HEREDITARY NEUROPATHY FOUNDATION

JULY-SEPTEMBER 2018

cmtupdate

BECOME A "CMT GAME-CHANGER" FOR CMT AWARENESS MONTH!

So what is a CMT Game-Changer?

A CMT Game-Changer is an empowered individual who accelerates CMT awareness, research, wellness, advocacy, fundraising and patient empowerment through their action and involvement in community events, programs, campaigns and studies. Additionally, they are individuals determined to end CMT and who believes in the importance of their role towards advancement.

What better time to push for change than during the month of September for CMT Awareness Month? We're so excited to present our wide selection of 2018 initiatives with easy-to-follow activities and postable fun. There's a section for everyone and each action you take adds fuel to our mission!

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IN THIS ISSUE

1-855-HELPCMT (435-7268)

www.hnf-cure.org

The Hereditary Neuropathy Foundation's mission is to increase awareness and accurate diagnosis of Charcot-Marie-Tooth (CMT) and related Inherited Neuropathies, support patients and families with critical information to improve quality of life, and fund research that will lead to treatments and cures.

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HERE'S HOW YOU CAN GET INVOLVED!

September Awareness CMT Kits

HNF has designed a September Awareness Month kit to assist you with spreading the word about CMT. Learn, laugh, and spread awareness with each kit.

Visit: CLICK HERE

Docs That Rock

Have excellent doctors or healthcare providers? Nominate your Docs That Rock and tell us how they meet your needs as a CMT patient. We'll share your submission on our dedicated web page for fellow patients nationwide!

Visit: CLICK HERE

Shop Our Store

Spread CMT awareness with style! We've designed new T-shirts, phone cases, and more. 100% of the profits will go to CMT research and programs.

Visit: CLICK HERE

Follow Us On Social Media

Like, comment, and share the latest HNF CMT news and events on all your social media channels with the hashtag #CMTWeGotThis. Don't forget to print your Selfie Card and post pictures, making sure you tag a friend.

Visit: CLICK HERE

Reach Out To Your Representatives

Be the change you wish to see. Find your local representative through our linked directory and then download, print, and mail them our custom CMT advocacy letter.

Visit: CLICK HERE

Band Together For CMT

Receive your exclusive set of resistance bands with our guide book of CMT-specific exercises with every $25 donation.

Visit: CLICK HERE

Top Ways To Get Involved

HNF has several ways you can make a contribution to help raise awareness and funds for CMT research.

Visit: CLICK HERE

Voice of the Patient Weekend:

Expanding the Science of Patient Input in Drug Development and Tapping into Personal Potential

BY **ALLISON MOORE**, FOUNDER/CEO, HNF

The time for our community to shine is coming as we prepare for the **Patient-Focused Drug-Development Meeting on September 28, 2018!** Susan Dentzer, editor of Health Affairs Today, has referred to patient engagement as the "**blockbuster drug of the 21st century**". On this monumental day, our patient voices will finally be heard to give the FDA and other stakeholders a deeper understanding of the experience of living with CMT and other Inherited Neuropathies. By integrating our patient perspectives at the meeting, we actively involve patients in their health and healthcare system.

Why are PFDD Meetings Important?

Decision-makers are increasingly realizing that the voice of the patient is a critical element in understanding how to develop and approve therapies that provide clinically-meaningful benefit to those living with a particular disease. Without patient insights, the FDA's ability to assess the benefits and risks of a particular therapy, and its ability to provide real benefit to patients, is significantly compromised.

As such, the purpose of this meeting will be to provide the FDA with insights and information from CMT patients, families, and caregivers on the impact that CMT has on their day-to-day lives, including their perspective on the symptoms that are most important. Through this meeting, the FDA also seeks to understand the ways in which patients treat and manage their CMT (symptoms) and the decision factors that they take into account when choosing a treatment. This feedback will, in turn, inform the FDA regarding the severity of the CMT, along with the benefit-risk balance of treatment options and unmet medical needs.

Ultimately, your voices, whether you are panelists, attendees, participants in the webcast and the overall feedback gathered by all who participate, will directly help to inform the decisions made about potential drugs in development and the overall assessment of future therapeutics in CMT.

If you haven't signed up yet, please do: CLICK HERE
For those that have, thank you!

Patient-Centered CMT Behavioral Health Summit

More good news...through the Hereditary Neuropathy Foundation Patient-Focused Research Initiative, we will be addressing emotional needs of the CMT community with the first ever, Patient-Centered Behavioral CMT Health Summit. The Summit is to be held on Saturday, September 29, 2018, at the College Park Marriott Hotel and Conference Center in Hyattsville, MD.

This year's annual summit will be a life-changing experience for CMT patients and families, with a strong focus on behavioral and emotional health. "We often hear so much in regards to the physical aspects of disease and disability, but very little on how it impacts our mental well-being, which for many can be more devastating. Our goal with this summit is to provide a curated "CMT Toolbox" that attendees can apply to their daily lives for increased fulfillment and wellness across all avenues," says Estela Lugo, HNF Medical Outreach Manager.

The day will begin with an interactive empowerment session developed specifically for the CMT community by highly-accredited thought leader, Lisa McCarthy, co-founder of The Fast Forward Group. Attendees will also learn how surgery outcomes can affect behavioral health from orthopedic surgeon, Glenn B. Pfeffer, MD. Two breakout workshops, moderated by Lainie Ishbia, founder of Trend-Able and Alana Kessler, Registered Dietitian and Holistic Health Coach will dive deeper into improving lifestyle, nutrition, confidence and social interactions.

This single day has the potential to drastically shift the CMT patient perspective on how they approach self-care and wellness, as well as empower them to tap farther into their personal potential.

