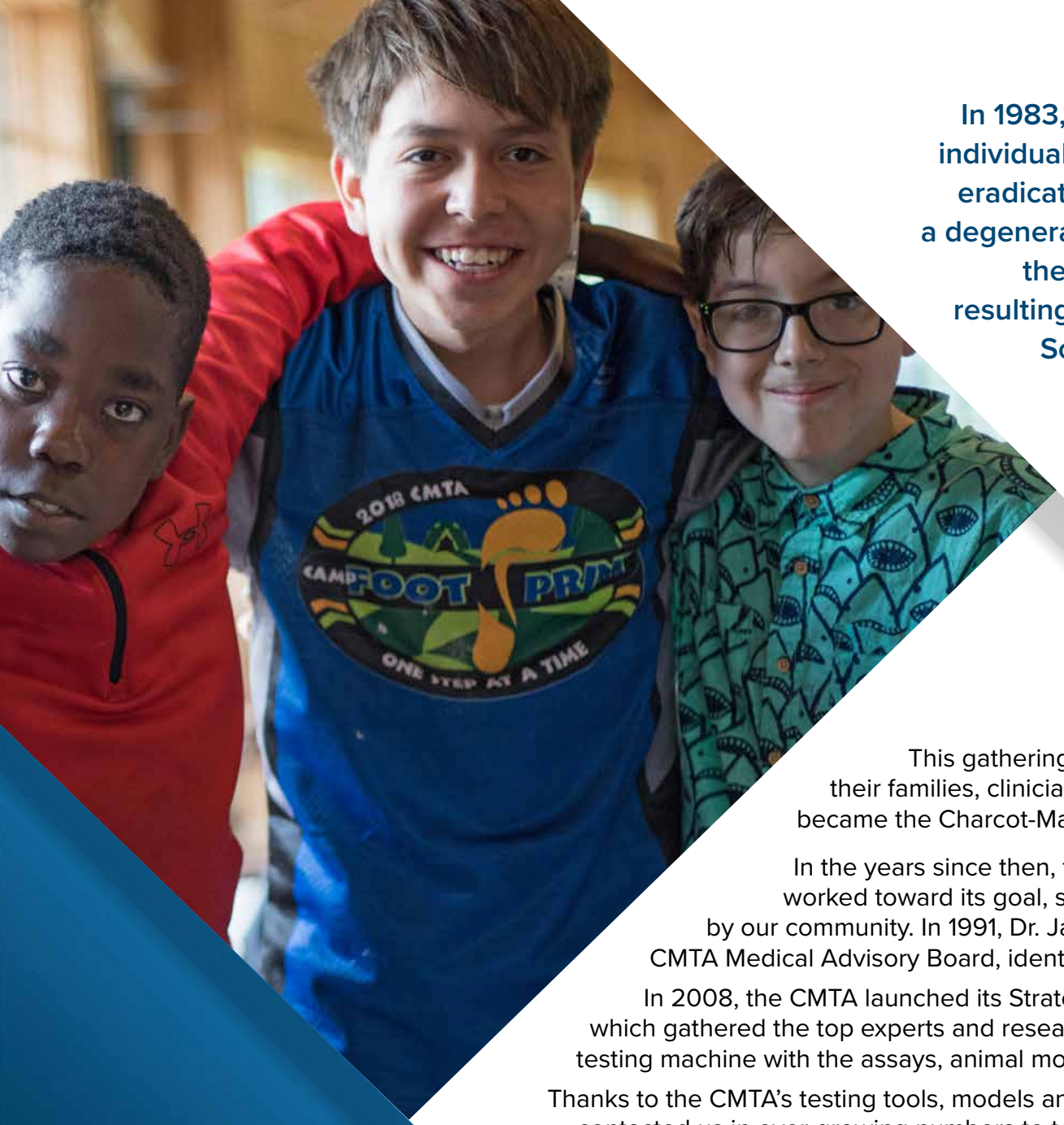
ACCELERATING RESEARCH

EMPOWERING PATIENTS

CMTA
Charcot-Marie-Tooth Association

# A COMMUNITY DRIVEN TO SUCCEED




**In 1983, a small group of thoughtful, committed individuals came together with the singular goal of eradicating Charcot-Marie-Tooth disease (CMT), a degenerative neuromuscular disease that damages the long nerves to the hands and feet, resulting in atrophy in the muscles around them. Some 3 million people are affected.**

This gathering of patients, their families, clinicians and researchers became the Charcot-Marie-Tooth Association.

In the years since then, the CMTA has steadily worked toward its goal, supported and financed by our community. In 1991, Dr. James Lupski, a member of the CMTA Medical Advisory Board, identified the first gene causing CMT1A.

