



HEREDITARY  
NEUROPATHY  
FOUNDATION

JULY, AUGUST & SEPTEMBER

2016

# cmtupdate



## Patient-Centered

CHARCOT-MARIE-TOOTH SUMMIT

*New York City, October 6, 2016*

### Don't miss this landmark event!

On Thursday October 6, 2016, in New York City, HNF will host the first Patient-Centered Charcot-Marie-Tooth Summit.

The Summit will be focused on innovative content, bringing together top leaders in industry, research, treatments, and giving a voice to patients.

But, what do we mean by the term "Patient-Centered"?

"Patient-Centered" refers to the meaningful involvement of patients, caregivers, clinicians, and other healthcare stakeholders TOGETHER in a summit format.

This includes topic selection, panelist participation, audience engagement during Q&A sessions, and all the wonderful conversations in between speaker sessions

and at our evening reception!

We have an exciting agenda ([www.hnf-cure.org/cmtsummit-agenda](http://www.hnf-cure.org/cmtsummit-agenda)) which you don't want to miss! Be sure to check out the innovative and progressive topics that will be covered.

HNF believes that bringing all groups TOGETHER can influence research to be more patient-centered, useful, trustworthy, and ultimately lead to lifestyle guidelines and treatments for CMT/IPN, making a difference in your life TODAY.

This Summit is BY patients and FOR patients. We NEED patients to participate and make this Summit a...

### S-U-C-C-E-S-S!

**REGISTER NOW FOR HNF'S PATIENT-CENTERED CHARCOT-MARIE-TOOTH SUMMIT!**

[CLICK HERE!](#)

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**1-855-HELPCMT (435-7268)**

[www.hnf-cure.org](http://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.



## BREAKING NEWS

# ARE YOU A PATIENT WITH CMT 1A? NEW CLINICAL TRIAL OPPORTUNITY AVAILABLE FOR YOU!

For the first time investigators are looking for patients to participate in a pivotal Phase 3 clinical trial of Pharnext's lead investigational pleodrug, PXT-3003 for the potential treatment of Charcot-Marie-Tooth Disease Type 1A (CMT1A). The study which expects to enroll approximately 300 patients is taking place in the USA, Europe and Canada.

Nine of HNF's Centers of Excellence are now accepting patients for trial enrollment. Please visit: [www.hnf-cure.org/centersofexcellence](http://www.hnf-cure.org/centersofexcellence) to find out which centers in your area are recruiting and for contact information.

To learn about inclusion and exclusion criteria and for a complete list of sites visit: [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov) and enter protocol # NCT02579759.

Be sure to check back periodically as more sites are added, including Canada.

**BY GETTING INVOLVED YOU CAN HELP CHANGE THE FUTURE!**

**Please consider signing up—and help us get the word out to others so they, too, can get involved. Together, let's be a part of the first potential treatment for CMT!**

### HNF'S CENTERS OF EXCELLENCE

#### CALIFORNIA

*Currently accepting patients*

##### **Cedars-Sinai Medical Center**

127 S. San Vicente Blvd.  
Advanced Health Sciences Pavilion,  
A6600

Los Angeles, CA 90048

**Contact:** Phone: 310-423-1525

Email: Tami.Kendra@cshs.org

#### FLORIDA

##### **University of Miami**

Professional Arts Center (PAC)  
1150 NW 14th Street, 6th Floor  
Miami, FL 33136

**Contact:** Meri Jaime (for appointments)

Phone: 305-243-7400

E-Mail: Mjaime@med.miami.edu

#### MINNESOTA

*Currently accepting patients*

##### **University of Minnesota Health**

Maple Grove Specialty Clinic  
14500 99th Avenue North  
Maple Grove, MN 55369

**Contact:** Valerie Ferment

612-301-1535

E-Mail: ferm0016@umn.edu

#### WASHINGTON

*Currently accepting patients*

##### **St. Luke's Rehabilitation Institute**

St. Luke's Physiatry Practice  
715 South Cowley Street, Suite 210  
Spokane, WA 99202

**Contact:** Falishia Solano

Phone: 509-473-6706

#### CONNECTICUT

*Currently accepting patients*

##### **Hospital for Special Care**

Charles H. Kaman Neuromuscular  
Center

2150 Corbin Avenue

New Britain, CT 06053

**Contact:** Sharon McDermott

Phone: 860-612-6305

E-Mail: smcdermott@hpsc.org

#### KANSAS

*Currently accepting patients*

##### **University of Kansas Medical Center**

Landon Center on Aging

3599 Rainbow Boulevard, MS 2012

Kansas City, KS 66160

**Contact:** Aubrey Grover, LPN

Phone: 913-588-0683

E-Mail: agrover@kumc.edu

#### MISSOURI

*Currently accepting patients*

##### **St. Louis University Medical Center**

Department of Neurology

1438 South Grand Boulevard

St. Louis, MO 63104

**Contact:** Kathy Nesser

Phone: 314-977-4860

E-Mail: NesserK@slu.edu

#### FLORIDA

*Currently accepting patients*

##### **University of Florida (UF) Health**

2000 SW Archer Road  
Gainesville, FL 32610

**Contact:** Kelly Martin

Phone: 352-273-5550 or 352-265-8408

E-Mail: kelly.martin@neurology.ufl.edu

#### MASSACHUSETTS

*Currently accepting patients*

##### **Brigham and Women's Hospital**

Department of Neurology  
75 Francis St

Boston, MA 02116

**Contact:** Kristen Roe

Phone: 617-525-6763

E-Mail: kroe@partners.org

#### NEW YORK

*Currently accepting patients*

##### **Columbia University**

Department of Neurology

710 West 168th Street

New York, NY 10032

**Contact:** For clinical appointments

Phone: 212-305-0405

For research studies

Phone: 212-305-6035, ask for

Allan Paras.

# HNF – PATIENT-CENTERED CHARCOT-MARIE-TOOTH SUMMIT

## Agenda

7:30am – 8:15am

### CONTINENTAL BREAKFAST

8:15am – 8:30am

### OPENING REMARKS

8:30am – 9:00am

**KEYNOTE SPEAKER:** Culinary Nutritionist, Stefanie Sacks, MS, CNS, CDN and Author of *What the Fork Are You Eating? “An Action Plan for Your Pantry and Plate”*

Navigating nourishment and learning how to cook CAN make a difference in how you feel—small changes in food choice can be part of YOUR prescription for healing.

