



HEREDITARY
NEUROPATHY
FOUNDATION

SPRING 2019

Letter from the CEO	1
Featured Article	2
TRIAD	4
Center of Excellence	6
CMT-Connect	8
Community	10
Guest Author	12
Events Recap	14
Center of Excellence List	16
Upcoming Events	BC

cmtupdate

A Second Pediatric Center of Excellence

Pg. 6

Visiting Author:
A Plate of Pride
Pg 12

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HNF STAFF

Allison Moore
Founder, CEO
allison@hnf-cure.org

Courtney Hollett
Executive Director
courtney@hnf-cure.org

Joy Aldrich
Advocacy Director
joyaldrich@hnf-cure.org

Cherie Gouaux
Accounting Manager
cherie@hnf-cure.org

Estela Lugo
Manager, Medical Outreach
estela@hnf-cure.org

Jessica Roberts
Digital Asset Manager
jessica@hnf-cure.org

Bernadette Scarduzio
Social Media Coordinator
bernadette@hnf-cure.org

DESIGN

BolleDesign.com

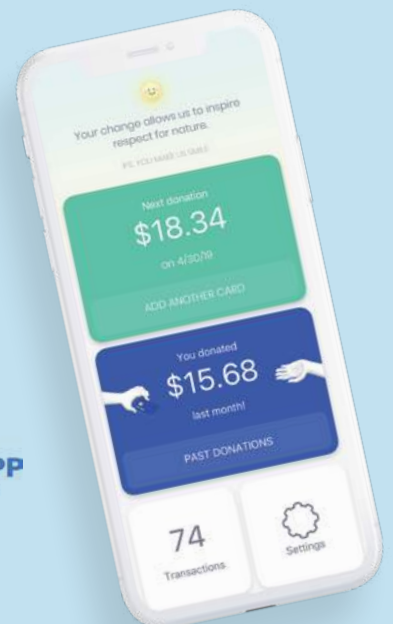
The Hereditary Neuropathy Foundation's mission is to increase awareness and accurate diagnosis of Charcot-Marie-Tooth (CMT) and related Inherited Neuropathies, support patients and families with critical information to improve quality of life, and fund research that will lead to treatments and cures.

www.hnf-cure.org

CLICK



ROUNDUP APP
EFFORTLESSLY GOOD



CMT UPDATE SPRING 2019



Allison T. Moore

Founder/CEO, Hereditary Neuropathy Foundation

Dear Friends,

Spring is in the air and warmer weather is lifting spirits, and for many of us, some of our cold-related CMT symptoms. We're happy to finally be rolling down windows and opening new doors for CMT research in 2019. **Our latest collaboration is with an exciting new fundraising app called, RoundUp!** It's a one-time, "set-it and forget it" initiative that really adds up!

Here's how it works:

The **RoundUp App** allows you to "round up" the change from your debit or credit card purchases and donate it directly to HNF. Even the smallest round up can have a huge impact on helping us reach our RoundUp \$250,000 goal. It's rather simple and painless, and all those small change round ups are tax deductible (HNF will issue a monthly tax deductible receipt).

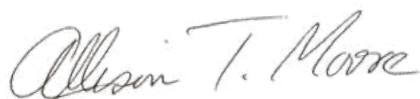
← **ROUNDUP APP** VISIT: www.hnf-cure.org/roundup-app/

Once you create an account, you do not need to use the app while making a purchase. Just swipe your card as usual. You can cap the amount monthly to as little as \$10, \$20 or \$25.

Download the app for iPhone, Android or create an account on the Web. You can also visit our site to download the app by clicking the [link above](#).

Without your support and donations, our programs can not continue to thrive!

We have a jam-packed year of research objectives, awareness and educational goals to deliver to you the patients and to our researchers and industry partners.