Hurry, limited spots left, sign up here: CLICK HERE

Voice of the Patient Weekend

thank you to our sponsors

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Charcot-Marie-Tooth Association

MDA Muscular Dystrophy Association

EVERYLIFE FOUNDATION FOR RARE DISEASES

Patients create a unique profile on www.neuropathyreg.org

ABOUT GLOBAL REGISTRY FOR INHERITED NEUROPATHIES (GRIN)

BY **JOY ALDRICH**, ADVOCACY DIRECTOR, HNF

The Global Registry for Inherited Neuropathies (GRIN) is a global patient-focused research database that represents patients and families via vital Natural History Studies, symptom specific studies and potential treatment studies that are reported by you, the patient. These groundbreaking study surveys track the course of disease over time and identify multiple aspects of the disease experience such as, demographics, genetic mutations, medical care interventions, environmental influences and other variables. GRIN uses this information to correlate patient outcomes so we may better understand CMT and Inherited Neuropathies. With this new and precise information on the CMT population, we can accelerate therapy development for all types of Inherited Neuropathies. These studies support HNF's Therapeutic Research In Accelerated Discovery (TRIAD) program — an effort with academia, government and industry — to develop treatments for CMT. Currently, TRIAD involves many groups that span the drug discovery, drug development, and diagnostics continuum.

Help us to improve outcome measures, develop biomarkers and improve patient care by joining GRIN! **www.neuropathyreg.org**

We aim to characterize the genotype-phenotype (genetic mutation-symptoms) correlations and establish baseline Patient-Reported Outcomes data, and when available, clinical data for Inherited Neuropathies caused by genetic mutations for all forms of CMT. Without your participation, researchers won't have the essential and necessary patient information to develop drugs, gene therapies, and clinical trials for Charcot-Marie-Tooth and other Inherited Neuropathies!

Why should you join GRIN? It's pretty simple...

As GRIN grows, we exponentially gain greater insights from you as patients to help accelerate therapies for Charcot-Marie-Tooth. This is an incredible opportunity for you to participate in Charcot-Marie-Tooth Patient-Focused Research.

Your vital data allows for researchers to study why individuals experience different symptoms and address what is most important to patients when thinking about drug development. Scientists can also learn how a particular mutation type may lead to different or unique symptoms helping us to develop treatments for all types of Inherited Neuropathies.

By completing your profile, your de-identified information will be utilized to advance research and support clinical trial designs. As a registrant, you will be informed when you may be eligible for clinical trials.

These Patient-Reported Outcomes Studies will enhance therapy development in collaboration with our industry partners. By informing Healthcare Providers and others with your critical data, we can work to improve diagnosis and enhance patient care.

Ready to Join?

Anyone diagnosed with Charcot-Marie-Tooth or other Inherited Neuropathies can join GRIN. Your information is always kept confidential: only approved research investigators and industry partners can see your de-identified information.

If you are already a participant, we still need you! It's important to update your profile and participate in many of our Patient-Reported Outcomes Research Studies.

With all of the current positive research momentum, there is no better time than right now to join GRIN! Become a part of the effort to find the treatments and cures for all Inherited Neuropathies!

Join Today: CLICK HERE

CANNABIS COMMUNITY CARE AND RESEARCH NETWORK℠

Cannabis Community Care & Research Network (C3RN) is excited to be partnering with the Hereditary Neuropathy Foundation (HNF) to advance Cannabis research, advocate for patients in need, and educate the community!

C3RN's team is passionate about ending stigma and advancing Cannabis research. The team is made up of Dr's, Scientists, Growers, Creators, and Doer's…but most importantly they are medical patients who understand the importance of education and awareness.

There are so many ways that this plant can help people with their ailments, but how do we know?

Stigma ends where education and awareness begins and whether it is via survey, verbal testimony, or social media...sharing your story is the start. Director of Sales; Andy Westerkamp shares his patient story on their website: CLICK HERE
Andy is living with HNPP and manages to improve his quality of life with the help of Cannabis.

Take a look at some of the national statistics from an ongoing IRB approved anonymous patient and consumer survey from C3RN and UMass Dartmouth:

62%
of Cannabis users are reducing prescription medication use

51%
report using Cannabis to ease anxiety

46%
use Cannabis to manage chronic pain

HNF, in partnership with Champlain Valley Dispensary/Ceres, is conducting a medical cannabis survey for CMT/HNPP patients to determine if its use is beneficial. Questions concerning types, amounts, methods of consumption and utilization of prescription drugs will help inform researchers.

Please find the survey here: CLICK HERE

This study is generously supported by Champlain / Ceres

SOUTHERN VERMONT WELLNESS
CHAMPLAIN VALLEY DISPENSARY

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TRIAD
THERAPEUTIC RESEARCH IN ACCELERATED DISCOVERY

HNF developed the Therapeutic Research in Accelerated Discovery (TRIAD) as a collaborative effort with academia, government and industry, to develop treatments for CMT. Currently, TRIAD involves many groups that span the drug discovery, drug development, and diagnostics continuum.

EXCITING NEWS:

HNF Joins Inherited Neuropathy Consortium

BY **ALLISON MOORE**, FOUNDER/CEO, HNF

HNF would like to announce, with great pleasure, that it has been invited to participate in the Inherited Neuropathy Consortium (INC), one of 22 groups under the Rare Diseases Clinical Research Network (RDCRN), funded primarily by the NIH.

The RDCRN was designed to encourage collaboration among scientists from multiple research sites and to include patient advocates to participate and serve as research partners. The network consortium also includes a training program for clinical investigators.

The INC is an integrated group of academic medical centers, patient support organizations, and clinical research resources whose primary purpose is to conduct research. If you are seeking patient care, however, these sites (some of which are HNF Centers of Excellence) also have the knowledge and resources to provide excellent patient care.

