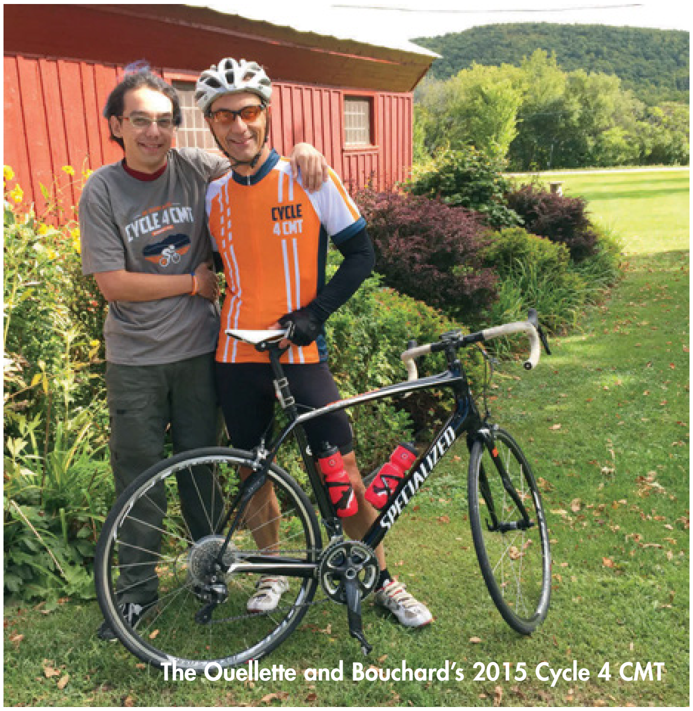
Throughout every year, people from all over North America gather to share stories, listen to expert speakers and swap ideas on how to live their best life with CMT. These groups have also become important centers for fundraising and raising CMT awareness.

In 2010, support group leaders from across the country gathered for the first time to meet each other and to learn more about the work of the CMTA and the STAR research program. The meeting included 53 leaders and co-leaders, six leading CMT physicians and several other presenters. The leaders left energized, motivated and better informed to unite the entire CMTA membership and CMT community.

According to Bob Budde, CMTA member and former support group liaison, “Different groups work in different ways, but we are all working toward the vision of a world without CMT.”

The Beron's 2015 Team Julia

2018 NYC Gala



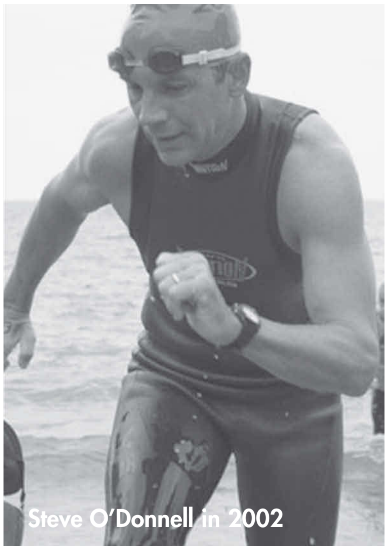
The Ouellette and Bouchard's 2015 Cycle 4 CMT

ADAPTED FROM THE CMTA REPORT – APRIL 2002

Board Members Step Up to Fund the CMTA's Mission

Leading by example, Steve O'Donnell began a fundraising tradition.

CMTA Board Member Steve O'Donnell organized the first "Swim for the Cure" in 2002. Following Steve's lead, many other board members have organized major events including Elizabeth and Chris Ouellette and Gilles Bouchard's Cycle 4 CMT, Herb and Rachael Beron's Team Julia and Phyllis Sanders and Alan Korowitz's New York City Gala. All together, these events have raised $4 million for the CMTA.