MOVEMENT IS MEDICINE SUMMIT

"If you don't use it, you'll lose it!" How many times have we heard that? Exercise and CMT can be a tricky combination. As we've seen, there are Team CMT members who regularly train and run marathons. For instance, Allison Moore, HNF's Founder/CEO, accomplished a lifetime goal of completing the New York City Marathon last fall! But, there are also plenty of us who mourn the loss of that level of mobility as our CMT has progressed, and could never fathom signing up for a marathon, let alone completing one.

But, although it's challenging, numerous studies have shown that movement and exercise are essential for CMT patients to maintain muscle tone and function. So, with that in mind, the Hereditary Neuropathy Foundation is excited to announce its latest patient-focused summit, Movement is Medicine™, being held at Ability360 Sports & Fitness Center in Phoenix, AZ, on November 8-9. The fully-adaptive fitness center provides a unique opportunity to try new activities in a judgement-free zone and learn tips and tricks from other people with CMT, as well as trainers who fully understand disability.

The groundbreaking Movement is Medicine™ Summit will be an interactive two-day event with inspirational speakers, awesome instructors and informational breakout sessions specifically curated for CMT patients. We plan to have a lot of fun, too!

At the Movement is Medicine™ Summit, you will receive interactive, hands-on instruction from leading fitness experts who will help jumpstart your ability to enjoy a more active lifestyle! You'll be able to join others with CMT in the warm therapy pool, via lift/elevator, if needed, for Ai Chi sessions that will focus on breathing, balance, flexibility, joint mobility and relaxation. If you're looking for something with a bit more intensity, you can try a Krankcycle® arm crank bike too! Learn how to adapt yoga around decreased balance or AFOs. Try rowing on a wheelchair-accessible rowing machine or climbing a rock wall. Learn how to feel more confident in the gym with Circuit Training and more.

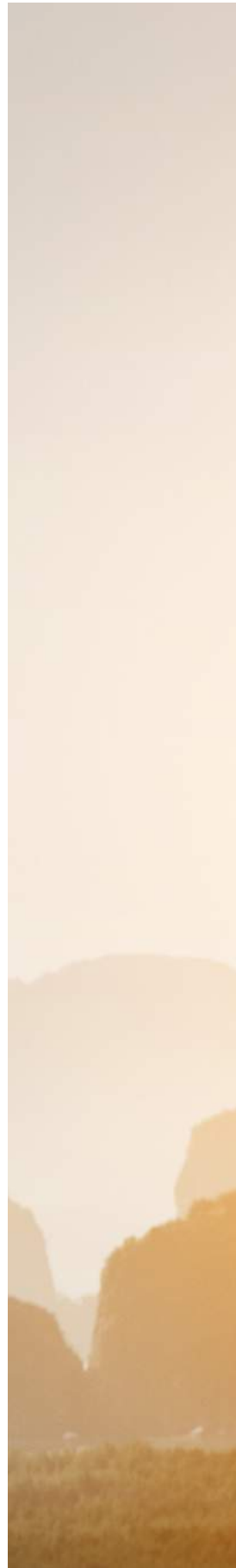
Your two-day pass will include all sessions, breakouts and evaluations, as well as breakfast, lunch and dinner both days. Click here to see the full agenda and complete class descriptions.

November 8-9th, Phoenix, AZ

Space is limited for this amazing event, so make sure to reserve your spot today!

CLICK HERE: www.hnf-cure.org/movement-summit

You don't have to have CMT to participate,
there will be something for all attendees



“If you don’t use it, you’ll lose it!”

How many times have
we heard that?





Meet James McCarthy

LINDSEY & KYLE MCCARTHY, FOUNDERS OF JAMES' CURE

We didn't know anything was wrong until the day James was born. At 39 weeks and two days, our precious boy was born. Unable to breathe, James was immediately intubated. After he was stable, the NICU team at our local hospital decided it was best to send James to our children's hospital that night.

We spent almost a month in the NICU there, where we learned that James has bilateral vocal cord paralysis, hypotonia, and is unable to suck or swallow. Because of all of this, James received his tracheostomy and gastrostomy tube surgeries within the first few weeks of his life. The doctors ran every test imaginable, and yet still couldn't tell us what was causing all of this; so they discharged our son from the hospital..