Since inception in 2009, the INC has conducted research that has enrolled close to 8,000 patients as part of their natural history studies (where patients are followed over time clinically) for a better understanding of CMT. Additionally, the INC has discovered new biomarkers and has developed special scales to help measure whether treatments are working in the day-to-day life of an individual, leading to deeper scientific understanding of the causes of CMT.

It's important for those patients that have been enrolled in the INC to put as much effort into visiting their admitting site each year to enhance the research program. These studies have forged the path today for industry to develop treatments for us.

To participate in the research, visit here: CLICK HERE

The more patients enrolled, the better it is for us!

Allison Moore, HNF CEO will represent HNF as a member of their Coalition of Patient Advocacy Groups (CPAG), which represents the perspective and interests of all patient advocacy organizations associated with the clinical research consortia. Through collaboration, patient advocacy groups and researchers can make faster progress toward new treatment options and cures, which can improve the lives of all persons and families affected by CMT. "I personally am honored to be part of the consortium that has for sure had an impact on the development of the clinical trials today and for others in the pipeline," says Allison. "As a representative I will serve our patient community and bring our collective voices to the consortium, as well as support all the consortium members."

I want to extend a special thank you to Dr. Michael Shy, who is the principal investigator of the INC, for inviting HNF to be part of the consortium.

RARE CLINICAL DISEASES RESEARCH NETWORK
Initiative of the National Center for Advancing Translational Sciences (NCATS)

The Rare Diseases Clinical Research Network (RDCRN) is an initiative that was established in 2003 by the Office of Rare Diseases Research, which is now part of the National Center for Advancing Sciences that today include a consortia of 22 rare disease groups. Thankfully, the INC is one of them. The network is intended to advance medical research by supporting clinical studies and facilitating collaboration and data sharing. With a collaborative research model that is multi-site, patient-centric, translational and clinical research focused, the consortia is working to address unmet medical needs for rare disease patients and to be clinical trial ready to enhance drug development and the ultimate success of clinical trials.

HNF Conducting Meeting for FDA to Advance Therapies for Charcot-Marie-Tooth

The Hereditary Neuropathy Foundation (HNF) is conducting a ground-breaking Externally-led Patient-Focused Drug Development Meeting (PFDD) for Charcot-Marie-Tooth Disease (CMT) and Inherited Neuropathies (IN) on Friday, September 28, 2018 at the College Park Marriott Hotel and Conference Center in Hyattsville, MD.

The meeting will bring together patients, caregivers, industry, healthcare providers, payors, NIH, and FDA, to understand the challenges of patients living with CMT and the unmet medical need where there are no drug treatments available. A live webcast will begin at 8:30am ET.

"This is an unprecedented time for the CMT community. The first CMT clinical trials are now reaching late-stage development, and additional pipeline clinical research is scheduled to take place in 2019. It's vital for all stakeholders to understand the life-altering impact that CMT has on patients and families," said Allison Moore, Founder and CEO of HNF. "This opportunity ensures patient perspectives are considered throughout the drug development continuum. We, as patients, are now steering the direction of our futures and have a responsibility to make our voices heard."

"Providing patients with an opportunity to speak directly to FDA representatives about what it's really like living with CMT and outlining what patients need in terms of new therapies, is critical to advancing much-needed medical breakthroughs," added Courtney Hollett, Executive Director, HNF. "We are grateful to all the patients who will bravely testify to represent our community."

Meeting agenda and information: http://www.cmt-pfdd.org

Distinguished Speakers at the Meeting:

* **Allison Moore**, Founder/CEO, Hereditary Neuropathy Foundation
* **Michael Shy**, MD, Director, Division of Neuromuscular Medicine, University of Iowa; Director, Inherited Neuropathy Rare Disease Consortium (INC)
* **Stephan Zuchner**, MD, PhD, Co-Director, John P. Hussman Institute for Human Genomics University of Miami Miller School of Medicine
* **Florian Thomas**, MD, MA, PhD, MS, Chairman, Neuroscience Institute, Hackensack University Medical Center
* **Lucas Kempf**, MD, Acting Associate Director for the Rare Disease Program at the FDA
* **Robert Moore**, Co-Founder, True Reply
* **James Valentine**, JD, MHS, former FDA official, and current Associate at Hyman, Phelps & McNamara

PFDD Event Sponsors

Pharnext, Acceleron Pharma, Cydan, Flex Pharma, Athena Diagnostics, Ionis Pharmaceuticals, Champlain Valley Dispensary, Ceres, Cresco Labs, Charcot-Marie-Tooth Association, Muscular Dystrophy Association, and EveryLife Foundation.

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for orphan and common neurodegenerative diseases that currently lack curative and/or disease-modifying treatments. Their lead drug is PXT3003, for the treatment of CMT1A, and benefits from orphan drug status in the United States and Europe. The results of this trial are expected by October 2018.

"We are honored to support and participate in the first FDA Externally-led Patient-Focused Drug Development (PFDD) meeting conducted by HNF dedicated to Charcot-Marie-Tooth disease," said Prof. Daniel Cohen, M.D., Ph.D., Co-Founder and Chief Executive Officer of Pharnext. "This meeting is a unique opportunity for CMT patients to have their voices heard in terms of their needs and the experience of living with this rare and debilitating disease. As of today, there are no therapeutic options for CMT except palliative care. But with new therapeutic options on the horizon, we believe it is critical for regulatory agencies, like the FDA, to grasp the daily challenges faced by CMT patients," he said.

