Externally-led Patient-Focused Drug Development Meeting

For Charcot-Marie-Tooth

NOTES

MEETING AGENDA – DAY 1

7:30am - 8:30am	**Registration / Continental Breakfast**
8:30am - 8:35am	**Welcome Remarks** Allison Moore, Founder & CEO, HNF
8:35am - 8:45am	**Opening Remarks**
8:45am - 8:55am	**Video: "A Day in the Life of CMT"**
8:55am - 9:15am	**Overview: What is CMT?** Michael Shy, MD & Stephan Züchner, MD, PhD
9:15am - 9:20am	**Goals and Objectives for the Meeting, Overview of Discussion Format, and Participant Polling of Demographic Questions** James Valentine, JD, MHS

Session 1: The Voice of the Pediatric/Young Adult CMT Patient

9:25am - 9:55am	**Panel Discussion: Symptoms and daily challenges that matter most to pediatric and young adults living with CMT** A panel of CMT patients will provide comments to start the discussion. Panelists: Drew Downing, Jakeb Lutz, Sean Tyree, Reagan Warren, Cassidy Wilson
9:55am - 10:25am	**Session 1 Polling**
10:25am - 10:55am	**Group Facilitated Discussion:** Patients, caregivers, and patient representatives in the audience will be invited to add to the dialogue

Session 2: The Voice of the Adult CMT Patient

11:00am - 11:30am	**Panel Discussion: Symptoms and daily challenges that matter most to adults living with CMT** A panel of CMT patients will provide comments to start the discussion Panelists: Michelle Brose, Lainie Ishbia, Estela Lugo, Bernadette Scarduzio, Lorenzo Woodson
11:30am - 11:55am	**Session 2 Polling**
11:55am - 12:25pm	**Group Facilitated Discussion:** Patients, caregivers, and patient representatives in the audience will be invited to add to the dialogue

MEETING AGENDA - DAY 1

12:25pm - 1:10pm	**Lunch**

Session 3: Patient Perspectives on Treatments for CMT

1:10pm - 1:25pm	**Pipeline Overview Therapies in Development:** Florian Thomas MD, MA, PhD, MS
1:30pm - 2:00pm	**Panel Discussion: Current and Future Approaches to Treatments** A panel of CMT patients/parents will provide comments to start the discussion. Panelists: Joy Aldrich, Stephanie Carmody, Kristin Gelzinis, Lori Sames
2:00pm - 2:30pm	**Session 3 Polling**
2:30pm - 3:00pm	**Group Facilitated Discussion:** Patients and patient representatives in the audience will be invited to add to the dialogue

Session 4: Summary and Closing

3:00pm - 3:05pm	**Video: "Voice of the CMT Patient"**
3:05pm - 3:15pm	**Summary from FDA:** Lucas Kempf, MD
3:15pm - 3:30pm	**Closing Remarks:** Robert Moore

For Wi-Fi, please follow the steps below.

1) Make sure you are connected to the hotel's network: **UMUC Guest**

2) Open any Internet Browser and you will now be prompted to accept the Terms of Service.

Make sure to use #HNF4CMT in all of your social media posts!

WELCOME LETTER

Welcome to the Externally-led Patient-Focused Drug Development Meeting for Charcot-Marie-Tooth (CMT)! HNF, and its collaborating partners, are very pleased to have all of you in attendance. We are excited to have representation from all the key CMT stakeholders at this meeting – senior leaders from the Food and Drug Administration (FDA), National Institute of Health (NIH), industry professionals, members of academia, clinicians, patient-advocacy organizations, individuals affected with CMT and their families and caregivers across the United States and worldwide.

We thank all of you for coming together today to show your support and let your voices be heard!

Bringing the patient's voice to guide the evaluation of future therapeutics for CMT and enhancing the FDA's ability to assess the benefits and risks of a particular therapy is directly connected to the mission of HNF, to find treatments and cures for all types of CMT and Inherited Neuropathies.

HNF's Therapeutic Research In Accelerated Discovery (TRIAD) Program is 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. To date we have funded over 2 million dollars of research and we provide families the support they need for today.

We are proud today to be featuring patient-focused research data which was important in developing today's agenda to ensure your voices are heard. The pre-polling patient-reported outcomes research survey studies through our patient registry: Global Registry for Inherited Neuropathies (GRIN) were developed to capture what matters most to all of you and our patient community. Please be sure to make your voice heard today, by participating in the on-site polling.

