ACCELERATING RESEARCH

EMPOWERING PATIENTS

CMTA®
Charcot-Marie-Tooth Association

COMMUNITY LED

COMMUNITY DRIVEN

WHO WE ARE

IN 1983, a small but determined group of patients, their families, clinicians, and researchers united with one mission: to eliminate Charcot-Marie-Tooth disease (CMT). CMT is an inheritable neuromuscular disease that affects 1 in 2500 people. It harms the nerves connecting the body to the brain, leading to weakened muscles and sensory loss. There is no effective treatment or cure.

FROM BONDS SHARED THROUGH CMT, the Charcot-Marie-Tooth Association (CMTA) was born. Since its beginning, the CMTA has steadily worked towards our community-led, community-driven mission to support the development of new treatments for CMT, to improve the quality of life for people with CMT, and, ultimately, to find a cure.

Beyond research to bring treatments and a cure, the CMTA supports the CMT community with a wide array of patient and family services including local support groups, publications, online educational sessions, and Camp Footprint—the first camp in the United States just for kids with CMT. Every decision we make is based on the needs and priorities of the community.

"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

Accelerating Research and Empowering Patients are the polar stars of the CMTA's mission, guiding everything we do.

WHAT WE DO

In 2008, the CMTA launched its pioneering Strategy to Accelerate Research (STAR) program. Through STAR, the CMTA unites the community with clinicians and industry experts to accelerate the advancement of treatments. Thanks to the generous support from the community, the CMTA has invested more than $24 million since starting the STAR program. However, our mission is far from complete. To achieve our vision of a world without CMT, our strategic plan calls for an additional investment of more than $10 million in the coming years.

As the CMTA accelerates CMT research, it also empowers the CMT community through an extensive support network of dozens of local Branches, more than 50 patient-centric, multidisciplinary Centers of Excellence CMT clinics that are staffed by the world's top CMT specialists, volumes of free education materials, quarterly virtual community events, in-person regional education events, the largest gathering of CMT community members anywhere—our flagship annual patient and research summit, the one-of-kind Camp Footprint for kids who have CMT, and so much more. Everything we do, we do because of you, and because you make it happen.

WHY WE DO IT

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?"

DRIVEN BY AN UNENDING DESIRE TO IMPROVE THE LIVES OF ALL WHO HAVE CMT,

our community members generously provide 100% of our funding. The CMTA's careful stewardship of these donations has led to receiving Charity Navigator's prestigious 4-Star ranking for the fourth consecutive year. The CMTA is the only CMT nonprofit to achieve this standing.

People with CMT experience physical limitations daily. For many, walking is difficult, running can be impossible, and many have to use mobility aids such as leg braces or wheelchairs. Something as mundane as picking up a coin from a counter, tying one's shoes, or zipping up a jacket can be challenging. Peers often exclude children with CMT from after-school sports, from neighborhood pick-up games, and frequently choose them last for team sports. CMT can be very painful for some, can lead to breathing problems and hearing loss, and so much more.

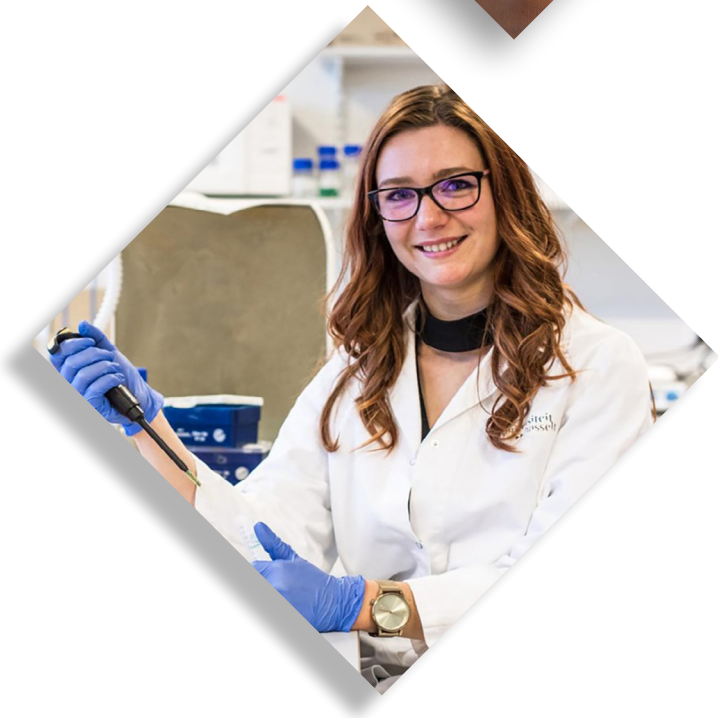
The CMTA aims its efforts at providing the CMT community help for today and hope for tomorrow. We do these things, together, hand-in-hand, with our community to create a brighter future for everyone with CMT. From the little girl who dreams of playing soccer to the father who dreams of holding his grandson, we do it for the future. Together, we will achieve our vision: a world without CMT.