Acceleron Pharma, Inc., a leading biopharmaceutical company in the discovery and development of TGF-beta therapeutics to treat serious and rare diseases, is advancing its lead neuromuscular program, ACE-083 in Phase 2 trials of patients with CMT. ACE-083 is a locally-acting therapeutic designed to have a concentrated effect on muscle mass and strength in target muscles for diseases that cause focal muscle weakness. ACE-083 utilizes the "Myostatin+" approach to inhibit multiple TGF-beta ligands.

In July, Acceleron presented positive preliminary results from the dose-escalation, Part 1 of its Phase 2 trial in patients with CMT. Preliminary results of Part 2 of the Phase 2 trial are expected by the end of 2019.

"People suffering from CMT currently have no drug therapy options to address the major consequences of this serious disease, such as foot drop leading to mobility impairment and frequent falls," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "We are honored to support this important patient-focused event and hope that ACE-083 will prove to be an important agent to increase lower leg function, improve the ability to walk, and reduce falls."

Voice of the Patient Report

Under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V), these meetings were designed to more systematically gather patients' perspectives on their condition and available therapies to treat their condition. The Voice of the Patient reports summarize the input provided by patients and patient representatives at each of these public meetings.

"We are thrilled to partner with the Charcot-Marie-Tooth Association and the Muscular Dystrophy Association to bring one voice to the FDA as we prepare the post-meeting Voice of the Patient Report," said Joy Aldrich, Advocacy Director, HNF.

To attend the PFDD Meeting: CLICK HERE

HNF to Host Educational CMT Symposium at AANEM Annual Conference

BY **ESTELA LUGO,** MANAGER, MEDICAL OUTREACH, HNF

When presented with the chance to educate the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) members on Patient-Focused Research and how Healthcare Providers can be part of the research continuum on therapy development their Annual Conference on October 10-13 in Washington, D.C., the team at HNF jumped at the chance!

The AANEM is a professional membership based non-profit with a network of over 4,000 physician and nonphysician members worldwide with a mission to improve the quality of patient care for neuromuscular diseases.

This annual meeting attracts more than 900+ neurologists, PMR physicians, neuromuscular specialists, and other healthcare professionals, and we are honored to be holding a 90-minute educational session just on CMT.

Our agenda will include, but is not limited to, insight on the importance of Patient Reported Outcomes in CMT patient care, therapy development, and the use of new technology for an international observational real-world data study to explore the impact of CMT on patients in the real-world setting.

Highlights from the "Voice of the Patient" weekend overview will be included as well as the importance of early and accurate diagnosis. Leaders in genetic testing will educate members on new and affordable testing options for our CMT patient community.

Additionally, tips from leading CMT Experts will be shared and dive deeper into the patient's condition through the use of CMT-validated clinical testing scales to enhance treatment options and patients' functionality. This may improve patient care management and expand to optimal targeted treatments.

HNF resources will enhance the neuromuscular clinicians ability to have access to other HCPs and allow for qualified referral resources for their patients. This integrated approach will provide more comprehensive care for the CMT patient. We will also highlight the necessary steps for transitioning pediatric patients to adult neurologists and other healthcare providers.

HNF, in collaboration with AANEM, has developed a CMT Assessment Test and CME accredited course to access and manage this multi-faceted approach to care for patients. Stay-tuned as HNF and AANEM announce the exciting new 2019 initiative!

HNF Presents at RARE Patient Advocacy Summit Hosted by Global Genes®

BY **JOY ALDRICH,** ADVOCACY DIRECTOR, HNF

The Annual RARE Patient Advocacy Summit, hosted by Global Genes, is in its seventh year and will take place on October 3-4 at the Hotel Irvine in Irvine, California. It is the largest gathering of rare disease patients, advocates and thought leaders worldwide. Global Genes is one of the leading rare disease patient advocacy organizations in the world. There mission is to eliminate the challenges of Rare Diseases.

For the first time, HNF will be part of the agenda and has been asked to speak on two sessions: Architecting Your Disease Community, as well as Data and Technological Innovation.

Allison Moore, HNF CEO and Joy Aldrich, HNF Advocacy Director will present "Update from the Community: Patient-Focused Drug Development Meetings" alongside Dr. Jill Jarecki, Chief Scientific Officer, Cure SMA. They will lead a discussion on the process, challenges and benefits of engaging in the effort to help understanding of the patients' disease experience and that their treatment priorities are included in drug development and review.

Allison Moore will present the new proprietary GRIN platform and technological innovations that are revolutionizing the type of data that can be collected when capturing specific patient reported outcomes. By utilizing Voice Activation Technology (VAT), HNF can better capture in the patient's voice PROs on pain and behavioral health. This type of data may enhance a better understanding of what patients are going through and a tool once validated to be incorporated into clinical trials. Kent Thoelke, Executive Vice President and Chief Scientific Officer from PRA Health Sciences, a leading CRO company will present on the challenges in conducting research in the rare disease populations. He will emphasis the importance of the patient perspective on clinical research and leveraging data to guide clinical trial design and the utilization of technology to create patient-focused virtual clinical trials.

These innovations provide a way not just to collect better data, but also to collect data that is truly patient-centric and may improve the future of drug development and clinical trials.

#2018GGSummit

HNF's CMT Centers of Excellence

The national network of HNF-designated Centers of Excellence (COE) provides patients with resources to find hubs of expertise in caring for and treating CMT, as well as locations where CMT research is being conducted. Our primary goal is to ensure care results in positive outcomes for each individual patient's clinical experience. We are honored to partner with these premier Centers and their leading experts to improve the future for people with inherited neuropathies.