Two weeks after being discharged, we received a call from our geneticist who let us know she had found our answer. It turns out that James has an extremely rare genetic mutation of the CNTNAP1 gene.

What is CNTNAP1?

Recessive mutations in CNTNAP1 that lead to a loss of this gene and its protein product, CASPR, lead to disruption of the myelinating cells from the signal transmitting nerve cell axons. This has profound negative effects on the development and maintenance of many nerve fibers in the central and peripheral nervous system. The effect on the peripheral nerves is a profound demyelination and weakness. This adds CNTNAP1 to the severe CMT syndromes.

This genetic disorder makes it difficult for James' brain to communicate to his muscles, and so now we understand why his body reacts the way it does. James is unable to move like a "normal" child and is unable to use his voice. There is no cure, yet.

Since coming home from the hospital and with lots of therapy, James has learned how to swallow, move his head and his arms and legs (at times), and recently learned how to smile. We

pray for a day that we can hear our sweet boy cry or laugh. We are hopeful that with consistent physical therapy and occupational therapy, James will continue to have "inch" stones that we can celebrate.

My husband and I have found four other families from around the world whose children also have this rare genetic mutation, the oldest is five and the youngest is our son, James, who just turned one. When we first got the diagnosis for our son, it was pretty lonely and we didn't know where or to whom to turn to when we had questions. These other four families have given us so much support and guidance at times when our doctors didn't know what to tell us, none of them have ever seen a child like James before and don't know what our future looks like. We are hoping to connect with other families whose children have the CNTNAP1 mutation and offer the same support and friendship that we have received.

We have teamed up with the Hereditary Neuropathy Foundation in hopes of finding other families that have children with CNTNAP1 to work together on research.

TO LEARN MORE ABOUT JAMES AND CNTNAP1 VISIT:
www.jamescure.org

TO REACH OUT TO JAMES CURE VIA FACEBOOK VISIT:
www.facebook.com/cntnap1familysupport/

Fast Track

What is *Fast Track*?

THE FDA HAS AWARDED FAST TRACK DESIGNATION TO TWO DRUGS CURRENTLY IN DEVELOPMENT FOR CMT: ACCELERON'S ACE-083 AND PHARNEXT'S PXT3003.

Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious conditions.

Fast Track designation must be requested by the drug company. The request can be initiated at any time during the drug development process. FDA will review the request and make a decision within sixty days based on whether the drug fills an unmet medical need in a serious condition.

Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

SOURCE: U.S. FOOD AND DRUG ADMINISTRATION

www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm

Acceleron ACE-083

HNF partner, Acceleron Pharma, announced in November, 2018, that the FDA had granted Fast Track status to ACE-083, an injected myostatin drug intended to increase muscle mass and strength. Acceleron announced the exciting, preliminary positive results of the part 1 of the phase 2 clinical trial in January, 2019.

Currently, ACE-083 is recruiting patients with CMT Type 1 and CMT Type X in a part 2 of a phase 2 clinical trial.
www.cmtstudy.com

"We're pleased that the FDA has granted this designation for ACE-083," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "Patients with CMT currently have no approved therapies. To date, results from our Phase 2 trials have shown that patients treated with ACE-083 experience robust increases in muscle volume. If our ongoing clinical studies show that ACE-083 also improves functional outcomes and confirm the favorable safety profile observed thus far, the Fast Track process could help us work with the FDA to deliver it to patients as quickly as possible."

Preliminary results from part 2 of the trials are expected by year end 2019.

Pharnext PXT3003

HNF partner, Pharnext, announced in February, 2019, that they had been granted *Fast Track* designation for the development of PXT3003 for the treatment of patients with Charcot-Marie-Tooth disease Type 1A (CMT1A). PXT3003 is a novel synergistic combination of baclofen, naltrexone and sorbitol, formulated as an oral solution that is given twice a day.