9:00am – 9:20am

**SPEAKER:** Kim Goodsell, CMT Patient, *“The Patient of the Future: Patient as Expert, Health Maker, and Collaborator”*

How an extreme athlete uncovered her own genetic flaw. When Kim Goodsell discovered that she had two extremely rare genetic diseases, she taught herself genetics to help find out why.

9:25am – 10:00am

**PANEL DISCUSSION:** Lori Sames, Founder of Hannah’s Hope Fund, *“Genotype and Phenotype: Managing Symptoms, The Role of Researchers/Clinicians”*

CMT has many different forms – each with different causes and symptoms. Lori Sames, “Fighter Mom” of a child with a form of IPN, champions gene therapy and shares how researchers and clinicians can help.

10:00pm – 10:20pm

### BREAK

10:20am – 11:00am

**PANEL DISCUSSION:** Tim Boyd, NORD’s Associate Director of State Policy and Advocacy *“Public Policy”*

Better AFO (leg-bracing) options, fast-tracking drug discoveries and clinical trials, applying for disability benefits, Medicare/Medicaid and services you may be eligible for. These pose a real challenge to CMT/IPN patients. How can we empower one voice for change in the system?

11:05am – 11:55pm

**SPEAKERS:** Lucia Notterpek, Ph.D., Robert Burgess Ph.D., Steven Gray Ph.D., and Sean Ekins, Ph.D., DSc *“Research Presentations”*

Hear the latest from researchers on translational drug discovery, clinical trials and more!

12:00pm – 1:00pm

**WORKING LUNCH:** Gary Barg, Editor in Chief, Today Caregiver Magazine, CMT patient and advocate, *“The Fearless Caregiver: The Caregiver’s Voice Matters and the Role of the Caregiver in Patient-Driven Research”*

Survey results show that 39% of adults, that’s 90 million Americans, are caregivers. Let’s discuss reducing the caregiver’s stress, learning ways to communicate effectively with healthcare providers, and the caregiver’s role in clinical trials, and more.

1:15pm – 2:00pm

**PANEL DISCUSSION:** Stefanie Sacks, Moderator *“Personalized Nutritional and Exercise Prescription for the CMT/IPN patient”*

How many times have we heard, “Exercise, but don’t overdo it”? “Eat more protein and less veggies and fruits”? We’ll discuss what works best from actual CMT patients, and nutritional and exercise experts.

2:05pm – 2:40pm

**PANEL DISCUSSION:** James Nussbaum, P.T., Ph.D., S.C.C., E.M.T. *“Gaps in Available Patient-Reported Outcome Measures and Barriers to Therapy Development”*

Discussion about the importance of the accurate assessment of how a patient improves from a baseline measurement and new technology tools to be used by clinicians to quantify change.

2:40pm – 3:00pm

### BREAK

3:00pm – 3:40pm

**PANEL DISCUSSION:** Florian P Thomas, MD, MA, PhD, MS; Chairman, Neuroscience Institute, and Director, Hereditary Neuropathy Center, Hackensack University Medical Center, *“The Squeaky Wheel Gets the Grease: The Art of Being a Successful Patient is to Know How to Get on Your Doctor’s Nerves and Feel Good About It”*.

The Chair of HNF Centers of Excellence speaks out on knowledge gaps among health care professionals, how to identify providers that are well versed in CMT, what patients should expect from their providers, and how they can advocate for themselves or loved ones.

3:45pm – 4:25pm

**PANEL DISCUSSION:** Tim Cote, M.D., MPH, CEO Cote Orphan *“Orphan Drug Clinical Trials: The Good, the Bad and the Benefits”*

Now is an exciting time! Finally there are clinical trials for CMT, but what does that mean for you? You need to know the facts. Dr. Tim Cote explains why orphan drugs are special and how being a rare disease impacts your clinical trial.

4:30pm – 5:00pm

**SPEAKER:** Michael Sereda, M.D., Professor of Neurology and Group leader in the Department of Neurogenetics, Max Planck Institute (MPI) of Experimental Medicine, Göttingen, Germany *“Translational Medicine in CMT: Update on Preclinical and Clinical Trials”*

Hear the latest on these exciting new potential treatments for CMT.

5:05pm – 5:25pm

**SPEAKER:** Robert Moore, husband to Founder/CEO of HNF *“The High-arched Foot: This is the Beginning? The Disease Awareness Challenge”*

Robert knows all too well the ins and outs of CMT/IPN’s. Over 90% of patients with CMT have a high-arched foot. Can this be the solution to the awareness problem?

5:25pm – 5:30pm

### CLOSING REMARKS

5:30pm – 7:30pm

### POSTER SESSION & EVENING RECEPTION



*with Summit Attendee*

# Jim Matthews

**Q: Tell us about yourself?**

**A:** My name is Jim Matthews and I'm a Licensed Clinical Social Worker in Indiana. I'm 59 years old and will be celebrating my 30th anniversary with my wife Cathy this year. We have two adult children. One lives with us and the other lives in a town about an hour away.

**Q: Why are you participating in the Patient-Centered Charcot-Marie-Tooth Summit?**

**A:** The first answer is about coincidence. My daughter and I will be visiting New York the week of the Summit, and neither of us have been to NYC. When I learned the Summit is being held the day after we arrive in New York, I knew I wanted to go.

I participate in a number of CMT-related sites, where I've met many wonderful people. Some of them will be attending the Summit and I'm looking forward to meeting them. I also hope to learn more about CMT, and what researchers say about future treatments and the genetic transmission of CMT.

**Q: What do you hope to take-away from the Summit?**

**A:** I hope to make new friends, strengthen existing friendships in the CMT community, and gain more knowledge about CMT causes and possible treatments.

**Q: Are you active in the CMT Community?**

**A:** I participate frequently on CMT Facebook forums. One, "Don't Throw in the Trowel," is a page for disabled gardeners. I also participate in a CMT support group in Indianapolis. Recently, I lead a discussion on chronic illness and grief, depression, and anxiety.

**Q: Are you planning to share news of the Summit with others and your health care professionals?**

**A:** Yes, I will share the information with the CMT support groups I'm a part of and with my healthcare team.

**Q: Why do you think others with CMT, or their caregivers, should participate in this Summit?**

**A:** I think any time people with shared circumstances can get together to commiserate, laugh, and cry about their situation, it's good for all involved. Plus, there promises to be a lot of great information presented at the Summit.