Steve O'Donnell in 2002

2018 Funathlon

ADAPTED FROM THE CMTA REPORT – JANUARY 2008

The Strategy to Accelerate Research (STAR) is Launched with the Mission to Cure CMT

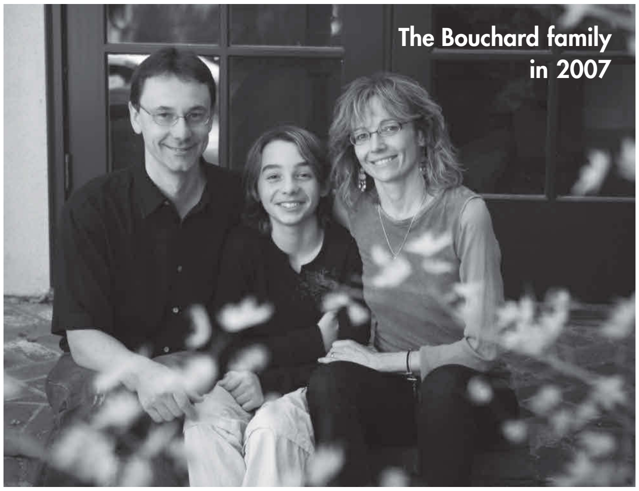
STAR program grows over a decade.

Originally called the Accelerated Research Collaboration, the Strategy to Accelerate Research was launched in 2008. In just 10 years, STAR has spent more than $8 million to finance dozens of research projects.

The genesis of STAR was in 2007 at the Second International CMT Consortium in Snowbird, Utah. Sponsored by the CMTA, the Consortium brought together researchers and clinicians from around the world. Together with the leadership of the CMTA, a group of these researchers agreed to embark upon a unique, cutting-edge approach: managing research according to sound business principles. STAR aimed to bring researchers out of their silos to collaborate with each other, holding them accountable to projects that focused on the known causes of CMT and required them to deliver results.

The Strategy to Accelerate Research also presented a huge fundraising challenge for the CMTA: $10 million would be needed to fund it. The first seed money came from a Pennsylvania couple, John and Joan Chernega, who made a $100,000 gift to the CMTA in honor of their son, Jim, who has CMT.

Gilles Bouchard, who is now chair of the CMTA Board of Directors, and his wife Elizabeth Ouellette, also a CMTA board member, also committed $500,000 to create "The Yohan CMT Research Fund," a donor-advised fund designed to support the critical research being conducted by STAR.


The Bouchard family in 2007

"Now is the time to create the financial foundation necessary to support these efforts," Gilles explained. "Creating the fund was very easy, and Elizabeth and I urge others to follow our lead. Working together, we can make a difference."

In addition to creating the fund in honor of their son, Yohan, who has CMT, Gilles and

(continued on page 10)

Remembering John Chernega

We are deeply saddened to announce the passing of our cherished friend and community member, John Chernega. John, a retired electrical engineer, his wife Joan and his entire family have been staunch supporters of the CMTA over the years, and we genuinely appreciate their dedication, kindness and generosity. The Chernega family's contributions have provided hope, resources and inspiration to individuals living with CMT, including their son, Jim. Regarding his gift to launch the CMTA's STAR initiative, John said, "We would just like to see some sort of breakthrough in finding a cure and providing new hope for patients." In John's memory, we will continue to fight relentlessly for a world without CMT. Our hearts go out to the Chernega family at this very difficult time.


The Chernega family provided the first seed money for STAR.

LAUNCHING STAR

(continued from page 9)

Elizabeth exemplify the commitment of CMTA board members who contribute their expertise to further the mission of the CMTA.

Now retired, Gilles spent the majority of his career as an executive at Hewlett Packard, after which he ran several companies in the San Francisco Bay Area, including Opnext and Livescribe. After his son Yohan was diagnosed with CMT, Gilles took a yearlong sabbatical to work closely as a volunteer with members of the CMTA Board of Directors to develop the business objectives and strategic direction for STAR.

Elizabeth, who has a master of arts in counseling psychology, developed a unique presentation to teach children and adults about the kinds of challenges facing those with CMT, and she has a decade-long history of helping organize and run CMT support groups—now CMTA branches—around the country.

ADAPTED FROM THE CMTA REPORT – NOVEMBER 2010

The STAR Advisory Board Propels CMT Research

Advisory board helps STAR shine even brighter.

The STAR program has benefited from the leadership and guidance of the world's top CMT experts. These researchers have come together to guide STAR in its goal to discover treatments—and eventually a cure—for CMT.