CALIFORNIA

Cedars-Sinai Medical Center
127 S. San Vicente Blvd.
Advanced Health Sciences Pavilion, A6600
Los Angeles, CA 90048
Contact: Dana Fine
Phone: 310-423-8497
Email: Dana.Fine@cshs.org

Stanford Neuroscience Health Center Neuromuscular Clinic
213 Quarry Road, 1st Floor
Palo Alto, CA 94304
Phone: 650-723-6469.
Contact: Jennifer Fisher
Email: jnfisher@stanford.edu

CONNECTICUT

Hospital for Special Care
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WASHINGTON

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715 South Cowley Street, Suite 210
Spokane, WA 99202
Contact: Ann Cooper
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HNF Announces The First Pediatric Centers of Excellence for CMT

BY **ALLISON MOORE**, FOUNDER/CEO, HNF

There are those situations in life where you are destined to meet and for me, meeting Dr. Jahannaz Dastgir — "Naz for short" — was one of them. She is Director of the Pediatric Neuromuscular Medicine program at Goryeb Children's Hospital in Morristown, NJ and an incredible physician dedicated to helping our CMT pediatric patient community. Her primary passion in life is the care of children with childhood onset neuromuscular disorders. It has been very clear to her in clinical practice that though there has been great emphasis on research and treatment of muscular dystrophies, myopathies and motor neuron disorders, inherited neuropathies have been somewhat overlooked and require more attention.

Over the past 7 years, she has worked on a number of clinical trials including the Ionis trials for spinal muscular atrophy (which led to the development of the now FDA approved Nusineren), Sarepta trials for the now FDA approved Exondys51, and many others. While a clinical research fellow, she was also involved with the development of a clinical protocol for the Giant Axonal Neuropathy trial – the first intrathecal vector mediated gene therapy currently underway at the National Institutes of Health.

Q: Tell us about yourself?

A: A good friend of mine passed away from a brain tumor when I was in 2nd grade, and I was angry about her being taken away from me. It was truly from that point on that I became obsessed with the brain and neurology. In college, I majored in both neuroscience and philosophy in order to study how the nervous system works and how it thinks. I also had the opportunity to be a work-study student in Dr. Kurt Fishcbeck's neurogenetics lab at NIH, just around the exciting time that he discovered the mutation in the androgen receptor gene which causes spinal and bulbar muscular atrophy. In medical school rotations, I realized the joy and passion I had for the care of children because I spent a lot of time smiling in pediatric clinic. In pediatrics residency, I had the great fortune of treating children with CMT, SMA and muscular dystrophy. I also had nights on call with parents telling me about the frustrations of not having therapeutics in the neuromuscular field, which at that time was primarily palliative. I carried their concerns and grief with me as I slowly started to realize that I wanted to spend the rest of my life with and dedicated to these amazing individuals. I was inspired and given a reason to be who I am today.

Q: Why is CMT your passion?

A: CMT and all hereditary neuropathies are of great importance to me. I still have many cases that are undiagnosed, and many patients who have a diagnosis, but I have nothing to offer them as treatment. This is an area in major need of attention, and I find it imperative to work toward the goal of therapeutics. If SMA and duchenne can do it, why not CMT?

Q: Tell us what patients will experience when they come to pediatric neuromuscular program at Goryeb Children's Hospital.

A: Firstly, you will be established under my care, which guarantees exposure to some probably bad jokes in an effort to make you smile...prepare yourselves! Then, in all seriousness, I'll get cracking on the case. I will listen to your story and work to ensure that you have a genetic (or acquired) diagnosis. I will also make sure that you are plugged into the the multidisciplinary team that is necessary for your condition. In cases of CMT, the team is primarily myself, a physiatrist, orthopedic surgeon, physical and occupational therapists and an orthotist. All of our specialists are located in an easily accessible building. I will make sure that you are registered with your appropriate registries, such as the one offered by the Hereditary Neuropathy Foundation. And, if there are any clinical research trials available for your diagnosis, you will know about it. At the end of the visit, I will make sure that our team's plan is clearly explained to you as a family and to your pediatrician.

Q: How can patients make an appointment at the Atlantic Health System?

A: Please call 973-971-5700 to set up an appointment with me first. You can also feel free to reach out to me over email: **jahannaz.dastgir@atlantichealth.org.**

Our office location is:
55 Madison Ave.
Morristown, NJ 07960

AUTHOR AND TEAM CMT FOUNDER CHRIS WODKE RELEASES HER SECOND BOOK

BY **CHRIS WODKE,** FOUNDER TEAM CMT

Author and Team CMT Founder Chris Wodke has released her second book, "Soup Sunday—A Journey to Healthy Living". The book is a follow-up to her book "Running for My Life, Winning for CMT," which detailed her journey to run the Boston Marathon.

"Soup Sunday" is a compilation of the recipes published on her weekly blog. For most weeks of the year, soup is on the menu and cooked on Sunday afternoons, hence the title. The base of most of the soups is bone broth, which is a hot health trend right now. The book includes a recipe for creating this base.

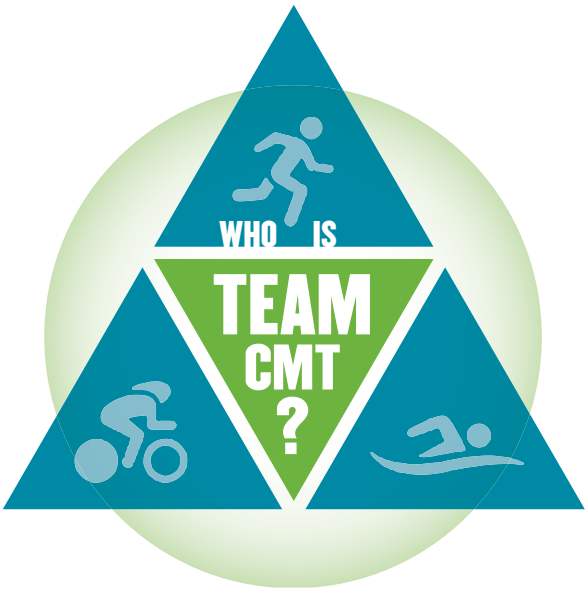
The book also discusses the author's experience with CMT. In addition, there is a section in the book about CMT and CMT resources. She hopes to extend her reach by raising awareness of CMT to a new audience. She notes, "Someone may buy a cookbook to make soup and learn a bit about CMT." The book is being published in electronic form to keep costs low. You can find both a Kindle and iPad version on Amazon.