**Q: You and your daughter have never been to NYC. What are your plans during your first visit to the Big Apple?**

**A:** We plan to visit the Tenement Museum, Ellis Island, tour a part of New York with Big Apple Greeters, visit Central Park, see a Broadway/off-Broadway show, and eat at a real New York City deli! I'm open to any other ideas to add to our list.

Thank you for sharing your story with us, Jim! All of us at HNF are looking forward to meeting you at the Summit and helping you make your trip to NYC a most memorable one.

**FOR INFORMATION OR TO REGISTER FOR THE PATIENT-CENTERED CHARCOT-MARIE-TOOTH SUMMIT: [CLICK HERE!](#)**

# Quotes”

The first Patient-Centered Charcot-Marie-Tooth Summit is going to be a landmark event connecting the top leaders in industry, research, treatments, and giving a voice to patients.

Whether it's as a CMT patient, caregiver, scientist, friend, or family member, being part of this event means something special and unique to each of us. We asked our community members....

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## WHAT DOES ATTENDING THE FIRST PATIENT-CENTERED CHARCOT-MARIE-TOOTH SUMMIT MEAN TO YOU?

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Sean Ekins, Chief Science Officer, HNF

*“Attending the Summit means getting to connect the CMT patient, clinician, researcher, advocate, and community all in one place.”*

Lucia Notterpek, Scientific Advisory Board member, HNF

*“Hearing from the patients on how the disease affects their everyday life will help me in designing and analyzing experiments in the laboratory.”*

Kristin Gelzinis, Patient CMT4

*“Attending the Summit means connecting with practitioners, patients, and caregivers to educate and share ideas. Getting to meet new people and learn all about the new studies and other treatment available.”*

Chris Wodke, Founder Team CMT and CMT Patient

*“I am thrilled to be coming to the conference because I will get the chance to connect with so many others in the CMT community. I have connected with so many people through Facebook and Team CMT. It will be so great to meet them face to face and share our experiences.”*

Joy Aldrich, Advocacy Directory, HNF and CMT Patient

*“It's an opportunity that empowers me to actively fight this disease and make my voice heard regarding how CMT is affecting my life.”*

Matthew Downing, Board Member, HNF and CMT Patient

*“For me, it means getting a chance to guide research and therapies that could help me and my three kids fight against the progression of our CMT.”*

Kim Farren, Family Member

*“Joining together as a community to spread awareness and learn how I can support my family members that are affected with CMT and take away tools that can help me with my love ones emotionally, physically, and mentally.”*

# SUMMIT SPEAKERS



## KEYNOTE SPEAKER

# Stefanie Sacks

.....  
An Action Plan for Your Pantry and Plate  
.....

8:30 am – 9:00 am

The Hereditary Neuropathy Foundation is proud and privileged to introduce Stefanie Sacks, our keynote speaker at the first Patient-Centered Charcot-Marie-Tooth Summit on October 6, 2016 in New York City.

Stefanie Sacks, MS, CNS, CDN is a Culinary Nutritionist, author, consultant, speaker and food firebrand!

She has been studying food and healing for 30 years, has her Masters of Science in nutrition from Teachers College, Columbia University, is a Certified Nutrition Specialist, Certified Dietitian Nutritionist and is a graduate of the Natural Gourmet Institute for Health and Culinary Arts.

Stefanie works hands-on with individuals and groups seeking a healthier way of eating. For companies and organizations looking to do the right thing for their consumers and environment, Stefanie is the go-to- gal—her unique background in food and agriculture as well as health and wellness gives her the knowledge and skills to guide groups towards true food transparency, sustainability and ultimately health as a consultant, speaker and media expert.

Her clients range from chefs and others in the industry—including Jean Georges Vongerichten and Chef's Warehouse as well as Titan Tea, a food product company—as they aim to create a more transparent supply chain as well as bring real nourishment to their offerings. She is also allied with many consumer, health and environmental organizations locally and globally offering her expertise to several including United Nations, A Greener World and Environmental Working Group.

In addition, Stefanie was the co-starter of the very visible and successful #WhatTheFork petition at Change.org to push Whole Foods Market and Walmart to start selling ugly fruits and vegetables thus addressing massive food waste in the United States.

Her teaching experience is extensive. She is as a preceptor for Columbia University's Dietetic Internship program and was an adjunct professor in the graduate nutrition program at the University of Bridgeport. Sacks also served as faculty at Kripalu Center for Yoga & Health as well as Food As Medicine, the universally acclaimed professional training program. She has also

inspired many through workshops at Donna Karan's Urban Zen Center, various resort spas, wellness centers, schools and universities around the country.

Her book, *What The Fork Are You Eating? An Action Plan for Your Pantry and Plate* (Tarcher | Penguin Random House) provides an aisle-by- aisle rundown on how to shop and cook healthier. She is a firm believer that small changes in food choice can make big differences and is a sought after speaker, consultant and media guest expert on the topic of healthy choices, with frequent appearances on the Dr. Oz Show, PBS, Fox Media, and multiple radio programs.

She also hosted her own radio show, *Stirring the Pot*, on Hamptons NPR, WPPB 88.3FM for three years. Her extensive online and print contributions include The Huffington Post, Alternet, Family Circle, Prevention, fortune.com, foxnews.com and foxbusiness.com.

Sacks lives on the East End of Long Island with her husband, two very active young boys and Blossom, the family dog.

**HNF: How have you seen nutrition impact those with chronic illness and disease?**

**Stefanie:**

I revert back to my own experiences and see what changing my food choices over the years has done to my own health.

And professionally, I can only speak about what I see and I can truthfully say that I have helped hundreds of people (if not thousands), over the years, on navigating food choice and shifting dietary patterns to support health. When committed and compliant with my suggestions (and those of some of my trusted colleagues), my clients, students, readers and listeners tend to see a **great change in how they feel** even with small changes like removing all foods containing artificial ingredients and of course more complex ones like removing pro-inflammatory foods or following a specific “medical” diet.

**HNF: What role can nutrition play for CMT patients and their caregivers?**

**Stefanie:**

There is no research that I am aware of that focuses on CMT and food choice. However, Dr. Terry Wahls, an MD with multiple sclerosis (also a demyelinating disease) has done extensive research into the effects of the Paleolithic diet on improving symptoms.

I believe that educating CMT patients on how to navigate healthy food choices (within the context of what they can and can't do) as well as exploring a modified Paleolithic diet may be a good first step!

We can't wait to hear more from Stefanie at the Summit! Want to ask Stefanie your questions in person? Come join Stefanie and all of us at the first Patient-Centered Charcot-Marie-Tooth Summit October 6th in New York City.