As the CMTA's STAR strategy has continued to facilitate breakthroughs, our commitment to research has kept pace. In 2010, 35 researchers who specialize in CMT Type 2 met in San Diego to discuss the status of their research and to make plans about which form of CMT they should collaborate on first. Several were members of the STAR Advisory Board, which, eight years later, now includes 26 of the top scientists in CMT and related fields, and is comprised of scientific, therapy expert and clinical expert boards. These advisors guide our path by providing scientific input for ongoing and proposed projects, ensuring that each research project has translational value and supporting our pharmaceutical partners to ensure the success of our clinical trials.

In 2010, 35 researchers who specialize in CMT Type 2 met in San Diego to make plans to collaborate on CMT research.

CMTA
Charcot-Marie-Tooth Association

35 Years
Serving the CMT Community

$150,000

35th Anniversary Research Challenge Match

Thirty-five years ago, a group of caring individuals with CMT founded the Charcot-Marie-Tooth Association (CMTA) because they wanted to ensure a better future for themselves and their families. Thanks to your continued support, the CMTA has become the leader in CMT research through our STAR (Strategy to Accelerate Research) program. Working collaboratively, our 27 expert scientific advisors have advanced 18 cutting-edge projects, tackling virtually all types of CMT. We've also increased our research alliance network with pharmaceutical, biotechnology, and research service companies from two to 20 over a two-year period.

We are determined to further accelerate our progress and gain more ground on all fronts. That's why we are asking you to make your urgently needed year-end gift today so we can continue our fight against the progressive and devastating effects of CMT and ultimately find a cure.

All donations designated for this urgent year-end appeal will be matched dollar-for-dollar, up to $150,000 between now and December 31, 2018!

DETACH AND MAIL TO: ***Charcot-Marie-Tooth Association PO Box 105, Glenolden, PA 19036***

Yes, double my contribution so we can continue the fight against the progressive and devastating effects of CMT.

☐ $75 ☐ $150 ☐ $300 ☐ Other: $____________

☐ I am interested in learning about leaving a legacy gift to the CMTA.

☐ Check enclosed, payable to the Charcot-Marie-Tooth Association. Donate online at www.cmtausa.org/35years

☐ Please charge my credit card. ☐ Visa ☐ MasterCard ☐ American Express

Name______________________________ Card #______________________________ Exp. Date__________

Signature______________________________ Address______________________________

City______________________________ State______________ Zip________ Phone______________________

Please send me CMTA updates via email. My email is:

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Camp Footprint Provides Fun and Friendship for Teens with CMT

Established in 2016, the CMTA's Camp Footprint is the only camp in the United States just for kids with CMT. It is a free, five-day sleepaway camp in Western Pennsylvania for kids ages 10–18.

ADAPTED FROM THE CMTA REPORT – JANUARY 2015

The CMTA Celebrates the Life of Pat Dreibelbis

Beloved staff member and friend was with the CMTA for 23 years.

Every organization has one person who seems to know everyone—and every thing—about the organization. For the CMTA, that person was Pat Dreibelbis.

As the editor of *The CMTA Report*, Pat played a crucial role in disseminating news and information to the CMT community. When publishing helpful resources for parents and children became a focus for the CMTA in 2008, Pat paved the way, creating the booklet *My Child Has CMT: A Guide for Parents of Children with Charcot-Marie-Tooth*. Pat's memory lives on through the numerous written treasures she left behind, including *The History of the CMTA* and her personal favorite, her popular stories featuring Archy the Turtle.

For Pat, the highlight of her job was the opportunity to form relationships with the people who called her asking for help, advice and feedback. Herb Beron, a CMTA board member, remembers: "Pat Dreibelbis was one of the first people I ever spoke with when I contacted the CMTA about my daughter Julia. We knew nothing about the disease, and she was a wealth of information to me and my family. Over the years, as I came to know Pat better, we became good friends. I have always considered her to be the 'matriarch' of our organization, and her sudden passing left a void within our organization that we can only hope to fill with someone of Pat's character and kindness."

Everyone who ever had the privilege of working or speaking with Pat found it a special pleasure. Her quick wit and incredible sense of humor brought smiles and laughter to many. The indelible mark she left on every layer of the CMTA and the treasures she left behind continue to be a source of inspiration and information to all.