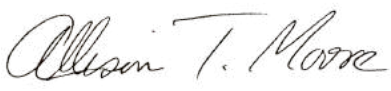
We especially want to recognize the panelists for selflessly giving their time and generously and vulnerably sharing their lives with each of us and for the CMT expert presentations. We also want to thank the patients, families, and caregivers for coming out today and speaking up about the realities of your lives with CMT; for sharing your hope for you and your loved ones, and your expectations and desires for future treatments in CMT. Your voices will impact the future of CMT and without your contribution this meeting would not have been possible!

We also want to thank our honorable FDA speaker, Lucas Kempf, MD, PLLC, Acting Associate Director Rare Diseases Program at FDA, Office of New Drugs, CDER, FDA, for his time and support of this meeting, and all senior leaders attending this meeting today. A special thanks to Meghana Chalasani, Decision Support and Analysis Team FDA for her expertise and guidance in planning this meeting. Our entire CMT Community is grateful for your support.

Last, we would like to take this opportunity to thank the Muscular Dystrophy Association and the Charcot-Marie-Tooth Association for their sponsorship and support of this initiative on behalf of the entire CMT Community.

Finally, HNF thanks each of our sponsors for their ongoing commitment to finding treatments for this devastating disease.

We know that each and every participant in the CMT community plays an essential role in a better future with treatments for CMT.



Allison T. Moore
CEO & Founder

Courtney Hollett
Executive Director

Thank You

To Our Sponsors!

PHARNEXT

ACCELERON

Cydan

FLEXPharma

athena diagnostics

IONIS PATIENT ADVOCACY

ceres

SOUTHERN VERMONT WELLNESS

CHAMPLAIN VALLEY DISPENSARY

CRESCOlabs™

EVERYLIFE FOUNDATION FOR RARE DISEASES

CMTA Charcot-Marie-Tooth Association

MDA Muscular Dystrophy Association

HEREDITARY NEUROPATHY FOUNDATION

ABOUT THIS PATIENT-FOCUSED DRUG-DEVELOPMENT MEETING

The Patient-Focused Drug Development Initiative is part of FDA's commitments under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V), which aims to more systematically obtain the patient's perspective on the burden of specific diseases and current treatments available.

Through this Patient-Focused Drug Development Meeting, the FDA provides a forum where CMT patients, families, and caregivers are invited to share their unique insight on the impact of CMT on their day-to-day lives. The FDA is also interested in gathering the CMT community's perspective on available treatment options and how well these may help to treat their symptoms, strategies for managing their CMT, and expectations for approved treatments for CMT, among other important topics.

At today's meeting panelists, representing all types, ages and stages of CMT, will bring their voices and stories to depict the real and specific ways in which their lives are impacted by CMT.

Each round of panelists will be followed by a polling session and a period of facilitated discussion with participants here in Hyattsville, MD, and from across the US via our livestreaming webcast. The goal of this meeting is to increase the FDA's understanding of how patients, families and caregivers manage CMT, and the factors that are taken into account when a treatment is chosen. This in turn, will inform the FDA regarding the benefit-risk balance of treatment options, the severity of the condition, and the urgency of unmet medical needs. Ultimately, your voice and feedback will directly inform decisions made about the drug development process, and the overall assessment of current and future therapies for CMT.

"Congratulations for holding this Patient-Focused Drug Development Meeting. This is an important step in understanding the impact of this disease on patients and potentially expanding drug development to improve the outcomes for people living with Charot-Marie-Tooth."

- Janet Woodcock M.D.
Director Center for Drug Evaluation and Research
Food and Drug Administration

SPEAKER & PANELISTS' BIOS

Allison Moore, Founder/CEO of Hereditary Neuropathy Foundation, CMT1A

Allison Moore is the Founder/CEO of Hereditary Neuropathy Foundation and an advocate in supporting patients with CMT. HNF with its many CMT stakeholder partnerships are leading efforts to the development of treatments. Her unique personal experiences drives her commitment to the importance of putting patients first. She has been the PI on two PCORI grants and has experience in patient engagement methods, with an emphasis on identifying the gaps in PROs that are hindering CMT patient care, issues with diagnosis, and enhancing therapy development to ensure successful clinical trials. HNF is hosting this FDA Externally-led PFDD Meeting with the primary goal to inform the FDA, drug developers and other stakeholders, what is most important to patients and how they view the benefits and risks of treatments for CMT. She is excited to incorporate innovative technology to help her community and other rare disease groups.