In 2008, the CMTA launched its Strategy to Accelerate Research (STAR), which gathered the top experts and researchers in the field, then built a top-notch testing machine with the assays, animal models and stem cells needed for research.

Thanks to the CMTA's testing tools, models and experts, pharmaceutical companies have contacted us in ever-growing numbers to test their drugs and technologies on CMT.

***Never doubt that a small group of thoughtful committed individuals can change the world. In fact, it's the only thing that ever has."***
***- Margaret Mead***

Some companies use the traditional "small molecule" approach, while others are leaders in the latest genetic and neurological technologies such as CRISPR, gene therapy, gene silencing and axon and muscle regeneration. All told, the CMTA now has more than 50 active research projects running with 40 alliance partners from top biotech, pharma and gene therapy labs worldwide.

While STAR pushes hard for a cure, the CMTA supports the community with a wide array of patient and family services, including support groups, publications, online educational sessions and Camp Footprint, the first camp just for kids with CMT in the United States. The CMTA's numbers have grown since that early meeting, but it's still a group of thoughtful, committed individuals working together toward a world without CMT and willing to do whatever it takes to accomplish it.


With our community's support, the CMTA has invested over $17.5 million in CMTA-STAR research since 2008, making us the largest charitable funder of CMT research globally. But our work isn't done: Our strategy outlines investments of another $10 million in the next few years to accelerate the search for CMT drugs and therapies.

RESEARCH EDUCATION COMMUNITY

# WHAT WE DO

***Accelerating research and empowering patients are the polar stars of the CMTA's mission, guiding everything we do.***

While the CMTA is accelerating research, it also offers community members more immediate help, including 73+ local branches that provide the main point of contact with our members, and nearly 50 patient-centric, multidisciplinary CMT clinics, staffed by some of the best CMT clinicians and researchers in the world and a wide variety of educational materials and conferences. Facebook groups and a podcast provide online support.

The CMTA also has an array of offerings for young people with CMT, including a Youth Zoom hangout, a quarterly newsletter and Camp Footprint, the country's only summer camp for kids with CMT.

Children with CMT experience physical limitations daily. For many, walking is difficult and running impossible. Even something as mundane as picking a coin up off a table can be challenging. Kids with CMT are often excluded from PE at school, after-school sports and neighborhood pick-up games. They are almost always chosen last for team sports.

Camp Footprint is the one week a year kids from 10 to 18 can connect with their peers, an irreplaceable opportunity to share the hopes and fears of living with a rare neuromuscular disorder. Camp Footprint gives kids the chance to feel understood and to blend in; for many, it's their first time meeting another person with CMT.

***As the late John Lewis said at Dr. Martin Luther King, Jr.'s March on Washington, "If not us, then who? If not now, then when?"***

Community members provide 100 percent of our funding out of a deep and abiding desire to ensure that no one else has to suffer as they have. The CMTA carefully stewards those donations, recently earning Charity Navigator's 4-Star ranking for the third year in a row, the only nonprofit in the CMT space to do so.

CMTA Board Member Dan Chamby joined our community after retiring as a portfolio manager for BlackRock. Before committing to a board seat, he looked carefully at our mission, management and finances. He says he found an organization of really smart people with “energy and enthusiasm and a strong sense of mission and collaboration.” The diversity of the organization, which comprises scientists, business people, educators and patients all working toward the same goal, gives the CMTA the ability to “swarm” a problem with everyone putting everything they have into solving it.

The CMTA’s community outreach efforts provide energy and excitement for the research, Dan said, quoting Ralph Waldo Emerson’s words: “Nothing great was ever achieved without enthusiasm.”

Ultimately, all of the CMTA’s efforts are aimed at providing our community help for today and hope for tomorrow. We do what we do to create a brighter future for everyone with CMT—from the little boy who dreams of playing soccer to the father who dreams of walking his daughter down the aisle. We do it for the future.

CMTA-STAR

WHY WE DO IT

# HOW WE DO IT

**Virtually every type of CMT is covered by an active research project, even some unknown variants. Cross-type initiatives have the potential to benefit everyone no matter their type or the severity of their cases: Good news for one type may also be good news for another type.**

Donations to STAR are bundled and used for a multitude of important projects. After a rigorous review process, the CMTA makes grants to researchers based on how “translatable” a proposed program is, in other words, how easily findings can be transformed into therapies.

The CMTA works on multiple strategies and subtypes concurrently. Top leaders in gene therapy are now working on CMT subtypes 1A, 1X, 2A, 2E, 2F, 4A, 4C; and others are in process. We are working on a genome-editing/CRISPR Cas9 partnership for multiple CMT2 subtypes.

The CMTA is also harnessing new pathways to stabilize axons and prevent axon degeneration in CMT subtypes 1X, 2D, 2S, 2E, 1B and others are in the works, including testing axon-sparing strategies involving inhibition of SARM1.

