

Charcot-Marie-Tooth Association



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Mission

The mission of the Charcot-Marie-Tooth Association is to support the development of new drugs to treat CMT, to improve the quality of life for people with CMT, and, ultimately, to find a cure.

The CMTA began as the National Foundation for Peroneal Muscular Atrophy (NFPMA) in 1983, founded by Dr. Howard Shapiro, a patient with CMT. CMTA published its first newsletter as the association in 1987 and branched out into support groups, patient family conferences, volunteers, a database, and a consortium through the years.

Strategy/Approach

The association focuses its efforts on research and providing patient support and resources. CMTA is the sole funder of the STAR program for research on Charcot-Marie-Tooth disease and also provides research grants to institutions. To support patients, caregivers, families, and friends, the association has a robust online community as well as support and action groups nationwide. CMTA also provides resources for those diagnosed with CMT including articles, expert support, webinars, and helpful products for treating the disease.

Research Portfolio

In 2008, CMTA launched the Strategy to Accelerate Research (STAR) to capitalize on breakthroughs in genetics and speed up the pace of CMT research. An international network of scientists with a dedicated scientific advisory board, this program is funded solely by the CMTA. The goals of the STAR program are:

- Within 5 years, introduce effective therapies for the three most common types of CMT;
- Within 10 years, stabilize or even reverse, symptoms of the disorder in some instances.

By directly engaging some of the world's foremost peripheral nerve and myelin experts and having them work together in a well-defined research initiative, the STAR program set out to achieve four phases of research:

- Grow CMT1A cell line that expresses PMP22;
- Work in conjunction with NIH National Chemical Genomics Center to screen more than 300,000 compounds against the CMT1A cell line;
- Further evaluate the most promising candidate compounds in animal models;
- Conduct human clinical trials.

In addition to the STAR program, CMTA also funds other research through grants including programs at the National Human Genome Research Institute, University of Miami,

California Institute of Technology, and University of Wisconsin Madison.

Partnership Practices

The association does not partner with biotech and pharmaceutical companies. *FasterCures* is not aware of any formal industry partnerships maintained by the organization.

Financials

The association is a 501(c)(3) charitable organization. The most recent financial information available is from 2012.¹

Year ending 12/31/12:

- Revenue: \$1,698,730
- Assets: \$1,106,759
- Grants: \$432,481
- Gifts Received: \$844,491
- Expenditures: \$1,446,000

Key Accomplishments

- Funded STAR Pathways program with \$25 million campaign to identify compounds to stop the progression of CMT.
- Screened over 350,000 pharmaceutical candidates, with efficacy proven in several compounds.
- Support and Action Groups in 40 states, Mexico, and Canada.

Leadership

The CMTA is led by a board of directors and a medical advisory board comprised of over 50 clinical and research medical professionals who are experts in neurology, genetics, orthopedic surgery, psychiatry, physical therapy, and podiatry.

- **Chairman of the Board:** Herbert Beron
- **CEO:** Patrick A. Livney, pal@ctmausa.org, 1-800-606-2682, ext. 102
- **Director of Community Services:** Jeana Sweeney, jeana@ctmausa.org, 1-800-606-2682, ext. 106
- **Director of Program Services:** Patricia Dreibelbis, pat@ctmausa.org, 1-800-606-2682, ext. 103

¹ Information obtained from GuideStar, <http://www.guidestar.org/organizations/22-2480896/charcot-marie-tooth-association.aspx> March 2014.