A PDF is available on Ebay: CLICK HERE

If you desire a hard copy, you can print a PDF. All proceeds of the Ebay sales are being donated to the Hereditary Neuropathy Foundation. Wodke is part of the HNF TCS New York City Marathon team, which has a goal to raise $50,000, $10,000 per participant. Five TEAM CMT members will be participating in the event on November 4th.

If you wish to support Chris as she runs to support CMT Research, visit her fundraising page: CLICK HERE

To join TEAM CMT: CLICK HERE



Team CMT is a grassroots community fundraising initiative made up of hundreds of Team CMT members worldwide. Our team members have participated in events all over the world to raise awareness and funds for CMT Research. To date, Team CMT has raised over $500,000!

It's easy…there are three ways to participate:

HNF Community Events:

You can sign up for the annual TD Bank Five Borough Bike Tour, the second largest athletic event in NYC. HNF this year is also participating in the largest NYC athletic event for the first time, the TSC New York City Marathon. If we reach and/or exceed our fundraising goal, we likely will be accepted year after year. These two events are special and when participating as a charity partner, the VIP service is incredible! **To guarantee a spot for next year, contact courtney@hnf-cure.org**

These two events require a minimum fundraising goal, set by the sponsors and HNF.

Non-HNF Community Events:

You can simply join any event and wear your Team CMT singlet or shirt to raise awareness for CMT. There are thousands of events offered throughout the world for all types of athletic feats. Some Team CMT members have even developed their own personal challenges for the cause.

One Team CMT member completed the First Documented Solo Circumnavigation of the Big Island of Hawaii by Kayak.

Read about her journey:
CLICK HERE

These events may or may not require a minimum fundraising goal set by the sponsor. We encourage you that if you do want to fundraise for CMT, you contact the sponsor of the event or HNF so we can provide the resources and support for you to fundraise.

Other HNF Team CMT Events or To Host Your Own:

To date HNF has hosted walks, spin-a-thons, motorcycle rides, golf events, and more. These events can be made available to you for easy set up and support by us.

These events require an agreed upon fundraising goal, set by you and HNF, with 100% administrative support; at least one HNF staff or board member will be in attendance.

There is still time this year to sign up to host your own event or join one of our final two events of the year.

Join us for the inaugural "CMTee It Up" golf event in Evanston, IL on October 15, 2018.

Learn more:
CLICK HERE

You can also join our first ever Spin-for-Cure Spin-a-thon in Boston. **For more information contact allison@hnf-cure.org**

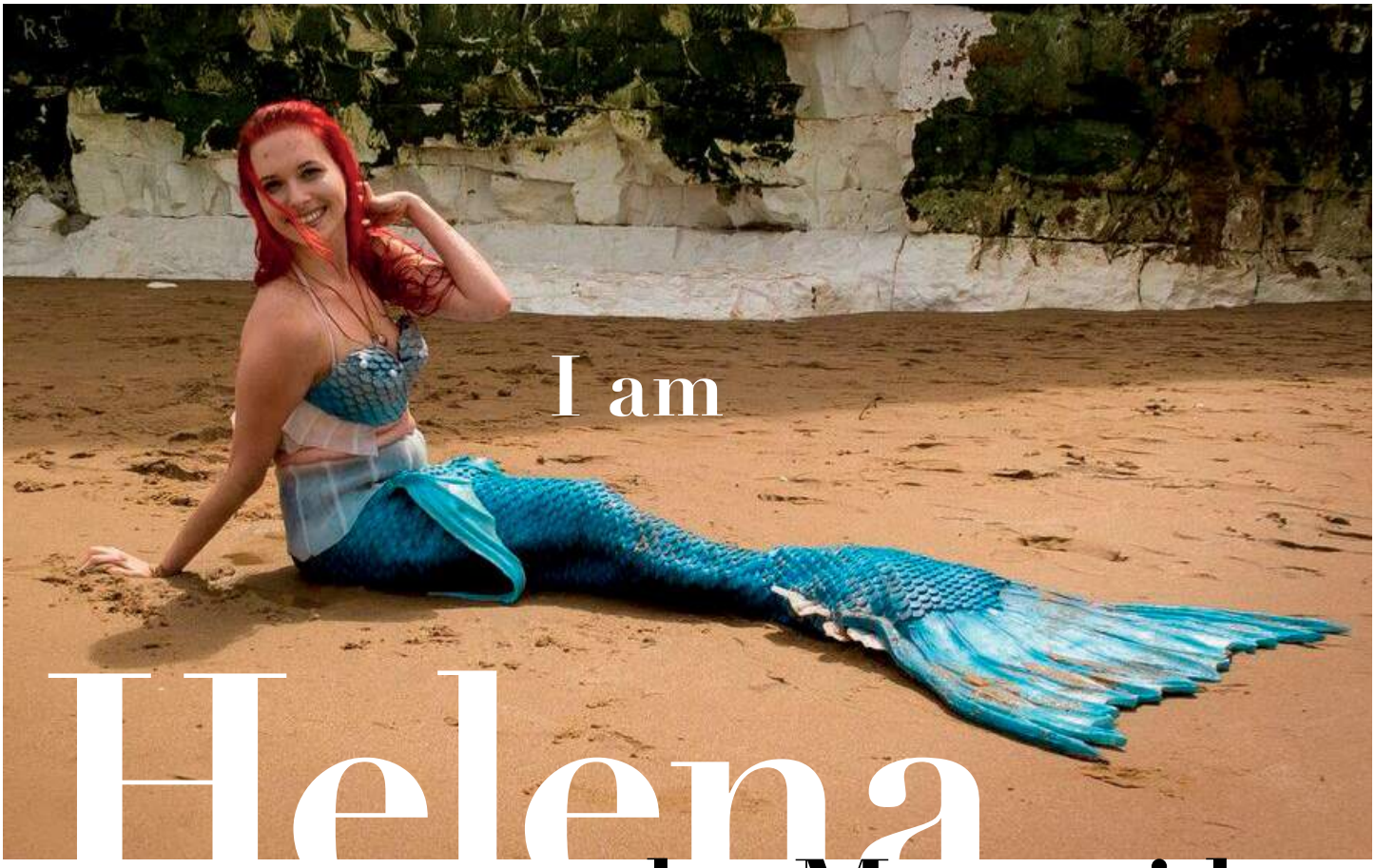
So don't wait, make a difference today and join Team CMT!