You can read more about Stefanie and her mission on her website: [www.stefaniesacks.com](http://www.stefaniesacks.com)



# Kim Goodsell

.....  
The Patient of the Future: Patient as Expert, Health Maker,  
and Collaborator  
.....

9:00 am – 9:20 am

Kim Goodsell is the patient of the future. She is the expert. The maker. The collaborator.

In the years prior to her CMT diagnosis, Kim discovered she had another genetic disorder. She also has arrhythmogenic right ventricular cardiomyopathy (ARVC), an inherited heart disorder.

Advocating for her own health, Kim was able to harness the power of genetic information to devise a treatment plan that not only reduced, but reversed much of her disability with CMT.

Let's learn more about her journey in her own words:

It's been over stated for sure, but my do-it-yourself diagnosis of the confounding continuum of rare diseases I present: CMT - a disabling nerve disorder and ARVC - a rare life-threatening heart condition, has been said to mark an important watershed in medicine.

Dr. Eric Topol, one of the world's foremost physicians and medical visionary, publicly characterizes me as **"The Patient of the Future."** I am deeply committed to exploiting my health odyssey to help catalyze medicine's "Gutenberg moment" and the rise of the Patient of the Future: the Expert, the Maker, the Collaborator.

A UCSD college dropout, I earned an honorary Doctor of Medicine degree, tuition free, from the infamous Google University of Medicine. My googled research has been accepted for publication in the peer reviewed Journal of Cardiovascular Electrophysiology. With great humility, I have found myself at center stage of **the digital/genomic revolution in medicine**, participating with its vanguards in the co-production of medical intelligence.

Harnessing the power of genetic information, **I devised a successful treatment plan that not only attenuated, but reversed much of my disability.** The success of my treatment strategy is dismissed as anecdotal. I actively advocate for the scientific legitimization and the embrace of anecdotal evidence.

At the epicenter of the medical community's debate on the dangers and advantages of giving power to the people to direct and take control of their healthcare, **I co-star with Angelina Jolie** in the Medical Blockbuster Book Of The Year: *"The Patient Will See You Now - The Future of Medicine is in Your Hands."*

Defying the medical profession's expectations of today's "google and go" patient, I have won international acclaim and high praise as a "unique", "one-of-a-kind", and "extraordinary" patient.

I reject this characterization. It's socially disempowering, concealing an implicit suggestion that I am an outlier and will continue to be. In my community, the rare disease community, **greatness is commonplace.**

It's the people living with rare disease, chronic disease, and disability that are pushing the edges, driving research, and health innovation from the bottom up. This is where you see the future of healthcare.

**Greatness is not some rare DNA strand.** We are ordinary people, hackers, artists, and activists. We are creating a future of #HealthMadeByUs. My husband, CB, and I are members of a growing alliance of DIY #HealthMakers. We have spent the better part of our 35 years together roving through remote wildness perfecting the art of movement, engaged in what some might consider extreme endurance activities.

Today we are employing our art to restoring meaningful mobility to our vulnerable disabled community who are disproportionately poor. We are makers of ROVA, an award winning walker and founders of ROVA RealTime, a young California Benefit Corporation coming of age with the attitude that good design is a human right.

**HNF: How has CMT affected your life?**

**Kim:**

It has unleashed an element of genius and helped me find my greatness.

**HNF: What do you hope attendees take away from the Summit?**

**Kim:**

I'd like to leave you with a quote:

*"Somehow we've come to believe that greatness is a gift reserved for a chosen few ... for prodigies ... for superstars and the rest of us can only stand by watching. You can forget that! Greatness is not some rare DNA strand ... it's not some precious thing. Greatness is no more unique to us than breathing. We're all capable of it ... all of us!"*

Nike - The Jogger

We can't wait to hear more about her journey and what it takes to become a patient of the future!

You can watch Kim's video autobiography *ROVA REAL WARS: Episode 1 - Remembering the Future* - YouTube - Part Alice in Wonderland, part Star Wars, part Monty Python, part Looney Tunes, part music video, 100% inspirational medical epic, 100% DIY.



## Lori Sames

Genotype and Phenotype: Managing Symptoms,  
The Role of Researchers/Clinicians

9:2 5am – 10:00 am

Raising global awareness of inherited neuropathies is a daily challenge we face head on at the Hereditary Neuropathy Foundation. Recognizing and understanding the people living with these debilitating and sometimes fatal disorders are critical to developing the treatments and finding cures for these diseases.

One of these rare genetic diseases is Giant Axonal Neuropathy (GAN): an autosomal recessive genetic disorder caused by mutations on both copies of the GAN gene. The disease generally appears in early childhood, and progresses slowly as neuronal injury becomes more severe. Children with GAN don't survive past their teens or twenties.

HNF has the honor of knowing one such GAN patient, Hannah Sames. She was diagnosed with GAN at four years old, and now at age twelve, Hannah can no longer walk on her own.

Her mom Lori Sames, and her husband Matt, formed Hannah's Hope Fund (HHF) following Hannah's diagnosis in 2008. They work tirelessly to fulfill HHF's mission: to raise funds to support the development of treatments and a cure for GAN, and be the resource for doctors, scientists, and families around the world.

In 2012, Allison Moore met Lori at the World Orphan Drug Conference in Washington, D.C. When Allison met Lori, she was drawn to her determination in learning the science behind the rare disease affecting her daughter.

Ever since that first meeting, these two "Fighter Moms" have joined forces, sharing their organizations' mission, researching business models, and the most effective approaches to engaging industry, initiating collaborations, and most importantly, never taking no for an answer!

And all the hard work, networking, and fundraising is finally paying off for Hannah and her family.

Hannah is currently taking part in a clinical trial at the National Institute of Health in Maryland. She is the fifth child to take part in the clinical trial, which started in May of 2015. Hannah will stay at the National Institute of Health for 60 days following her injection.

*"We are so very happy today. The blueprint of Hannah's life changed today. The pages are blank and a new future is before her."*

— Lori Sames

According to Matt Sames, it takes about two years to realize full clinical benefit, but the four children who have already received injections are doing well. The children are experiencing more leg strength and better breathing.

HNF welcomes Lori as a Summit speaker, where she will lead a discussion on Genotype and Phenotype: Managing Symptoms, The Role of Researchers/Clinicians/HCP.

We look forward to hearing how this Fighter Mom is bringing hope for children with GAN and their families.

