

CMT RESEARCH + PATIENT RETREAT

2 Days. 1 Event. Endless Possibilities...

Day #1: CMT Clinical Trial Readiness Summit

Day #2: CMT Patient Retreat for Health + Wellness

Event Dates: April 24-26

Location: Nashville, TN



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Overview & Agenda

Join us for the **2025 CMT Summit + Retreat** in Nashville! This transformative, two-day event merges cutting-edge clinical trial readiness with empowering health and wellness programming for patients and their care networks. We're uniting top-tier researchers, industry leaders, regulatory professionals, and the CMT community in a shared mission...

Accelerating + De-Risking CMT Clinical Trials.

CMT Clinical Trial Readiness Summit: Sessions are designed for clinicians, researchers, industry professionals, regulatory agencies, and patients participating in research!

Wednesday, April 23:

- **Clinical Evaluator Training *Ages 8-70:** Patients needed to participate in Clinical Evaluator Training. *Please arrive by Wednesday morning to participate and plan for additional Thursday Assessments as well.

Thursday, April 24 - All CMT Patients Wanted *Ages 8-70:

- **BioSensics Wearable Study + Clinical Evaluator Training Study:** Don't miss your chance to participate in this cutting-edge digital wearable study! New participants will undergo a clinical evaluation during a 1-hour on-site appointment and receive a lightweight pendant and wrist device to wear for 2 weeks. This innovative study helps advance CMT research and clinical trial readiness. Spots are limited—apply during registration check-out!
*Please arrive by Thursday morning to participate.

Welcome Evening Reception, Poster Session & Vendor/Sponsor Tables

- Kick off the summit with an evening of networking and connection. Our Summit Poster Session, Vendor, and Sponsor tables will be open, showcasing the latest CMT research, resources, and innovations. Enjoy refreshments, hors d'oeuvres, and a welcoming atmosphere as you explore and engage.

Weds
23

Thurs
24

CMT RESEARCH

Fri
25

Day #1: CMT Clinical Trial Readiness Summit

Agenda

7:45am: Breakfast & Vendor Tables

8:45am: Welcoming & Opening Remarks

9:00am: Keynote: Thomas Holm Pedersen, PhD, MSc, CEO of NMD Pharma

9:30am: Patient Voices | Why We Need Clinical Trials Now

Speakers: Reagan Warren, CMT1A Patient | Lindsey Flynt, CMT6 Mom, MTRFR/C12orf65, Alana Kohler, CMT4A Patient

Hear powerful stories from CMT patients highlighting the urgent need for clinical trials. Discover how these trials pave the way for treatments and cures, and learn how your participation can make a life-changing impact.

10:15am: Panel: What It's Like: Patients in Natural History Studies, CMT Trials, & Breakthroughs

Speakers: Jenneen DeFiore, Thermo Fisher Scientific | David Washabaugh, CMT1B | Gretchen Cappiello, CMT1A | Joshua Burns, PhD FFPM RCPS (Glasg) FAHMS, St. Jude Children's Research Hospital | Lawrence Van Helleputte, PhD, Droia Ventures

Hear firsthand experiences from patients who have participated in natural history studies and clinical trials, alongside insights from researchers, industry leaders, and pharmaceutical representatives. Discover how their collective efforts are advancing breakthroughs, refining trial designs, and shaping the future of CMT research and care.

11:15 AM: Panel: How to Run an Effective Center of Excellence & Prepare for Clinical Trials

Speakers: Bridget McGowan, MD, Sara Beyler, CO, LO, Rachel Galant, MSNM, OTR/L, Lurie Children's Hospital | Katy Eichinger PT, PhD, U of Rochester | Charles K. Abrams, MD, PhD, University of Illinois Health

Join trailblazers in the field as they share strategies for building impactful Centers of Excellence. Learn best practices for patient care, fostering collaboration, and achieving clinical trial readiness to drive breakthroughs in CMT research and treatment.

12:15pm: Lunch & Vendors

Take a break to enjoy lunch and connect with fellow attendees. Vendor and sponsor tables will be open, showcasing the latest CMT research, resources, and innovations. Don't miss this opportunity to explore and engage!



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Day #1: CMT Clinical Trial Readiness Summit

Agenda cont'd

1:15pm: Presentation: Will My CMT Subtype be Eligible for Therapeutics? How 1 Subtype Impacts All.

Speakers: Matt Jarpe, PhD, HNF Research Consultant, Vera Kiyasova, MD, PhD, NMD Pharma, Gideon Sharpiro, PhD, Founder & CSO, Miralinc | Ryan Castoro, DO, MS, University of Missouri School of Medicine

Explore how HNF is advancing innovative research approaches to connect CMT subtypes and prepare for basket trials. Learn how these efforts are accelerating therapeutic development, ensuring broader treatment eligibility, and demonstrating how progress in one subtype benefits the entire CMT community.

2:00pm: Presentation: Matilda, One in a Million,

Speaker: Natalia Salejko, HNF Board

2:10pm: Presentation: A Peek Inside a CMT Research Laboratory,

Speakers: Dianna Willis, PhD, Burke Neurological Institute & Weill Cornell Medicine

Join Dr. Dianna Willis for an exclusive look into the groundbreaking research advancing treatments for CMT. Learn how her team's work on knockout rat models is driving progress in gene replacement therapy and HDAC6 inhibitors. Discover how these findings have the potential to support multiple CMT subtypes, paving the way for transformative, cross-subtype breakthroughs in care.