Michael Shy, MD

My professional interests involve translational research to develop rational therapies for patients with inherited peripheral neuropathies and related neurodegenerative diseases. Because genetic neuropathies have known causes, research can concentrate on specific mechanisms and intracellular pathways by which mutant genes and proteins cause demyelination, axonal degeneration or impaired glial-axonal interactions. Careful evaluation of patients as well as animal or tissue culture models of inherited neuropathies or other genetic neuromuscular diseases are essential for a translational approach; accordingly, my research has involved all of these areas ranging from tissue culture to authentic murine models to material from patients with genetic neuropathies. The combination of molecular biology, clinical expertise and human genomics offer patients the best chance to have rationally based therapies to improve their quality of life. I am the PI of the INC consortium (2U54NS065712) of the Rare Disease Clinical Research Network (RDCRN). Goals of the INC are to develop natural history data, develop outcome measures, train new investigators, identify modifier genes and develop standards of care for CMT. I am particularly interested in developing and assessing Clinical Outcome Assessments (COA) and biomarkers to be used to detect progression of CMT that can also be used to determine potential efficacy in clinical trials.

Stephan Züchner, MD, PhD

Stephan Züchner, MD, PhD, MD (hc) FAAN, is a Professor of Human Genetics and Neurology at the University of Miami Miller School of Medicine. He serves as the Chairman of the Dr. John T. Macdonald Foundation Department of Human Genetics and the Co-Director of the Hussman Institute for Human Genomics. The Department has risen in importance in the past 5 years and is now ranked #7 in the US. Dr. Züchner obtained his medical degree from RWTH Aachen University Medical School, where he also completed residencies in Neurology and Neuropathology and his 'Dr. Med.' degree (PhD equivalent). Dr. Züchner completed his postdoctoral studies at Duke University Medical Center in Durham, North Carolina, where he subsequently achieved Assistant Professor status. In 2007, he moved to the University of Miami as one of the founding faculty of the John P. Hussman Institute for Human Genomics. In 2015 he received a honorary doctorate degree from the Semmmelweis Medical School at University of Budapest, Hungary, for his contributions to next-generation sequencing and international studies of rare diseases.

Dr. Züchner's research interests are focused on identifying genetic variation associated with disease. His lab is internationally renowned for being one of the most successful in identifying several dozen genes for Mendelian disorders, especially in Charcot-Marie-Tooth and related disorders. His group is amongst the pioneering groups to share data amongst scientists utilizing in house developed software solutions, which has greatly accelerated discovery of genes and mutations in CMT. Current efforts include genetic modifiers in CMT, database development for variants of unknown significance in CMT, and drug target discovery.

James E. Valentine, JD, MHS, Moderator

James Valentine is an Associate at Hyman, Phelps & McNamara, where he assists medical product industry clients in a wide range of regulatory matters, including new drug and biologic development and approval issues. Before joining the firm, James worked in FDA's Office of Health and Constituent Affairs where he facilitated patient input in benefit-risk decision-making and served as a liaison to stakeholders on a wide range of regulatory policy issues.

Drew Downing, CMT1A

Drew Downing is a 16 year-old high school junior from Long Island and has CMT1A. Two of Drew's three siblings have CMT, as does his father, grandfather, aunt and two cousins. To treat his CMT, Drew has undergone a number of corrective foot surgeries and goes to physical therapy. Drew has served as a local ambassador for the MDA and attended their summer camps. Drew enjoys video games, tennis and skiing.

Jakeb Lutz, CMT4J

Jakeb Lutz, is 12 years old and has a very rare form of CMT known as 4J. He resides in Johnstown, Colorado with his parents. He recently became the Colorado State Ambassador for the MDA and has been able to attend two of their camps. Jakeb helped raise money for CureCMT4J to fund an exciting new gene therapy development. He is hopeful for trials to begin, and has faith that a cure will be found. Although he would give anything to have his old life back, he's eager to do his part to beat this disease.

Sean Tyree, CMT1A

Sean Tyree is a 16 year old high school junior from Topeka, Kansas. He inherited CMT1A from his father and was diagnosed at age 7. He is active in several advocacy organizations: Families Together, Kansas Youth Empowerment Academy (KYEA) and Topeka Independent Living Resource Center (TILRC). He currently serves on TILRC'S Board of Directors and their Youth Advisory Board. Additionally, he served on the Board of Directors for the August 2018 Kansas Disability Caucus where he received an award for "being a strong advocate for himself and other youth with disabilities."