### More About Lori:

Lori Sames received a Bachelor's degree in economics from St. Michael's College, in VT, in 1992. She worked for IDX Systems Corporation as a computer software installation consultant for healthcare until 1996. She then became a Senior Project Lead for integrated healthcare delivery system installations.

Before becoming a stay at home mom in 2001, prior to the birth of her second child, Reagan, she lead web-based software installations on wireless networks, in the outpatient setting for Allscripts. She helped with her husband's Pet Lodge businesses from time to time, while caring for her three daughters until her youngest daughter, Hannah, was diagnosed with GAN in March of 2008.

From that moment forward her life has been dedicated to funding treatments and an eventual cure of GAN, and making lasting memories for her family.



## Tim Boyd

Public Policy

10:20 am – 11:00 am

The availability of affordable healthcare is an issue that affects each and every one of us. This is even more critical for patients with rare diseases.

HNF has partnered with NORD to help us find the solutions and realize a brighter future for those living with CMT and other INs. We've invited Tim Boyd, Associate Director of State Policy, National Organization for Rare Disorders (NORD) to lead a discussion on Public Policy at the Summit.

According to Tim, the cost and availability of health care services for patients with a rare disease is dependent upon state-based policy decisions. The design of public and privately-funded health plans does not always take the needs of the rare community into account in determining benefits and costs.

Tim has served as NORD's Associate Director of State Policy and Advocacy since 2015, and is responsible for their policy activity in all 50 states. Prior to that, he worked on federal and state public policy for eight years with the AIDS Healthcare Foundation (AHF). Tim earned his B.A. in Political Science

from the University of California Santa Barbara and is an M.P.H. Candidate (2017) George Washington University Milken Institute of Public Health.

His goal for the Summit is to provide attendees with a sense of NORD's perspective on state-based health care issues and inform them about ways they can engage with us on various initiatives.

We look forward to hearing from Tim and the panelists on what is sure to be an interesting and spirited discussion!

You can connect with Tim here: [www.rarediseases.org](http://www.rarediseases.org)



## Lucia Notterpek, Ph.D.

### Research Presentations

11:05 am – 11:55 am

The Hereditary Neuropathy Foundation is incredibly fortunate to have a group of ambitious and dedicated leaders on the front lines of Charcot-Marie-Tooth research. These inspiring researchers will be presenting their latest studies and what's on the horizon in CMT research at the Patient-Centered Charcot-Marie-Tooth Summit.

We would like to introduce you to Dr. Lucia Notterpek, an HNF Scientific Advisory Board member. She received her B.A. in Anatomy-Physiology from the University of California at Berkeley. She obtained her Ph.D. in Neuroscience at the University of California at Los Angeles working with Dr. Leonard H. Rome. Her postdoctoral training was under the guidance of Dr. Eric Shooter at Stanford University. Currently, Dr. Notterpek is Professor and Chair in the Department of Neuroscience at the McKnight Brain Institute of the University of Florida.

Dr. Notterpek investigates how the loss of glial insulation around axons, called myelin, contributes to the pathogenesis of hereditary and age-related neural disorders. She studies diseases that are specifically linked with defects in myelin including peripheral neuropathies, such as CMT and multiple sclerosis.

A major effort of Dr. Notterpek's lab focuses on approaches to maintain healthy myelin during lifespan and/or restore it in disease paradigms. The laboratory is equipped with models and reagents, including small molecule therapeutics and genetic models to attain these goals.

Other areas of active investigation include the optimization of lipid nanoparticles as therapy delivery vehicles for neural disorders.

Dr. Notterpek is the recipient of the 2004 Jordi Folch-Pi Memorial Award from the American Society of Neurochemistry, awarded to a young scientist for research excellence. She has authored and co-authored over 65 publications, reviews, and book chapters.

She is actively involved in the educational and research missions of the College of Medicine at the University of Florida.

Her research efforts have been supported by the NIH, the National Muscular Dystrophy Association, the National Multiple Sclerosis Society, the Facial Pain Foundation, and the Hereditary Neuropathy Foundation.

### HNF: How has CMT affected your life?

#### Dr. Notterpek:

I do not have CMT in my family, but I know people with the disease. Learning about the burden of the disease on the patients and their families had a major impact on my career decision, which has been committed to research on CMT for over 20 years.

### HNF: What do you hope attendees take away from the Summit?

#### Dr. Notterpek:

We will all gain new knowledge from different perspectives on issues related to CMT. I hope we will be even more energized in working together across the patient, the caretaker, and research communities in our efforts to alleviate the burden of CMT.

### HNF: Anything else you'd like to share with the community?

#### Dr. Notterpek:

I highly value my interactions with the patient population. Learning about the specifics of the disease from patients impacts my thinking in experimental design as well as in data interpretation.

We thank Dr. Notterpek for her commitment and dedication to making a difference for the entire CMT community!



## Robert Burgess, M.D.

### Research Presentations

11:05 am – 11:55 am

We are fortunate to have the leading CMT researchers share their latest progress at the Summit. HNF has invited Dr. Robert Burgess, a member of our scientific advisory board, to present his latest research.

Dr. Robert Burgess is a Professor at The Jackson Laboratory in Bar Harbor, Maine, where he has directed a research group since 2001. Dr. Burgess received his B.S. in Biochemistry from Michigan State University, his Ph.D. in Neuroscience from Stanford University, and was a postdoctoral fellow at Washington University, St. Louis prior to joining Jackson.

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A major focus of the Burgess lab is developing mouse models of inherited peripheral neuropathies using genome editing techniques. These animal models are then used to better understand disease mechanisms and for preclinical testing of therapeutic approaches. This research is funded by several agencies including the National Institutes of Health and the Muscular Dystrophy Association.

Dr. Burgess is on the scientific advisory board of the Hereditary Neuropathy Foundation, and also serves on the Cellular and Molecular Biology of Neurodegeneration grant review panel for the National Institutes of Health. Dr. Burgess is married with two (nearly) grown children, and enjoys living next door to Acadia National Park.

**HNF: Why did you choose to present your research at the Summit?**

**Dr. Burgess:**

I am a CMT researcher, but what started as a scientific interest has grown as I have integrated into the CMT research community and become more involved with patients and advocacy groups such as the HNF.

**HNF: How has CMT affected your life?**

**Dr. Burgess:**

I now appreciate this is an important but often overlooked condition, with a growing and dedicated community seeking a cure.