"We are pleased to receive Fast Track designation for PXT3003 in CMT1A" said Daniel Cohen, M.D., Ph.D., cofounder and Chief Executive Officer of Pharnext. "CMT1A is a rare, chronic neuropathy that affects at least 125,000 people across the U.S. and Europe. There is currently no approved treatment for this disease. We look forward to continuing our productive dialogue with the FDA to rapidly progress towards a U.S. New Drug Application for PXT3003, to deliver this therapy to patients as quickly as possible."



HNF Announces The Second Pediatric Center of Excellence for CMT

Ann and Robert H. Lurie Children's Hospital of Chicago

DR. VAMSHI RAO NEUROLOGIST, ASSISTANT PROFESSOR OF PEDIATRICS,
NORTHWESTERN UNIVERSITY FEINBERG SCHOOL OF MEDICINE



Dr. Vamshi Rao Neurologist

Q: TELL US ABOUT YOURSELF?

After completing medical school, I began my neuromuscular journey, while pursuing a Master's in Biomedical Engineering, by trying to understand the molecular aspects of muscle disease.

This was followed by an application of the knowledge gained from animal models to human disease, in Dr. Christine DiDonato's lab. My first contact with neuropathies including CMT happened during my child neurology training. I was immediately impacted on a deep emotional level, watching these resilient children and their parents struggle through the ever-progressive nature of CMT. I was fortunate enough to be trained by, and subsequently work with, Dr. Nancy Kuntz, who had set up a neuromuscular clinic for children at the Ann and Robert H. Lurie Children's Hospital in Chicago. The Lurie Children's Neuromuscular Clinic, which is affiliated with the Muscular Dystrophy Association and Parent Project to Cure Duchenne, has been expanded to include the first-ever, dedicated, peripheral neuropathy multidisciplinary clinic for children in Chicago. The resulting comprehensive care model will prevent delays in diagnosis, help circumvent complications, and decrease the overall burden of CMT for the entire family unit. We have also established a working database that continues to be refined, in preparation for clinical trials. My journey, hopefully mirroring that of CMT, comes full circle in that a continued study of the natural history of CMT will help guide fundamental research, to bring meaningful treatments back to the clinic.

Q. WHY IS CMT YOUR PASSION?

Inherited neuropathies, such as CMT, have been especially fascinating as they encompass a wide range of clinical presentations (phenotypes) and a wide range of genetic mutations (genotypes). They have been frustrating, on the other hand, as there have been no meaningful treatments to offer these individuals. Advances in neurophysiologic and genetic techniques have made diagnosis easier than before. Early diagnosis enables early institution of comprehensive care. While comprehensive care does make a dent in the natural progression of disease, it is still very distressing to watch a child lose motor skills over time. What makes it especially hard is that the slow decline fails to receive the attention that other rapidly degenerating neurological conditions garner, even though the impact on the quality of life for the individual and family may in fact be higher. I personally think neuropathies are the next frontier in neuromuscular and genetic medicine, the treatment of which will change the overall impact of the disease. I envision a future with treatments slowing or arresting the natural history of CMT, especially at a younger age, facilitating a seamless transition to adulthood with a better quality of life.

Q: TELL US WHAT PATIENTS WILL EXPERIENCE WHEN THEY COME TO THE PEDIATRIC NEUROMUSCULAR PROGRAM AT LURIE CHILDREN'S.