Reagan Warren, CMT1A

Reagan Warren, 9.5 years old, has CMT1A and lives in New York, NY with her mother, father, and a long awaited baby sister due in October. Her father also has CMT1A. Reagan is in 4th grade and is fluent in Spanish and English. She is an avid writer and loves to perform in musicals. To treat her CMT-associated hip dysplasia, at 5.5 years, 8 years, and 9 years old, Reagan endured three hip and femur reconstructive surgeries.

Cassidy Wilson, CMT2A

Cassidy Wilson, 19 years old, has CMT2A and lives in Richmond, VA, where he is a sophomore at Virginia Commonwealth University. There is no prior family history of CMT in Cassidy's family. He is a music and psychology student who enjoys playing the drums with other local musicians. Cassidy wears bilateral AFOs to get around campus and had a tendon transfer on his left hand when he was 13.

Michelle Brose, CMT1 (Multiple Mutations)

Michelle Brose was born with Charcot-Marie-Tooth disease and became severely disabled by it at age 19. With the advent of the internet, she learned web design and programming to find others with the disease, and still runs several websites dedicated to empowering people with disabilities. Michelle is currently pursuing a degree in biology at Columbia University, with the goal of studying genetics.

Lainie Ishbia, CMT1A

Lainie Ishbia is a mom, wife, blogger and self-proclaimed fashionista who lives in the Detroit area with her blended family of 9 (including two dogs). She holds a Masters degree in Social Work from the University of Michigan and has been helping teenagers and women with self-esteem related issues for almost 25 years. Lainie's passionate about empowering others with CMT to live their best lives and founded Trend-Able.com, a fashion and lifestyle website, and community for women with invisible physical disabilities.

Estela Lugo, CMT2

Estela is a CMT patient as well as the Medical Outreach Manager for the Hereditary Neuropathy Foundation. Estela Lugo and her younger sister, Melissa were both diagnosed with CMT by age four. From that point forward, their parents became heavily involved in advocacy and fundraising for CMT and Muscular Dystrophy throughout Long Island, NY.

She is an avid user and personal advocate for the AlterG anti-gravity treadmill (running for the first time in her adult life) and the MOTUS - Made for Movement program.

Bernadette Scarduzio, CMT1A

Bernadette (Berns) Scarduzio is a Certified Personal Trainer and received her certification from the National Personal Training Institute (NPTI) but had to halt training due to CMT. She also worked for her family business Cuz N' Company Salon and Spa that her dad started 30 years ago and took on many roles throughout the years. Currently, she works for HNF part-time as their social media coordinator, she is a spokesperson for Thermapools, and she does some acting on the side.

In addition, she worked in the community as a teacher's aide for Family Support Services. Berns is the star of the "Bernadette Documentary" which follows her journey with CMT. She is social media guru and is looking forward to continue the upward growth of HNF's social media. Berns resides in Drexel Hill, PA and enjoys spending time with her family and dogs.

Dr. Lorenzo A. Woodson, CMT (Type Unknown)

Dr. Woodson was diagnosed with CMT at the age of six. He graduated from Community College of Philadelphia with a degree in early childhood education, then earned his Bachelor's in Education and Master's in Human Services Counseling from Lincoln University of Pennsylvania with honors. He also earned his PhD in Human Services-Counseling from Walden University with honors.

Dr. Woodson currently lives and practices in Germantown, PA, as a Licensed Behavior Analyst (specializing in Autistic Spectrum Disorder and Developmental Disabilities). He has been married 24 years and lectures for social change groups and non-profit organizations nationally, as well as writes an editorial for a local Philadelphia newspaper. He has published research in a national peer review journal which currently is required reading at Walden for PhD students.

Florian Thomas, MD, MA, PhD, MS

For 20 years Dr. Thomas has practiced interprofessional care to help people with CMT. In 1998 he founded, with patient advocates, a CMT support group. He leads two CMT drug trials. He characterized novel neuropathology in CMT1B and a novel DI-CMT-C subtype caused by YARS gene mutations. He is board-certified in neurology and neural repair and rehabilitation. He holds graduate degrees in psychology, health outcomes research and molecular biology. He is a Professor of Neurology and Founding Department Chair at Hackensack Meridian School of Medicine and Hackensack University Medical Center. There he has established a holistic care paradigm, where neurologists work closely with rehab physicians and therapists, urologists, geriatricians, foot and ankle surgeons, counseling and neuro-psychologists to comprehensively address patients' needs and help them optimize health behavior, so that they can live life to the fullest despite illness and functional limitations.