**HNF: What do you hope attendees take away from the Summit?**

**Dr. Burgess:**

I hope that patients will take away that there is a focused research effort trying to develop treatments for CMT. While more effort and involvement would always be welcome, the real challenge is the underlying biology. These are difficult diseases to treat and we do not have a well-trodden path to follow in terms of developing therapies. Progress is being made and many people are truly dedicated to this problem.



## Sean Ekins, Ph.D., DSc.

.....  
Research Presentations  
.....

11:05 am – 11:55 am

Leaders in Charcot-Marie-Tooth research need to be progressive visionaries, willing and able to push the boundaries to explore every opportunity available to advance therapeutics and clinical trials for CMT.

HNF has the great honor to have Dr. Sean Ekins lead our research operations as our Chief Scientific Officer.

Dr. Ekins is the co-founder and CEO at Phoenix Nest, a company focused on Sanfilippo Syndrome rare disease drug discovery. He is also the founder and CEO at Collaborations Pharmaceuticals, Inc., which is focused on rare and infectious diseases.

He graduated from the University of Aberdeen, receiving his M.Sc., Ph.D. and D.Sc. in Clinical Pharmacology. He was a postdoctoral fellow at Lilly Research Laboratories. He has worked as a senior scientist at Pfizer, Lilly, Associate Director of Computational Drug Discovery at Concurrent Pharmaceuticals Inc. (now Vitae Pharmaceuticals Inc), and Vice President of Computational Biology at GeneGo (now Thomson Reuters).

Dr. Ekins is Adjunct Professor, Division of Chemical Biology and Medicinal Chemistry, UNC Eshelman School of Pharmacy, University of North Carolina at Chapel Hill, Adjunct Associate Professor, School of Pharmacy Department of Pharmaceutical Sciences, University of Maryland, and Adjunct Professor in the Department of Pharmacology at Rutgers University– Robert Wood Johnson Medical School, Piscataway, NJ.

He has authored or co-authored over 270 peer reviewed papers and book chapters, as well as edited four books and a book series on technologies for the pharmaceutical industry. He has been awarded eleven NIH grants as a primary investigator.

Dr. Ekins is interested in the application of computational approaches to big and small data for rare and neglected disease drug discovery. His hobbies include cycling and hunting for records.

**HNF: What should attendees know about CMT research today?**

**Dr. Ekins:**

As the CSO of HNF, I think it's important to highlight what we are doing to fund science on this rare disease.

**HNF: How has CMT affected your life?**

**Dr. Ekins:**

I have a better appreciation for those living with the disease as I interact with them.

**HNF: What do you hope attendees take away from the Summit?**

**Dr. Ekins:**

I hope attendees see that we are hopefully at a turning point with so much great academic science and so many companies actively working on treatments. The Summit will provide the big picture view of what is going on.

**HNF: Anything else you'd like to share with the community?**

**Dr. Ekins:**

I look forward to personally meeting as many of you as possible!



## Gary Barg

The Fearless Caregiver: The Caregiver’s Voice Matters and the Role of the Caregiver in Patient-Driven Research

12:00 pm – 1:00 pm

Those caring for patients living with CMT are invaluable members of our community. Caregivers are on the frontlines every day, helping patients manage their daily struggles and challenges of living with CMT.

These selfless people need to be recognized for their commitment and dedication, and deserve their own advocacy platform and a strong, influential voice to represent them.

That undeniable voice for the caregiver is Gary Barg.

Noted speaker, author, and publisher on caregiving issues since 1995, Gary Barg is the CEO and Editor-In-Chief Caregiver Media Group, and Founder and Editor-In-Chief of the first national magazine for caregivers, Today’s Caregiver. He is also credited with creating the original online caregiver community, caregiver.com.

But he didn’t stop there! Gary created The Fearless Caregiver Conference, uniting caregivers across the world to share their knowledge, experience, and priceless wisdom. He is a published author of two books, “The Fearless Caregiver” and “Caregiving Ties That Bind.”

His many accomplishments and awards has earned Gary the spotlight, interviewing with highly recognized media outlets such as The Today Show, Bloomberg Radio Network, Time Magazine, The Wall Street Journal, and USAToday.

Gary has been a keynote speaker for many events across the U.S including:

- The Huntington’s Disease Society of America
- Massachusetts Multiple Sclerosis Society
- The Sharing Wisdom Conference with Leeza Gibbons
- World Congress on Disabilities
- Living Longer/Living Better Conference

And Gary has Charcot-Marie-Tooth.

Although his busy schedule leaves little time to enjoy reading and traveling with his dog, Gary has been incredibly gracious to take the time to join us as one of our featured speakers at the first Patient-Centered Charcot-Marie-Tooth Summit.

**HNF: Why did you choose your particular topic for the Summit?**

**Gary:**

I think it’s very important to discuss all partners within patient centered research outcomes, and that includes the interaction between caregivers and loved ones.

**HNF: How has CMT affected your life?**

**Gary:**

CMT affects my life the more I age. I’m much more conscious of taking care of myself, and getting as much exercise as I am capable of handling.

**HNF: What do you hope attendees take away from the Summit?**

**Gary:**

Learn all you can from the experts and providers who are all dedicated to supporting people living with CMT/IN. Above all, we should take the time to learn from one another.

**HNF: Anything else you’d like to share with the community?**

**Gary:**

You are not alone. Stay involved and don’t isolate yourselves, for the sake of what you can learn from others living with CMT/IN, as well as what you can teach.

We can’t wait to hear Gary at the Summit, as he imparts his knowledge and experience to an audience eager to learn more about caregiving and CMT.

Want to learn more about Gary or have a question for him?

Website: [Caregiver.com](http://Caregiver.com)



## James Nussbaum, P.T., Ph.D., S.C.C., E.M.T

Gaps in Available Patient-Reported Outcome Measures and Barriers to Therapy Development

2:05 pm – 2:40 pm

HNF invites Dr. James Nussbaum, PT, PhD, SCS, EMT, to lead us in a discussion on “Gaps in Available Patient Reported Outcomes and Barriers to Therapy Development” at the Summit.

Dr. Nussbaum is the Clinical and Research Director of ProHealth & Fitness PT OT, a physical and occupational therapy organization in New York City. He founded the organization in 2000 in an effort to provide skilled rehabilitation services to patients with all levels of abilities and disabilities.

His career was fueled by his experiences as a physical therapy patient, working for Johnson & Johnson as a health and fitness specialist, and as a volunteer Emergency Medical Technician. James returned to school after working at Johnson & Johnson and

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graduated from Touro College with a Masters of Science degree in Physical Therapy, and later earning his PhD in Pathokinesiology. He is also a board-certified Sports Certification Specialist.