ADAPTED FROM THE CMTA REPORT – MAY 2009

Remember Archy, the CMTA Kids Mascot?

Archy the Turtle helps parents talk to their children about CMT.

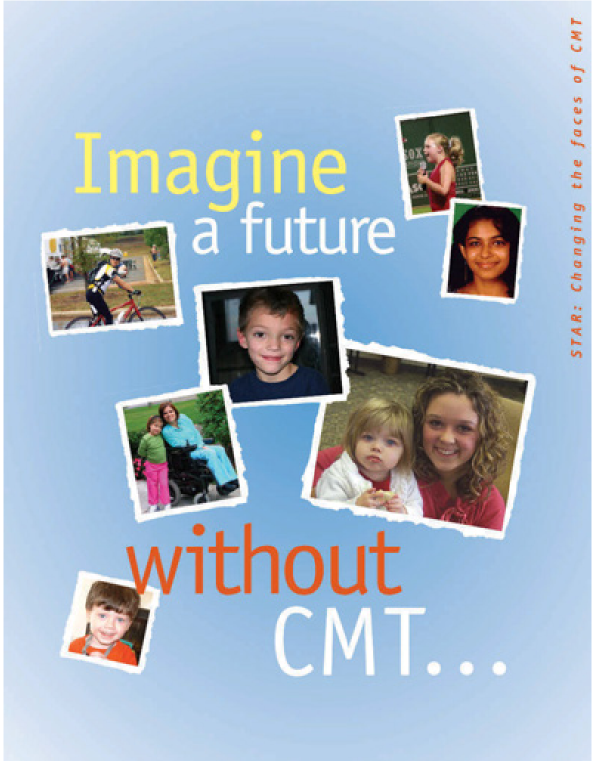
In 2008, Tina Marie Thomas created Archy, a turtle with CMT and high-arched feet. We thought he would be a great mascot for kids with CMT, and Pat Dreibelbis began writing *The Adventures of Archy*, a series of stories that explained how Archy coped with CMT in a variety of situations from going to school to going to the doctor to getting braces. Beloved by all, we're still not sure who has had the most fun with Archy—the young or the not-so-young. Another beloved member of the CMT community who is no longer with us, Jerry Cross, created the Archy Train, and Jeana Sweeney, now our Director of Development, created *The Archy Cookbook* to support proper nutrition in young patients with CMT.

ADAPTED FROM THE CMTA REPORT – NOVEMBER 2014, MARCH 2015 AND FALL 2017

STAR is Working

Research program brings breakthroughs for CMTA diagnosis and treatment.

The past 35 years have been marked by incredible advances in CMT research and knowledge. Thanks to patient support, STAR has identified promising drug candidates. Through STAR, the CMTA has formed powerful alliances and partnership agreements with the pharmaceutical, biotechnology and research service industries, major universities, other non-profit research organizations and the National Institutes of Health (NIH). Our list of partners has more than doubled in 2018 to more than 20 companies across three continents, including Genzyme (a Sanofi Company), Ionis Pharmaceuticals, Acceleron Pharma, InFlectis BioScience and Regenacy Pharamaceuticals, among others. These companies are leaders in the latest genetic and neurological technologies.



Through STAR, we can imagine a future without CMT. The faces of CMT in the March/April 2008 *CMTA Report* then, as now, remind us of the urgent need for research collaborations that deliver results.