Joy Aldrich, CMT1A

Joy joined Hereditary Neuropathy Foundation (HNF) in February, 2015 to focus on the growth of HNF's online patient support community as a Charcot-Marie-Tooth (CMT) Advocacy Director. Joy and her husband, Toby, live in Seattle, WA.

The diagnosis of CMT came when Joy was a teenager, after years of trips and falls and sprained ankles. Her mom and brother were also diagnosed at that time. Years went by before Joy noticed the more rapid progression of CMT symptoms and pursued a genetic diagnosis, which was confirmed as CMT1A. Joy feels that the best way to fight this untreatable disease is to advocate through her work at HNF. For the past three years, she has been involved in the planning and execution of three, Patient-Centered CMT Summits. This year, she helped HNF plan this Externally-led Patient-Centered Drug Development Meeting.

Stephanie Carmody - TRPV4 related Hereditary Neuropathy (CMT2C & SPSMA)

Stephanie Carmody lives in Chapel Hill, North Carolina with her husband Dennis. She received her undergraduate and Master's degrees from UNC-Chapel Hill in speech, language, and hearing disorders, and is the co-owner of a small family business in the healthcare field. She volunteers with the HNF, is dedicated to advocacy for others with TRPV4 HN, and created and moderates a Facebook group for the TRPV4 HN community to connect with others and raise awareness.

Kristin Gelzinis, LMSW: CMT4C

Kristin Gelzinis is a wife, mother, and Social Worker who works closely with the Hereditary Neuropathy Foundation as a patient advocate. She also helps run their CMT-Connect program workshops designed to empower those who are affected with CMT. Kristin was diagnosed with CMT4C and has made it her personal mission to ensure those who have this disease are empowered to fight for the best care possible and make them aware of every treatment available. During her free time, she enjoys spending time with her family. She believes it's important for her son to see that a disability doesn't always need to stop you in your tracks; teaching him that it's alright to fall down in life, as long as you continue to get back up.

Lori Sames

Lori is the co-Founder of Hannah's Hope Fund (HHF) for Giant Axonal Neuropathy (GAN) and received a Bachelor's Degree in Economics from St. Michael's College. Lori chose to stay in VT and pursued a career in healthcare informatics, leading software installations. In 2001, she became a stay-at-home mom before the birth of her second child. In 2008, Lori and her husband, Matt learned their youngest of three daughters, Hannah, had GAN. For the last 10 years, Lori has been managing a virtual biotech, leading a collaborative team of scientists focused on cell biology and developing treatments for GAN. Lori and Matt created Hannah's Hope Fund (HHF), a 501c3 public charity focused on raising funds for therapy development for GAN. GAN is the most severe form of Inherited Neuropathy, and in most cases is fatal in the early 20's. In 2016, Hannah finally received gene therapy to her central nervous system, a program independently funded by HHF through grass-roots efforts.

Lori is also an independent consultant for the translation of gene therapy programs.

Lucas Kempf, MD

Dr. Lucas Kempf is the Acting Associate Director for the Rare Disease program in the OND immediate office. Prior to joining FDA in 2012, Lucas spent eight years at NIH with a focus on neuroscience research, working to understand the genetics of neuropsychiatric disease and developing translational approaches and therapeutics to study these disorders. Lucas was trained at UC-Berkeley in molecular biology and genetics, received his MD degree from the University of Kansas, and did his post-graduate training in psychiatry at Georgetown and Johns Hopkins before moving to the NIH for fellowship.

Robert Moore

Robert Moore is a entrepreneur with over 26 years of experience creating successful businesses. As the founder or co-creator of digital companies; The Vendare Group, Baby-to-Bee, The Great American Photo Contest, and oneQube, Robert has developed a keen understanding of all the necessary elements for a company or technology to dominate their market space.

Robert is currently an advisor for voice technology company, True Reply, as well as the new celebrity social network, Holr. In addition, he is an Executive Producer of the celebrity entertainment news show, Celebrity Page.

Robert has been an advisor to HNF since its inception, and has played an instrumental role in helping HNF launch its proprietary patient registry, Global Registry for Inherited Neuropathy (GRIN).

ABOUT THE PATIENT-CENTERED BEHAVIORAL HEALTH SUMMIT - DAY 2

This year's annual Patient-Centered Summit will be a game-changer 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," said 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 CMT 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.