Dr. Nussbaum sits on numerous clinical and academic advisory boards, and is an active member of the NYS Task Force on Fall Prevention. He currently is a clinical instructor for numerous Physical Therapy schools, and teaches biomechanics, human gait and kinesiology at Yeshiva University's Stern College.

His clinical and research focus has been on the effects of innovative therapy interventions in medically complex patient populations. He has numerous ongoing IRB clinical trials, collaborating with many NYC physicians, hospitals, and agencies. Dr. Nussbaum has lectured and presented his research at medical conferences throughout the US and around the world.

He has a particular interest in finding ways to enhance and document patient improvements in activities of daily living, mobility, balance, function, and most importantly, quality of life. In 2014, he became one of the first 3 certified AposTherapy providers in the US and is proudly active in educating physicians and patients alike.

Dr. Nussbaum has been the recipient of numerous awards and designations and is passionate about improving the way people live through clinical care, research, and education.



## Florian Thomas, M.D., M.A., Ph.D., M.S.

The Squeaky Wheel Gets the Grease: The Art of Being a Successful Patient is to Know How to Get on Your Doctor's Nerves and Feel Good About It

3:00 pm – 3:40 pm

We must all become our own best health advocates.

For those living with Charcot-Marie-Tooth disease and for their caregivers, being the leading advocate for your health and wellness is essential to getting the right care and treatment you deserve.

He will be speaking about the knowledge gaps among health care professionals, how to identify providers that are well versed in CMT, what patients should expect from their providers, and how they can advocate for themselves or loved ones.

CMT has been a big part of Dr. Thomas' professional career for over 25 years. After obtaining his medical degree and an M.A. in psychology in 1982/1983, Dr. Thomas completed his training in Neurology at Case Western Reserve University in 1987, and in Neuroimmunology-Peripheral Nerve Disorders at

Columbia University in 1990.

He earned his Ph.D. in Molecular Biology at McGill University, and joined St. Louis University in 1995. Since 1999, he has engaged in clinical trials and recently obtained a M.S. in Health Outcomes Research.

In September 2016, Dr. Thomas will join Hackensack University Medical Center as Chairman of the Neuroscience Institute and Director of the Multiple Sclerosis and Hereditary Neuropathy Centers.

His research and experience with CMT includes:

- Columbia University: Described specific microscopic sural nerve abnormalities in CMT1B.
- CMTA: Chaired grant review committee for 4 years.
- St. Louis CMTA Support Group: Co-led for 15 years.
- Novel gene mutation: In 2006, he reported a novel gene mutation as the cause of one subtype of CMT (DI-CMT C).
- Pharnext Clinical Trial: In 2016 he enrolled the first patient in the Pharnext clinical trial for CMT1A.

### **HNF: Why did you choose your particular topic for the Summit?**

**Dr. Thomas:**

People who live with a chronic illness are more likely to live well, if they know a lot about it and can advocate for themselves.

### **HNF: How has CMT affected your life?**

**Dr. Thomas:**

For 20 years, I have accompanied the family whose disease mutation I identified. I visit the 94 year-old family patriarch every year during our family vacation on Lake Michigan. I have seen his grandchildren grow up, develop symptoms of CMT, and wonder if their children have CMT. This close relationship has given me a much better understanding of what it means to live with CMT.

### **HNF: What do you hope attendees take away from the Summit?**

**Dr. Thomas:**

Attendees will acquire an in-depth understanding of how patients can empower themselves to be active partners in their own care, how to consider the pros and cons of engagement in clinical trials, and how to maximize resilience and live well with a disease.

Dr. Thomas lives with his wife and their blended family with 5 children between the ages of 12 and 23. Dr. Thomas' wife founded a magazine dedicated to artists in St. Louis. His daughter is an undergraduate at Fordham Lincoln Center, not too far from the Summit location.

We are looking forward to this engaging topic that is sure to drive a spirited discussion at the Summit!



## Timothy Coté, M.D., M.P.H., C.E.O.

Participating in a Clinical Trial: The Good, the Bad and the Benefits

3:45 pm – 4:25 pm

Dr. Timothy R. Coté is a leading national regulatory expert in orphan drug development. With 23 years of Federal service at the FDA, NIH, and CDC, Dr. Coté recently served as the Director of the FDA Office of Orphan Products Development (OOPD) from Sept 2007 through May 2011.

In this role, he implemented the Orphan Drug Act and personally signed decisions on 1400+ orphan drug designation applications. An anatomic pathologist and medical epidemiologist, he has published 80 peer-reviewed articles on areas as diverse as HIV/AIDS-related malignancies, typhoid fever epidemics, and the impact of bicycle helmet laws on injury statistics.

Dr. Coté is founder and CEO at Coté Orphan; he directs and trains staff to create compelling regulatory submissions that are finely tuned to each client's business strategy and to the unwritten rules within the FDA.

Dr. Coté received a bachelor's degree from Syracuse University, a medical doctorate from the Howard University College of Medicine, and a master's degree in Public Health from Harvard School of Public Health.



## Michael Sereda, M.D., Ph.D.

Translational Medicine in CMT: Update on Preclinical and Clinical Trails

4:30 pm – 5:00 pm

We've invited HNF Scientific Advisory Board member Dr. Michael Werner Sereda, M.D., Ph.D, to discuss his latest research

on Charcot-Marie-Tooth at the Summit.

An HNF SAB member since 2015, Dr. Sereda is a Professor of Neurology at the University Medicine Göttingen in Germany (UMG) and Independent Group Head at the Max Planck Institute of Experimental Medicine (MPIEM).

His domain of research includes Schwann cell biology, disease mechanisms, experimental therapy and clinical (Phase II and III) trials, outcome measures, and biomarkers of CMT.

Dr. Sereda serves as the Coordinator of the German CMT-NET network on Charcot-Marie-Tooth disease, and has ties to the German patient organisations (DGN), with CMT-France, CMT-Rete in Italy.

### HNF: What is the focus of your CMT research?

#### Dr. Sereda:

We pursue a basic research interest in glial cell biology, axon-glia interaction, and mechanisms of diseases of the peripheral nervous system (PNS). We have generated a transgenic rat model of the most frequent human neuropathy, Charcot-Marie-Tooth disease type 1A (CMT1A). This disease is associated with a partial duplication of chromosome 17 which leads to an overexpression of the tetraspan protein PMP22.