2:45pm: Presentation: CMT Deploy, Our 3-Year Vision & Beyond

Speakers: Allison Moore, CEO, HNF | Eric Bolling, HNF Board | Laurie Kirkegaard, NPL Impact Agency

Get a front-row seat to HNF's bold vision for transforming CMT research and care! Discover how the groundbreaking CMT DEPLOY strategy is revolutionizing clinical trials, advancing innovative therapies, and driving real progress for all CMT subtypes.

3:15pm: Break

3:30 PM: Fireside Chat: Biomarkers in Action: How the Biobank is Driving CMT Solutions

Speakers: Terry Jo Bichell, PhD, MPH, CEO COMBINEDBrain | Bruce Carter, PhD, Vanderbilt School of Medicine | Michael Imperiale, MD, CMO, Actio Biosciences | Daniel Claassen, MD, MS, Vanderbilt University Medical Center

Explore the critical role biomarkers play in advancing CMT therapies and achieving FDA approval. Discover how patient-donated samples are driving breakthroughs in biomarker research and shaping the future of clinical trials. Learn about the impact of HNF's Biobank, onsite at the summit, and engage with experts in this interactive session.

4:30pm: Presentation: CMT Tech Frontier - Digital Wearable Study Phase 2 & Results

Speakers: Kayla Cornett, PhD & Marnee McKay, PhD, U of Sydney | Tina Duong PT, PhD, Stanford School of Medicine | Tim Estilow, OTR/L, CHOP | Florian Thomas, MD, MA, PhD, MSc, Hackensack U Medical Center

Join Dr. Kayla Cornett and the HNF team as they unveil Phase 2 results from their groundbreaking wearable tech study with BioSensics. See how real-world mobility data is transforming clinical trials, advancing biomarkers, and accelerating CMT treatments—while revealing how everyday factors like footwear, flooring, and clinician training impact outcomes.

5:15pm: C-Path & Closing Remarks

6:00pm: Vendors & Networking

Sat
26

Day #2 CMT Patient Retreat for Health & Wellness

Agenda

7:45am: Breakfast & Vendor Tables

8:45am: Welcoming Remarks

9:00am: CMT Speed-friending - Break the ice and kick off your day by connecting with fellow attendees in this fast-paced and interactive session! Exchange introductions, share laughs, and build new friendships in a lively, fun-filled atmosphere.



9:30am: CMT Recharge - Start your day with relaxation and renewal in this stretch, meditation, breathwork, and sound bath session led by NASM-certified experts Julie Stone and Shannon Johnson. Designed for individuals with CMT, this workshop promotes balance, stress relief, and overall well-being.

10:15am: CMT Pelvic & Posture - Discover how pelvic alignment & posture impact mobility and daily life with CMT. Join NASM-certified experts Julie Stone & Shannon Johnson for a movement workshop tailored to the unique challenges of CMT for improving comfort and function.

11:00pm: Alleviating CMT Pain & Deformities in Adults and Kids with Surgery -

Speaker: CMT Expert Surgeon Dr. Glenn Pfeffer of Cedars Sinai explores surgical options for pain relief and foot deformity correction, covering advancements, candidacy, and recovery insights. *Available for one-on-one consults Friday (9 AM - 5 PM) and Saturday (until 10:30 AM).

11:10am: CMT Pain Management & Fighting Fatigue - Share your experiences with CMT-related pain and fatigue. Explore effective strategies to manage discomfort and boost energy with practical tools and insights to help you feel empowered and in control.

12:10pm: Nutrition, CMT & Health: Eat to Beat Fatigue - Learn why the intersection of what, when, & how much we eat not only improves overall health, but also improves energy, pain, & mood when living with CMT.

Speaker: Melissia Schmidt, PN2-MHC, M.S.Ed, MindSet Nutrition Coaching, LLC

1:00pm: Lunch & Vendors - Take a break to enjoy lunch and connect with fellow attendees. Vendor and sponsor tables will be open, showcasing the latest CMT research, resources, and innovations.

CMT RETREAT

Sat
26

Day #2 CMT Patient Retreat for Health & Wellness

Agenda cont'd

2:00pm: CMT, Be Boldy You - Explore strategies to nurture your mental and emotional well-being while navigating life with CMT. This empowering workshop offers tools to build resilience, boost self-confidence, and embrace your authentic self with boldness and positivity.

3:00pm: Why Join Team CMT? - Discover the power of community and advocacy with Team CMT! Learn how joining this global movement supports CMT research, raises awareness, and connects you with others making a difference in the fight against CMT.

3:20pm: CMT, Test & Try Bracing & Mobility Aids - Test out and explore lots of options for bracing and mobility aids such as crutches, canes, wheelchairs, scooters, and more! Participate in hands-on demos, ask questions, and discover the best solutions to enhance your mobility, comfort, and independence.

4:45pm: Fashion & Adaptive Clothing Show - Join us for an interactive adaptive fashion showcase! Attendees will have the opportunity to show off their favorite pieces, including AFO-friendly shoes, easy-fastening garments, and other innovative designs. Celebrate style, function, and creativity while discovering how adaptive fashion meets everyday needs with flair.

5:15pm: Closing Remarks - Wrap up the day with inspiring reflections and key takeaways from the summit. Celebrate the connections made, the knowledge shared, and the progress toward a brighter future for the CMT community.

5:30pm: Dessert Party & Raffle Prizes! -

*Winners will be announced & dessert will be served

Free Surgery & Bracing Consults

CMT Biobank and CMT expert consultations will take place on Friday and Saturday via appts.

CMT Vendor & Sponsor Tables

Vendor and sponsor tables will be open on both days, showcasing the latest CMT products, research, resources, and innovations.



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