Genzyme

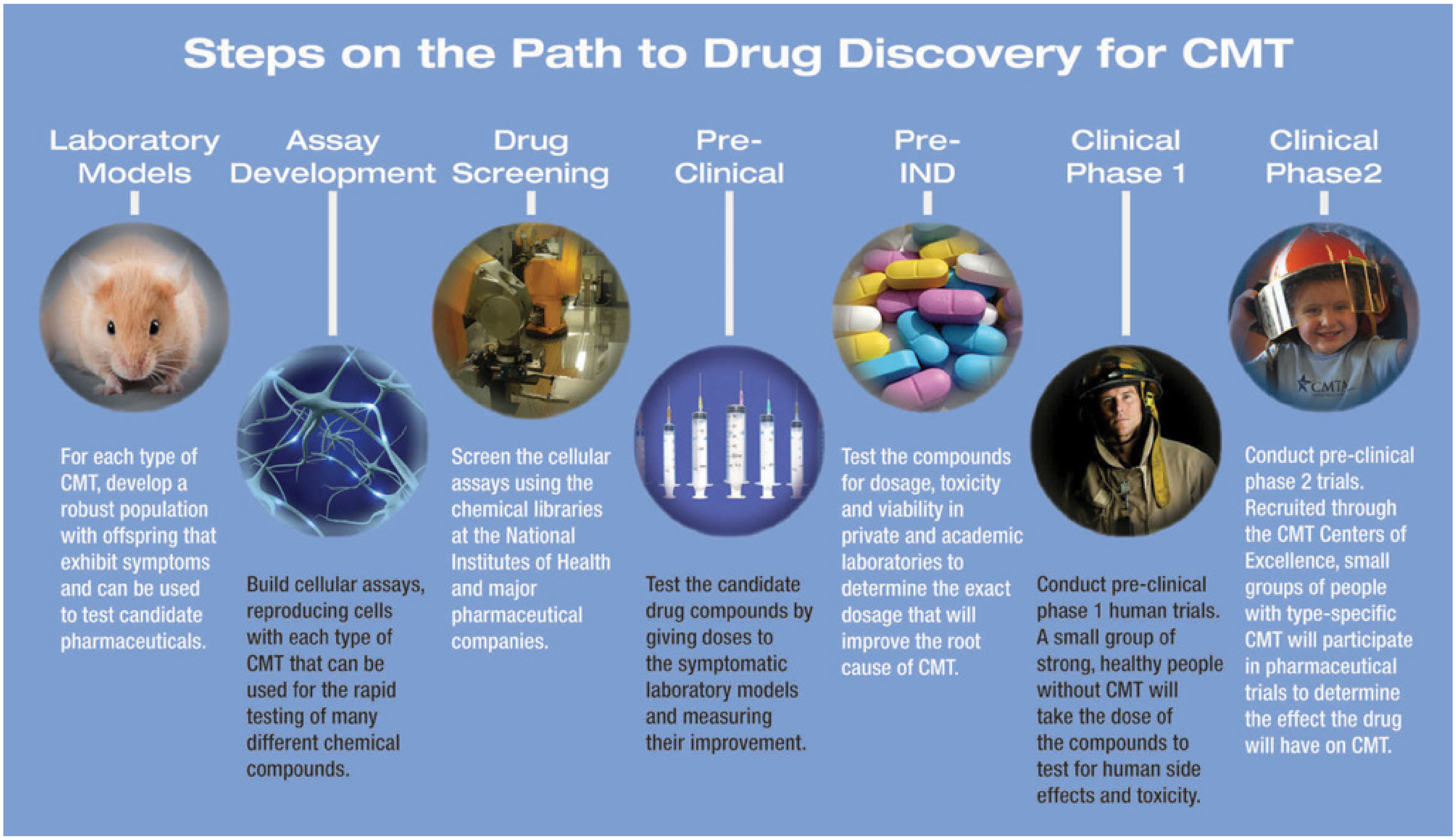
In the world of Sanofi, a major healthcare company headquartered in Paris, France, and of its rare disease affiliate, Genzyme, headquartered in Cambridge, Massachusetts, the Tucson Compound Library is highly acclaimed. The library includes more than 2 million compounds that can be tested to identify treatments and cures for many diseases. Even greater than the library is the team of Tucson researchers committed to finding drugs to treat rare diseases.

The CMTA's partnership with Genzyme unlocked access to the Tucson Library, where the team completed an initial screen of more than 1.8 million compounds in a CMT1A cell model developed in the labs of Drs. John Svaren and James Inglese, both CMTA investigators.

For three weeks, robotics were used to inject each of the 1,586 tiny wells in a series of well plates with CMT1A assays, and then with luciferase, the enzyme that makes fireflies glow. The compound screen is designed to detect the downregulation of PMP22, the duplicated protein that causes the nerves in CMT1A to demyelinate. After three weeks of robotically running the screen on hundreds of well plates, the data was translated through a computer program into readable graphs.