Transgenic "CMT rats" expressing additional copies of this gene share characteristic clinical features of the human disease, including muscle weakness, reduced nerve conduction velocities, and marked Schwann cell hypertrophy resulting in onion bulb formation. The CMT rat allows a better understanding of the cellular disease mechanism operating in human CMT1A, and is helpful in the analysis of modifier genes, epigenetic factors, and in the evaluation of experimental treatment strategies.

In an attempt to translate findings from the animal model to humans, we have recently identified biomarkers of disease severity in CMT1A patients. We are currently validating markers in patients across Europe which should help us to perform clinical trials in the near future. Moreover, we are involved in clinical (Phase II and III) trials in CMT1A.

### HNF: What do you hope attendees take away from the Summit?

#### Dr. Sereda:

I hope to increase awareness and participation in CMT-NET: a network and news hub on CMT research for doctors, scientists, and patients.

For more information on Dr. Sereda and his research, please visit:

Max Planck Institute of Experimental Medicine:

<http://bit.ly/2buoE6R>

CMT-NET: [www.cmt-net.de/](http://www.cmt-net.de/)



## Robert Moore

The High-Arched Foot: This is the Beginning? The Disease Awareness Challenge

5:00 pm – 5:25 pm

Did you know?

Multiple Sclerosis is the most common autoimmune disorder affecting the central nervous system, with more than **2.3 million people** affected worldwide.

Charcot-Marie-Tooth disease is the most commonly inherited peripheral nerve disorder, and affects about **2.8 million people** worldwide.

If you walked up to a group of people in your town, your city, your school, or your neighborhood...and asked them if they ever heard about MS, the majority would say “Yes.”

Now ask them if they ever heard about CMT...the majority would say “What?”

Even though the incidence of CMT is greater than that of MS, CMT goes largely unrecognized. Patients go years without a proper diagnosis, HCPs don't have the knowledge to help, and researchers are faced with limited information to develop drug therapies and clinical trials for CMT patients.

It's more important than ever to keep pushing the boundaries of raising awareness within the CMT patient and HCP communities. Without awareness, we don't have a voice.

And this Summit speaker has something to say.

Robert Moore, husband to Allison Moore, CEO and Founder of HNF, will be discussing the challenges of disease state awareness at the Patient-Centered Charcot-Marie-Tooth Summit.

Let's hear more from Robert:

**HNF: Why did you choose your particular topic for the Summit?**

**Robert:**

The biggest challenge for those living with CMT is bringing awareness of their CMT symptoms to their HCPs. I've seen Allison go through countless visits with medical professionals that only dismissed her symptoms. By raising awareness of the disease to the medical community, patients will have a fighting chance to get diagnosed quickly and correctly.

**HNF: How has CMT affected your life?**

**Robert:**

Watching Allison have trouble walking, or performing simple tasks such as buttoning a shirt or opening a bottle, has given me a very different perspective for anyone living with a chronic debilitating disease. She walks with leg braces, but will still suffer frequent falls, often resulting in broken bones, and sometimes a broken spirit.

But that has not slowed Allison down at all! She is a remarkable force of determination, persistence, and strength.

**HNF: What do you hope attendees take away from the Summit?**

**Robert:**

I hope attendees take away a renewed sense of commitment to raising CMT awareness. Whether it's as a patient, caregiver, or HCP, let's make a promise to be 100% committed to meeting the CMT awareness challenge head on!

## ABOUT THE SUMMIT VENUE

# 3 West Club, New York City

BY COURTNEY HOLLETT FUNDRAISING COORDINATOR, HNF

The Hereditary Neuropathy Foundation has selected historic 3 West Club to hold the first Patient-Centered Charcot-Marie-Tooth Summit on October 6, 2016.

The 3 West Club is a best-in-class event space and boutique hotel nestled in the heart of Midtown Manhattan. Located in an historical building, you instantly get that classic “Old New York” feel when you walk through their doors.

3 West Club is just off 5th Avenue, only steps away from Rockefeller Center and Radio City Music Hall.

Unique styling, prime location, and that classic New York atmosphere make 3 West Club the perfect place to hold the first Patient-Centered Charcot-Marie-Tooth Summit!

**FOR MORE INFORMATION:**

3 West Club  
3 West 51st New York, NY 10019  
(212) 582-5454  
marketing@2westclub.com

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Partnering on programs to increase the awareness around CMT disease and support the development of a stronger CMT patient and research community

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Partnering to increase awareness of the importance of early and accurate diagnosis of the various types of CMT and related inherited neuropathies (INs) and a proud sponsor of the Patient-Centered Charcot-Marie-Tooth Summit

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Partnering to increase awareness of CMT among the medical community and a proud media sponsor of the Patient-Centered Charcot-Marie-Tooth Summit

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# SEPTEMBER AWARENESS MONTH

## Charcot-Marie-Tooth

# MOON RUN

Sitting on the sidelines isn't something Estela ever planned on doing. Diagnosed with CMT when she was just 4 years-old, Estela had to opt out from participating in sports, dance recitals and simply running around with her friends and peers.

As an adult and mother of two children, she missed afternoons spent chasing her daughter around the yard, playing catch with her son and many other family adventures.

Estela is not alone. This is what CMT can look like for the 2.6 million people affected by this progressive disease.

Although CMT took away Estela's ability to run, it did not take away her dream or determination to find another way.

### That day has come!

After 28 years, Estela was able to run again within a few minutes of stepping onto an Alter-G anti-gravity treadmill. This innovative technology seals the patient into an air-filled bag and reduces a patient's body weight by 80%. While helping regain mobility, the Alter-G increases strength, speed, and enhances range of motion with natural movement...all while minimizing the risk of injury!

*"It was an incredible moment to say the least. I knew I had found something amazing that could help so many others affected with CMT or similar conditions."*

This experience sparked the idea for the "CMT Moon Run" fundraiser. In hopes of spreading the word about this incredible technology, we want people to run during the month of September, CMT Awareness Month.

Run outdoors, run in your gym, run in a pool, and if you have CMT, get on an Alter-G!

This is groundbreaking technology for CMT patients. We want to dive in deeper and fund research based on our encouraging results! All donations collected for your Moon Run will go towards funding our fitness-based clinical study on the Alter-G and CMT.

Ready To Run and spread awareness this September?

### It's easy!

VISIT THE CMT MOON RUN DONATION PAGE AND REGISTER TODAY!  
TO REGISTER: [CLICK HERE!](#)



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