Work on biomarkers, which indicate if a particular treatment is effective, is underway in CMT subtypes 1A, 1X, 2A and 1B. Reliable biomarkers that can measure results within three to six months are essential for clinical trials.

The CMTA’s drug development efforts include initiatives in CMT subtypes 1A, 1X, 2A, 1B, 4B1, 2D, 2E and other Type 2’s, as well as SORD and others that are in the pipeline, including eight active testing alliances, new small molecule approaches and a human stem cell approach for 1A.

# A HISTORY OF SUCCESS

**1983**

Carolyn Redell organizes a meeting of CMT families and physicians in NYC. One of the invitees, Dr. Howard Shapiro, creates a CMT organization: The National Foundation for Peroneal Muscular Atrophy (NFPMA).

**1985/89**

With a mailing list of 24 people, Dr. Shapiro publishes the first newsletter! He then organizes patient/family gatherings, establishes a database of CMT clinicians and focuses on CMT research.

**1987/96**

Although we knew CMT was caused by genetic mutations, the exact genetic causes were unknown. Within a 9-year period, the genetic causes of CMT1A, HNPP, CMT1B, CMT1D and CMT1X are pinpointed.

**1990**

Name Change! The NFPMA becomes the Charcot-Marie-Tooth Association (CMTA).

**1995**

The CMTA Board of Directors begins awarding $35,000 fellowship grants to CMT researchers.

**2001**

In conjunction with Wayne State University, Dr. Michael Shy and the CMTA establishes the North American CMT Database.

**2003**

CMT advocate and volunteer Bob Budde becomes the liaison for CMTA support groups.

**2006**

Joining forces with the MDA, the CMTA funds the first-ever ascorbic acid clinical trials.

**2008**

In a vote of confidence for the CMTA's new research initiative, two CMTA families make large contributions and kick start fundraising campaigns to support the **Strategy to Accelerate Research (STAR).**

**2009**

Less than one year after STAR is set into motion, the first CMT1A cell line is grown. Using High Throughput Screening (HTS) at the National Institutes of Health, CMT1A drug discovery begins.

**2010**

The CMTA brings together globally recognized CMT specialists for a CMT Type 2 research symposium in San Diego, CA.

**2011**

CMTA board member Elizabeth Ouellette organizes the first CMT Awareness Week. Her effort sparks an international movement and becomes an annual month-long celebration to spread awareness about CMT.

**2012**

The CMTA establishes 11 Centers of Excellence to help ensure CMT patients receive the best possible evaluation and care, and their information is collected for possible recruitment into clinical trials.

**2013**

Dr. John Svaren, from the University of Wisconsin, creates state-of-the-art cell lines employing genome editing technology, while other scientists in the CMTA network develop and utilize human stem cells in CMT research.

**2014**

The CMTA establishes 11 Centers of Excellence to ensure CMT patients receive the best possible evaluation and care, and their information is collected for possible recruitment into clinical trials.

**2016**

The CMTA establishes Camp Footprint - the first camp in the US for children living with CMT.

**2017**

In collaboration with Ionis, the CMTA announces a major breakthrough in CMT1A research.

**2018**

Gene therapy summit. New Gene therapy projects and SAB members. Multi-year, multi-partner initiative to test drugs developed to slow axon degeneration in many CMT models.

**2019**

Passage Bio commits to CMT2A gene therapy CRISPR project with top lab at UC (associated with 2020 Nobel prize laureate), CMTA partner Inflectis completes phase 1 clinical trial for Sephin (1A and 1B potential). Renewed support for the INC.

**2020**

Gene therapy projects in progress for 1A 1X 2A 2E 2F 4C and 4A with world-class labs and 4 biotech partners. SORD story. Working with Pharnext on biomarkers for their 2021 1A trial.

**2021**

One of fifteen health-based organizations to receive a Perfect 100 Score from Charity Navigator for financial responsibility and transparency.

**DOLLARS SPENT:** **$17.5M**

**RESEARCH PARTNERS:** **40+**

**NUMBER OF PROJECTS:** **50+**


# Every Donation Keeps Hope Alive For Everyone Living With CMT

**$100**
Might pay for information packets for newly diagnosed patients at CMTA Centers of Excellence

**$250**
Could support patient education through CMTA Reports

**$500**
Could support one camper at Camp Footprint

**$2,500**
Might go to major biomarker testing initiatives needed for clinical trials

**$5,000**
Could go to clinical trials for CMT

**$10,000**
Could support application of CRISPR Cas9 to CMT research

CHARITY NAVIGATOR
PERFECT 100
★★★★
CHARITY NAVIGATOR
Four Star Charity

Please Note: The facts and figures are an estimate only. Donations are tax-deductible to the full extent allowable by law. This list is not intended to be comprehensive.

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