The ultimate goal for our partnership with Genzyme is to identify compounds to stop CMT in its tracks and perhaps even reverse it.

genzyme
A SANOFI COMPANY



Steps on the Path to Drug Discovery for CMT

Laboratory Models: For each type of CMT, develop a robust population with offspring that exhibit symptoms and can be used to test candidate pharmaceuticals.

Assay Development: Build cellular assays, reproducing cells with each type of CMT that can be used for the rapid testing of many different chemical compounds.

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

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

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

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

Clinical Phase2: Conduct pre-clinical phase 2 trials. Recruited through the CMT Centers of Excellence, small groups of people with type-specific CMT will participate in pharmaceutical trials to determine the effect the drug will have on CMT.

Ionis

A new paper published in the *Journal of Clinical Investigation* revealed an exciting potential treatment for CMT. Scientists at Ionis Pharmaceuticals, in collaboration with the CMTA, identified a promising early-stage therapeutic approach with antisense technology for CMT1A, the most common form of the disease, accounting for half of all cases of CMT.

CMT1A is caused by the duplication of the Peripheral Myelin Protein 22 gene (*PMP22*) which affects the myelin sheath of the nerves, causing neuropathy and muscular atrophy. Reducing the level of PMP22 has long been considered the most promising treatment approach for CMT1A. For the study, Ionis researchers used their expertise as a world leader in developing antisense oligonucleotides, which precisely target the genes that cause diseases.

The antisense drugs they developed reduced the amount of the PMP22 gene product (PMP22 messenger RNA). The researchers then tested the most potent antisense compounds in two different animal models of CMT1A. In both models, weekly injections of the PMP22-targeting antisense drug inhibited the progression of neuropathy, and they even improved some aspects of neuropathy during the treatment.

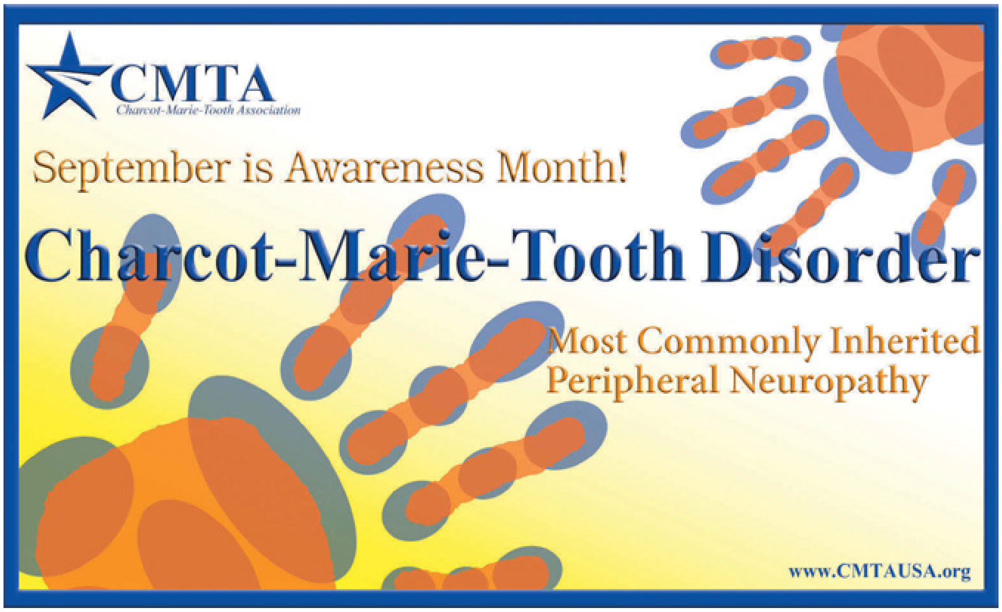
"Our researchers have successfully developed antisense drugs for other neurologic diseases, such as spinal muscular atrophy and TTR polyneuropathy," says C. Frank Bennett, PhD, senior vice president of research and leader of the neurological disease franchise at Ionis. "The results we have seen thus far for CMT1A are very promising, but more work is needed to determine if these studies in rodents can be translated to a therapy for CMT1A patients. Our scientists are working diligently with the CMTA and academic collaborators to translate these exciting preclinical studies to the identification of a potential drug."