HOW WE DO IT

Our research covers virtually every type of CMT. Cross-type initiatives have the potential to benefit everyone no matter their type or the severity of their CMT. Good news for one type may also be good news for another type.

The CMTA bundles CMTA-STAR donations and strategically invests them in a multitude of critical 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 researchers can transform findings into treatments. And the CMTA works on multiple strategies and types all at the same time.

Top leaders in gene therapy are now working on CMT1A, X1 (aka 1X/CMTX), 2A, 2E, 2F, 4A, and 4C; and others are in planning stages. We're harnessing new pathways to stabilize axons and prevent axon degeneration in 1B, X1, 2D, 2E, and 2S; and others are in the works.



Work on biomarkers, which show if a particular treatment is working, is underway in many types, including 1A, 1B, X1, 2A, and 2F. Reliable biomarkers that can measure results within months rather than years are essential for clinical trials and we're the leaders in this area.

The CMTA's drug development pipeline includes initiatives in 1A, 1B, X1, 2A, 2D, 2E, 4B1, and other types such as CMT-SORD, which the CMTA, with its alliance and research partnerships, took from discovery to the current INSPIRE Phase III clinical trial in less than three years. CMTA partnerships also include eight active testing alliances. The CMTA is more focused on bringing treatments and a cure than any other organization.

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).

1985-1989: 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.

1987-1996: With an NFPMA-funded gene discovery initiative beginning in 1987, researchers found the genetic causes of CMT1A, HNPP, CMT1B, CMT1D, and CMTX1 by 1996. They found the first one, the cause for 1A, in 1991. Gene discovery remains a CMTA priority to this day.

1990: 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 establish the North American CMT Database.

2006: Joining forces with the MDA, the CMTA funds the first-ever drug trial for CMT1A, which was ascorbic acid as a possible treatment.

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 CMTA's new Strategy to Accelerate Research (STAR).

2009: Less than one year after STAR launched, researchers grow the first CMT1A cell line. Using High Throughput Screening (HTS) at the National Institutes of Health (NIH), CMT1A drug discovery begins.

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 in September as CMT Awareness Month.

2012: The CMTA establishes 11 Centers of Excellence to help ensure CMT patients receive the best possible care, and the clinicians begin collecting relevant information 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.

2016: The CMTA launches Camp Footprint—the first camp in the US for children with CMT.

2019: Passage Bio commits to a CMT2A gene therapy CRISPR project with a top lab at UC (associated with 2020 Nobel prize laureate). **Inflectis completes Phase I clinical trial** for 1A and 1B potential treatment. Both companies are CMTA alliance partners.

2020: CMTA-funded breakthrough CMT-SORD discovery. Gene therapy projects are in progress for 1A, X1, 2A, 2E, 2F, 4A, and 4C with world-class labs and four biotech partners.

2022: Received a Perfect 100 score from Charity Navigator for financial responsibility and transparency for the third year in a row (now four years running).

2023: CMT-SORD goes from CMTA-funded discovery in 2020 to Phase III INSPIRE clinical trial with alliance partner, Applied Therapeutics, in less than 3 years.

DOLLARS SPENT: **$24M+**

RESEARCH PARTNERS: **40+**

NUMBER OF ACTIVE PROJECTS: **50+**

Every Donation Keeps Hope Alive For Everyone Living With CMT

$100
Pays for Information Packets for Newly Diagnosed Patients at CMTA Centers of Excellence

$250
Supports Patient Education Through CMTA Reports

$500
Supports One Camper at Camp Footprint

$2,500
Supports Major Biomarker Testing Initiatives Needed for Clinical Trials

$5,000
Supports Clinical Trials for CMT

$10,000
Supports Genetic Therapies for CMT

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

The CMTA is a 501(c)(3) nonprofit organization. Donations are tax-deductible to the full extent allowable by law. Above list is intended to be illustrative.

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