Kids can join too!

Team CMT Kids was developed to support children with CMT and other disabilities through our school outreach program.

Register your school/team and join us in the great cause! For more info: CLICK HERE

Fiona Doyle
Corks Ladies Mini Marathon



Michael G Photography

I am Helena the Mermaid

and I have Charcot–Marie–Tooth disease.

I am Helena the Mermaid, and I have Charcot–Marie–Tooth disease.

It's an extremely rare condition that I was born with, although no one else in my family has it. It did not affect me most my life and I was able to participate in sports, go on hikes, and live a normal happy life. I was not diagnosed till I was 14 years old when I had a spinal fusion for scoliosis. Before then, my high gait and clumsiness was generally brushed off on me just being a clumsy child.

Aside from my scoliosis, reflexes, and clumsiness, I was mostly unaffected by my CMT. When I reached my 20s, I fell in love, got married, and had a baby. Unfortunately, the pregnancy caused my CMT to drastically progress. After having my baby I became very imbalanced. I could still walk short distances, but now required a wheelchair for getting around. I was worried I would lose my strength and began physical therapy and workouts in the water.

I found mermaiding during this time. I needed a Halloween costume that didn't require much walking because of my balance, and mermaids don't walk. When I told my physical therapist my idea, she mentioned swimming as a mermaid (just a monofin at first, no tails till later) for therapy too and BAM my career was born!

Despite my working out, my disease progressed and walking became harder and harder while my swimming ability stayed consistent. That is until the start of the year when I caught a bad flu and bronchitis and my body did not handle it well. Now when I'm in the water I find I do not have the strength I need to properly kick and I panic. Therefore, I had to retire as a "swim mermaid" for safety reasons.

It was very upsetting to lose something that made me so happy and feel so beautiful. It upset me so much that I became depressed. I announced my retirement to my community and fans, but the response I received made me see how much of a positive influence I was and still can be. Both the mermaid community and disability community thought I should keep being a positive and magical influence and help bring more awareness to CMT and disabilities.

I still do dry events, including charity events for children with disabilities, and am currently traveling around the UK spreading awareness and education through the magic of mermaids. I try to be a positive influence to others in wheelchairs or with disabilities, and hope I can share beauty and magic with those who need it most.

I now have a magical life and have made friends with performers in many countries. From other mermaids, to fire artists, to belly dancers, I have a whole group of new spectacular friends who I love to work with and visit.

I can be found on Facebook, Instagram, and twitter under my name Helena the Mermaid.

Child Neurology Foundation (CNF) Leads a Collaborative Peer Support Work Group

BY **ESTELA LUGO,** MANAGER, MEDICAL OUTREACH, HNF

Earlier this year, Child Neurology Foundation (CNF) was awarded a Eugene Washington Patient-Centered Outcomes Research Institute (PCORI) Engagement Award to help convene multi-stakeholder advocacy groups, called the "Collaborative Peer Support Work Group", to conduct an environmental scan and assess the strengths and gaps of peer support in the larger child neurology community.

HNF was honored to be chosen by the CNF as the one of 11 advocacy groups. The Work Group's goal is to legitimize peer support as an effective intervention for supporting our caregivers that are caring for the pediatric neurological patient community.

"Patient advocacy organizations are providing peer support to an incredibly large number of families around the world who are facing the challenges of a rare disease diagnosis. Despite challenges of funding, strained staff, and the endless quest for information, patient advocacy organizations are providing families in distress with support, resources, and a sense of community."

–Amy Miller, Executive Director, CNF

HNF is excited to join this emerging movement. We recognize, on a daily basis, the gaps within the CMT Community that need to be addressed. Through this working group, we collaboratively have identified the need for social connections and support for the caregivers of children suffering from neurological diseases. Patients and families require connection with those who understand their challenges and who have already experienced what they are going through. There is simply no replacement for someone "who gets it."