SUMMIT AGENDA - DAY 2

8:30am - 9:00am	**Registration / Continental Breakfast**
9:00am - 9:15am	**Opening Remarks / Introduction**
9:15am - 11:15am	**Fast Forward for CMT Curriculum** Elevate performance and fulfillment in all areas of your life with Fast Forward's CMT-focused tools and coaching.
11:15am - 11:30am	**Break**
11:30am - 12:00pm	**How Surgery Outcomes Affect Behavioral Health** CMT Orthopedic Surgeon - Glenn Pfeffer, MD
12:00pm - 1:00pm	**Lunch**
1:00pm - 3:00pm	**Behavioral Health Workshops:** **Assertiveness & CMT** with Lainie Ishbia - Gain tools for practicing assertiveness, navigating social interactions, and asking for help. **Nutrition & CMT** with Alana Kessler - Gain tools for improving emotional health through nutrition & lifestyle.
3:00pm - 3:15pm	**Closing Remarks**

SPEAKERS & BIOS

Lisa McCarthy, The Fast Forward Group

Lisa McCarthy is the Founder and CEO of The Fast Forward Group (FFG), a training and coaching company known for helping leaders and teams dramatically elevate performance and fulfillment — in all areas of their lives. Fast Forward participants get a practical and actionable regimen for high performance living that helps them thrive and succeed professionally and personally. As a facilitator and keynote speaker, Lisa's energy, passion and interactive sessions leave individuals inspired and in action. With twenty years of experience in senior leadership roles with Fortune 500 companies such as Univision, Viacom and CBS, Lisa is known for fueling high performance teams and as a driving force of transformation.

Glenn B. Pfeffer, MD, CMT Orthopaedic Surgeon

Glenn B. Pfeffer, MD is Director of the Foot and Ankle Program at Cedars-Sinai's Orthopaedic Center and Co-Director of the Cedars-Sinai/USC Glorya Kaufman Dance Medicine Center.

Dr. Pfeffer is a board-certified orthopedic surgeon, a diplomat of the American Board of Orthopaedic Surgery and a fellow of the American College of Surgeons. He is a past president of the American Orthopaedic Foot and Ankle Society, the American Association of Foot and Ankle surgeons and the California Orthopaedic Association. He served on the national board of the American Academy of Orthopedic Surgeons.

Dr. Pfeffer has shared his expertise through many media outlets, including ABC News, the Associated Press, CBS News, CNN, Dateline NBC, the Los Angeles Times, Good Morning America, the New York Times, People magazine, Readers' Digest, Runner's World magazine, USA Today and the Wall Street Journal. He is frequently seen as a consultant on ABC's Dancing with the Stars.

SPEAKERS & BIOS

Lainie Ishbia, Trend-Able.com

Lainie Ishbia is a mom, wife, blogger self-proclaimed fashionista who lives in the Detroit area with her blended family of 9 (including two dogs). She holds a masters degree in social work from the University of Michigan and has been helping teenagers and women with self-esteem related issues for almost 25 years. Lainie's passionate about empowering others with CMT to live their best lives and founded Trend-Able.com, a fashion and lifestyle website, and community for women with invisible physical disabilities.

Alana Kessler, Holistic Health Coach

Alana Kessler, MS, RD, CDN, E-RYT is a Registered Dietitian, nutritionist, weight management expert, an accredited member of the CDR and American Dietetic Association, yoga and meditation teacher, Ayurveda specialist, and the founder of BE WELL, by Alana Kessler. A graduate of NYU with a BA and MS in Clinical Nutrition, Alana is dedicated to helping others find nutritional balance through counseling, teaching, and writing. Alana leads Yin Yoga workshops, trainings, as well as yoga retreats in exotic locations. She is a sought after wellness and empowerment speaker whose health, nutrition, and fitness expertise has been featured in Aaptiv.com, Droz.com, EatThis.com, RD.com, Redbook, WomensHealthmag.com, and Vogue.

LEARN MORE ABOUT OUR SPEAKERS HERE:

Lisa McCarthy, The Fast Forward Group: www.fastforwardgroup.net
Glenn B. Pfeffer, MD: http://bio.csmc.edu/view/3814/Glenn-B-Pfeffer.aspx
Lainie Ishbia: www.trend-able.com
Alana Kessler: www.bewellbyak.com

GLOBAL REGISTRY FOR INHERITED NEUROPATHIES

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... We need YOU!

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 Charcot-Marie-Tooth.

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!

HEREDITARY NEUROPATHY FOUNDATION

www.neuropathyreg.org