Our initial meeting will be aimed at understanding one's CMT diagnosis. This will be aided by the fact that we have the ability to perform any necessary nerve and muscle studies (nerve conduction/electromyogram, ultrasound, nerve biopsy etc.) and to obtain genetic testing. All children with a confirmed diagnosis of CMT are encouraged to return for follow-up in our dedicated, multidisciplinary neuropathy



clinic. This clinical program at Lurie is staffed by pediatric neuromuscular specialists (myself and Dr. Kuntz) in addition to a team of advance practice nurses, physical therapists, occupational therapists, orthotists, dietitians, genetic counselors, metabolic bone health specialists, social workers, a wheelchair consultant and neuromuscular nurses. The neuropathy clinic strives to be a "one stop shop" that will reduce the need for multiple visits. Further consultation with specialists such as orthopedic surgeons, pulmonologists and cardiologists will be available as needed. We will also work with Dr.Christine DiDonato, who leads our basic science initiative in neuromuscular disease and will help with understanding the molecular/genetic aspects of CMT. Finally, a team of neuromuscular clinical research

professionals will be ready to screen each child for eligibility in available neuropathy clinical trials.

Q: HOW CAN PATIENTS MAKE AN APPOINTMENT AT LURIE CHILDREN'S?

Call 1-800-KIDS-DOC for an appointment. If there is already a confirmed diagnosis of CMT, call 312-227-4471. Our clinic location is at the Ann and Robert H. Lurie Children's Hospital of Chicago, 225 East Chicago Avenue, Chicago, IL 60611.



CMT-Connect Webinars

ESTELA LUGO, MEDICAL OUTREACH MANAGER

HNF is dedicated to providing educational and enriched online webinars along with in-person workshops to support CMT patients & families. CMT-Connect is an interactive series designed to shed light on our communities' most important topics. We seek out wellness experts, thoughtleaders, medical professionals, adaptive programs, emerging technologies, disability advocates, and so much more! Living with a disability is far from simple, but our mission is – to EDUCATE, EMPOWER AND CONNECT!

As CMT patients and caregivers ourselves, many on our team at HNF are personally aware of the hurdles when considering disability benefits, programs and finances. It's easy to become overwhelmed by all of the complexities of regulations, applications, programs and procedures. James Traylor is an experienced consultant helping individuals and families navigate the state and federal systems towards health, security and emotional wellness. CMT-Connect recently hosted a informative webinar with James to answer some of our community's most commonly asked questions. Topics discussed included:

- * Types of financial benefits
- * Types of healthcare services
- * Wills/Trusts
- * Employment Insurance
- * Tax benefits
- * And much more!

To view the entire webinar, please click here.

Be sure to check out HNF's **NEW State Resource page** with state program and office contact info.

TIPS



Our 2019 Movement is Medicine Summit™ has been announced, and is now open for registration! This year, HNF will be celebrating adaptive exercise and sharing a custom designed two-day program for improved function and health for CMT patients. We've chosen the state-of-the-art adaptive fitness and sports center, Ability360 in Phoenix AZ to host this exciting weekend. During our recent CMT-Connect webinar with program director Brielle Carter, we discussed this impressive 62,000 square foot campus, its wide range of programs, special events and why Brielle and her team love truly what they do! Take a virtual tour of Ability360 on our summit overview page and watch the exclusive [webinar click here](#).

View our past webinars Active Hands and Zoe's Story here:
www.hnf-cure.org/cmt-connect-webinars

Stay tuned for new CMT-Connect webinars posted each month and feel free to email Estela Lugo, Medical Outreach Manager at estela@hnf-cure.org with any questions or webinar suggestions.

UPCOMING WEBINARS

**Telemedicine with
Dr. Raghav Govindarajan**
May 30, 2019 @8pm EST

**CMT & Mental Health
with Amber Reiff**
June 1st @12pm EST

**Swim Up Hill with 2020
CMT Paralympic Swimmer
Jamal Hill**
June 13, 2019 @8pm EST

Register for Upcoming Webinars:
www.hnf-cure.org/cmt-connect

PAST WEBINARS

- * Bemer Technology
- * Panetta Physical Therapy
- * CMT & Balance
- * CMT & Your Nutrition
- * CMT&Me App
- * CMT & Finances
- * Ability360 Sports & Fitness Center
- * Active Hands
- * Cannabis & CBD for CMT
- * CMT & Canine Companions
- * Discrimination in the Workplace

VIEW PAST WEBINARS:
www.hnf-cure.org/cmt-connect-webinars

CMT Symptoms Checklist

HNF and members of the Inspire community worked together to develop a CMT Symptoms Checklist to help you keep track of your symptoms. Download the checklist and take it with you to each visit with your doctor to help in discussing your health and treatment plan.