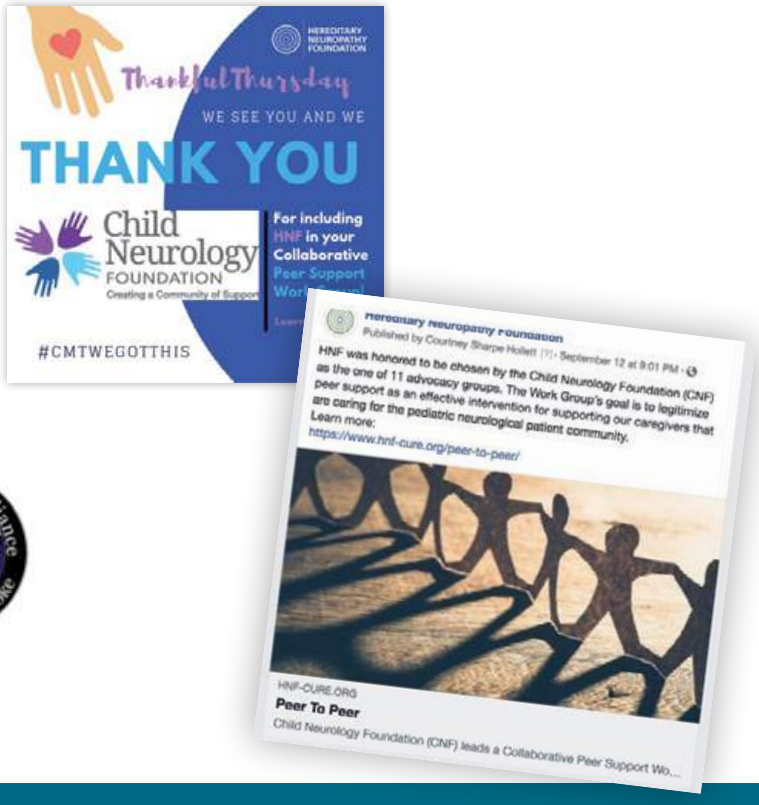
The distinguished advocacy groups participating are:

HNF officially partners with CNF to co-brand the Family Support and Empowered Program to bring this wonderful program to our CMT families. Stay-tuned as we continue to work with our CMT community caregivers and CNF to build a customized Peer Support Network. HNF is looking forward to the upcoming Peer Support Boot Camp hosted at the Global Genes RARE Patient Advocacy Summit on October 2nd in collaboration with CNF to learn best practices, key elements, and effective techniques for providing peer support to our CMT families that is both helpful and healthy.

In pursuing this common vision and working side by side with like-minded advocacy groups, HNF will be ensuring that families and caregivers have access to others who will listen and walk with them on their journey.

To learn more about the Collaborative Peer Support Work Group: CLICK HERE

Link to HNF site: CLICK HERE



Events

UPCOMING 2018 EVENTS

Save the date!

9/28
Externally-led PFDD Meeting
Hyattsville, MD

9/29
CMT Behavioral Health Summit
Hyattsville, MD

10/15
CMTee It Up
Evanston, IL

12/2018
Spin for a Cure
Boston, MA

"CMTee It Up"

A Golf Event to Benefit CMT Research

Join us for golf, dinner and giving as we tee it up for CMT, benefiting the Hereditary Neuropathy Foundation!

Location: Evanston Golf Club in Skokie, IL
Monday ~ October 15th

Schedule: 11:30 AM Check-In & Lunch
1:00 PM Golf Shotgun
5:00 PM Drinks, Dinner & Auction

This is the inaugural: "CMTee It Up" outing! Money raised will be used by the Hereditary Neuropathy Foundation to support Charcot-Marie-Tooth disease (CMT) research.

The silent auction will go live 10/1 – keep an eye on the site as items will be continuously added! If you have an item or experience to donate, please contact **cmteeitup2018@gmail.com**.

A note from Gretchen Cappiello, event chairperson:

"CMT has impacted three generations of my family — my father, my brother and me, two nephews, one niece and two of my three children. My family members have endured several surgeries and since the disease is progressive, unfortunately, we expect weakness and deterioration to continue. That said, we now have hope that a cure is possible — we are closer than ever before!

My brother Matt and I are board members of the HNF and are excited to be actively involved in supporting CMT research, which is showing promise for treatments and maybe even a cure. In fact, Matt and I are enrolled in a clinical drug trial that is showing promise and may be brought to market!"

This is a critical time and breakthroughs are on the horizon. We thank you in advance for supporting our event, "CMTee It Up", and look forward to a fun day of golf, camaraderie and exciting auction items!

Even if you don't golf, come for the drinks and dinner!

Register today: CLICK HERE

BH Kitchen, An Evening for Charity!

On Saturday, June 2nd, HNF hosted its annual New York City event, an exciting night filled with food, drinks, music, auction items and casino tables. Guests mingled and enjoyed dining and dancing, while admiring and bidding on unique silent auction items. One guest summed up the evening:

"The Hereditary Neuropathy Foundation event at BH Kitchen & Bar was a blast! It was a fabulous, casual, fun evening for a great cause. The Foundation shows promise for a cure and it is well worth supporting."

–Irene Dellis, HNF Supporter

Thanks to all who joined us, this special evening raised over $20,000 for HNF's programs! Proceeds will help support the FDA Externally-led Patient Focused Drug Development Meeting (PFDD) on September 28, 2018.

We were thrilled to see so many people with CMT (old friends and new ones), family members, and friends coming out to support CMT and HNF. We are grateful for their generosity. A special "thank you" goes out to the Loucas', Dellis' and Cyprus' family, owners of BH Kitchen.

Marguerite Loucas, restaurateur, is a childhood friend of HNF CEO Allison Moore and a board member of HNF.

"It was an honor and a privilege to host this great event in our newly renovated BH Kitchen & Bar. Allison and the team at HNF are doing amazing work to help find a cure for CMT disease. We are looking forward to hosting a bigger and better event next year!"

–Marguerite Loucas

It's not too late to donate: CLICK HERE

HEREDITARY NEUROPATHY FOUNDATION

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Hereditary Neuropathy Foundation
@CMTNeuropathy

PHARNEXT

Publication of this newsletter was made possible with the financial support of Pharnext.