IONIS™
PHARMACEUTICALS

CMTA Celebrates Its First CMT Awareness Week—Then Awareness Month!

Recognition aims to raise awareness about CMT.

Empowered by and equipped with education about CMT, the CMTA community moved forward and began to educate others about CMT. One special week morphed into a full month of special activities from webinars to Walks 4 CMT.



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CMTA CENTERS OF EXCELLENCE

www.cmtausa.org/coe

CMTA CENTERS OF EXCELLENCE 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. The Centers roughly correspond to the 21 international sites that make up the NIH Inherited Neuropathies 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.

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CMT PATIENT MEDICATION ALERT:

Definite high risk (including asymptomatic CMT):

Taxols (paclitaxel, docetaxel, cabazitaxel)
Vinca alkaloids (Vincristine)

Moderate to significant risk:

Amiodarone (Cordarone)
Arsenic Trioxide (Trisenox)
Bortezomib (Velcade)
Brentuximab Vedotin (Adcetris)
Cetuximab (Erbitux)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddl, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Eribulin (Halaven)
Fluoroquinolones
Gold salts
Ipilimumab (Yervoy)
Ixabepilone (Ixempra)
Lefluonamide (Arava)
Lenalidomide (Revlimid)
Metronidazole/Misonidazole (extended use)
Nitrofurantoin (Macrodantin, Furadantin, Macrobid)
Nitrous oxide (inhalation abuse)
Nivolumab (Opdivo)
Pembrolizumab (Keytruda)
Perhexiline (not used in US)
Pomalidomide (Pomalyst)
Pyridoxine (mega dose of Vitamin B6)
Stavudine (d4T, Zerit)
Suramin
Thalidomide
Zalcitabine (ddC, Hivid)

Uncertain or minor risk:

5-Fluouracil
Adriamycin
Almitrine (not in US)
Chloroquine
Cytarabine (high dose)
Ethambutol
Etoposide (VP-16)
Gemcitabine
Griseofulvin
Hexamethylmelamine
Hydralazine
Ifosfamide
Infliximab
Isoniazid (INH)
Lansoprazole (Prevacid)
Mefloquine
Omeprazole (Prilosec)
Penicillamine
Phenytoin (Dilantin)
Podophyllin resin
Sertraline (Zoloft)
Statins
Tacrolimus (FK506, Prograf)
Zimeldine (not in US)
a-Interferon

Negligible or doubtful risk:

Allopurinol
Amitriptyline
Chloramphenicol
Chlorprothixene
Cimetidine
Clioquinol
Clofibrate
Cyclosporin A
Enalapril
Glutethimide
Lithium
Phenelzine
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THE CMTA Report

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WHAT IS CMT?

- More than 2.8 million people worldwide have CMT, which is one of the most commonly inherited nerve disorders and affects the motor and sensory nerves.
- CMT is slowly progressive, causing the loss of muscle function and/or sensation in the lower legs and feet, as well as hands and arms.
- Men and women in all ethnic groups may be affected by CMT.
- CMT is genetic, but it can also develop as a new, spontaneous mutation.
- CMT can vary greatly in severity, even within the same family.
- CMT causes structural deformities such as high-arched or very flat feet, hammertoes, hand contractures, scoliosis (spinal curvature) and kyphosis (rounded back).
- CMT can also cause foot drop, poor balance, cold extremities, cramps, nerve, muscle and joint pain, altered reflexes, fatigue, tremor, sleep apnea, hearing loss and breathing difficulties.
- CMT rarely affects life expectancy.
- Some medications are neurotoxic and pose a high risk to people with CMT, notably Vincristine and Taxols. See full list (at left) of medications that may pose a risk.
- More than 100 different genetic causes of CMT have been identified. ***The first was discovered in 1991!***
- Many types of CMT can be determined by genetic testing. Please consult with a genetic counselor or your physician for more information (www.nsgc.org).
- Although there are no drug treatments for CMT, a healthy diet, moderate exercise, physical and/or occupational therapy, leg braces or orthopedic surgery may help maintain mobility and function.
- The CMTA's STAR research program and extensive partnerships with pharmaceutical companies are driving remarkable progress toward delivering treatments for CMT, bringing us closer to a world without CMT.