- ▶ www.hnf-cure.org/symptoms-checklist
- ▶ [Join HNF's Inspire Community](#)



Marvin, my Service Dog

DIANA STRUTHERS, MARVIN'S PARTNER

Charcot-Marie-Tooth disease affects about 2.8 million people in the world. There are many forms of CMT disease, as well as different ages of onset; some more severe than others. Most of us have heard of bracing, surgeries, physical therapy and occupational therapy, but there is one more form of therapy that can not only make our lives better physically, but also emotionally: A service dog!

As my children grew and the last one left for college, I realized that being alone in a two-story house was probably more than I could handle. I had stopped work 10 years before due to my CMT. My limbs continued to weaken, and walking, as well as balance, was difficult. My hands would no longer grip well, nor could I manage fine motor skills. Although I tried to stay active in seeing friends, volunteering, and gardening, I found my world shrinking smaller and smaller, as I stayed home more and more.

Now thanks to Marvin, my Canine Companions for Independence® service dog, my life is so much fuller. Marvin provides assistance in many everyday tasks that cause me pain, such as tugging the laundry basket outside so I can hang up my clothes. He also opens and closes doors, cabinets and the refrigerator. One of the most valuable things he does is pick up things I've dropped, like my keys or phone. He will also carry small bags or my clutch when needed.

While Marvin is trained in more than 40 commands, having him at my side has caused an even bigger impact emotionally. With Marvin, I feel confident, able to go out into the world knowing that Marvin can help me and that I won't need to ask for assistance. Marvin brings out the best in me and others. Instead of people focusing on my disability, they are focused on this incredible dog. I know each time we go out, I will have multiple discussions about Marvin. I actually met several new friends this way.

Marvin can go with me any place the public is allowed. He is an avid traveler, having been on trains, planes, cars and buses; always the perfect gentleman. My activity level, as well as my quality of life, has increased due to Marvin. I now travel more, visiting friends and family, as well as taking a few vacations I'd always dreamed about but could never have done without Marvin at my side! Marvin and I volunteer with hospice and visit care facilities weekly. We also volunteer with the SPCA and



provide care to neonatal kittens without mothers. Marvin loves each and every kitten we have raised. Marvin is so much more than a dog to me. He is my best friend, my companion and my assistant. He has given me back my life.

Canine Companions for Independence is a 501(c)(3) nonprofit organization that was founded in 1975. It helps people with disabilities by providing them with expertly-trained assistance dogs and ongoing support to ensure quality partnerships, all at no cost to the recipients.

Each assistance dog spends their first year and a half with volunteer puppy raisers learning basic commands and being socialized. Then they are returned to Canine Companions to move on to their six to nine months of professional training at one of the six regional Canine Companions training centers across the country. Once the dog has completed his/her training and a candidate has reached the top of the waitlist, the magic begins! They are brought together for a two-week group class known as team training. At one of the regional centers, the new owner will be provided a private room with two beds (for you and your support person) as well as a private fully accessible handicapped bathroom. There is a large group kitchen, living room and several training rooms. Here, you will learn to manage the assistance dog's behavior, to direct the dog in commands he has learned, and to assume responsibility for the health and well-being of your dog.

On the third day, pre-matches happen. The staff has watched each person with the dogs over the previous days. They know each individual dog's strengths, as well as each candidate's individual needs. Now after watching the interactions with each dog, the patient is introduced to the dog that is going to change his/her whole life! They tell people not to have a preconceived notion of the dog they will get....of course not! I silently hoped for a black, cuddly, female. Then, up strutted Marvin, a yellow male with a wagging tail who prefers to show his love by sitting his boney butt on my feet, and a offer an occasional lick to the face. I cried with joy meeting my new friend. Our match is so perfect. He is so much like me, a bit of a attitude but willing to give his all. His love is deep and unconditional. Together, we greet each day head on, ready to meet the challenges awaiting us!

To see if a CCI dog is right for you go to: cci.org

Dear Kristin,

My boyfriend broke up with me because he said he's worried about our future together because I have CMT.

Yours Truly,
Heartbroken with CMT

Dear Heartbroken with CMT,

Relationships are for better or worse. Each person in a solid relationship has decided to love you through the good and the bad— no matter what. If you ask me, you dodged a bullet. This man is essentially telling you he won't (or can't) be there for you in bad times. That's not someone you want in your life.

It is best to know your partner's true character before you're married. It's far easier to break up than to get divorced! Someone who truly loves you will stay by your side no matter what — in sickness & in health.

Everyone deserves unconditional love. I think CMT sufferers sometimes think of ourselves as "less than," so we take the first person who comes along who can handle the CMT, even if it's not a love match, or if the other person has serious baggage. Instead: Hold yourself to high standards and have confidence. Be active, follow interests and hobbies, and you will attract someone with that same energy. And remember: You are looking for an equal partner, a best friend, and a lover. Not a caretaker. Deciding on a life partner will determine your happiness or misery more than any other decision you will make. But in short: You are NEVER 'less than.'

Stay strong,



Kristin Gelzinis LMSW
HNF Patient Advocate
info@hnf-cure.org

*A recipe for asking for help
with an invisible disability*



A PLATE OF PRIDE

GUEST CONTRIBUTOR: LANIE ISHBIA, FOUNDER TREND-ABLE
WWW.TREND-ABLE.COM

Disclaimer:

I don't cook. In fact, when my twin step-daughters were home from college for the holidays, they opened the oven to bake cookies and found a cookie sheet filled with shriveled up, burnt, miniature hot dogs inside. Apparently, I must have forgotten to serve that second tray of appetizers at the party we had almost two months prior. With that said, I am not your go-to cooking expert. You should feel free to adjust any ingredients needed, and modify any recipe I ever give you.

Key Ingredients:

Confidence, Humility, Honesty, Empathy & Humor

5 Cups Of Confidence

The most important ingredient for getting what you want and need when you have non-obvious physical challenges is confidence. It's the base that every other ingredient adheres and mixes into.

Confidence is not a fast food item that you can easily find. It must be grown and nurtured in-house. Everyone has the ability to get confidence, but some people have easier access and more resources.

Confidence is not seen; it is felt. A person with a disability can shout it from a rooftop wearing an "I have a disability" or, "I survived _____," but still lack true self-confidence. This can be all show. Likewise, a person can conceal her leg braces, or large scars, and be über self-assured and not want or need an exterior label.

Confident people can, and do, have insecure moments. No one is 100% confident in every situation. A confident person knows this and owns her insecure feelings. Learn more about building confidence in my post, [How To Photoshop Your Self-Image.](#) **CLICK HERE.**

2 Cups of Humility

It is never easy asking people for help to do seemingly easy tasks. This is especially true if you're a Type A person like me.

But, we have to remind ourselves that able people are not mind-readers, and unless a person has physical challenges of her own, she cannot intuitively get your need for an arm to hold for balance, or your need, rather than want, for that close-to-the-exit space in barre class.

Swallowing our pride doesn't have to cost us anything. In fact, it can be low, or zero calories if we remember to use a sugar substitute. We are not entitled to the space in exercise class or the lounge closest to the pool. No one wants to help people they find to be rude and condescending.

We may need it and want it, but in order to get something when someone else already has it, we must be kind and "use our words," as you hear the moms of young children lecture. We also must accept the fact that 99% of the time people will do the right thing when we ask them for something in a respectful way. We should not give any of our power away to the 1% of people who won't. This is something I struggle with (just ask the guy who took my lounge at a certain Miami resort and is probably throwing darts at my photo), but I'm working on it.

2 Cups of Honesty

Mix in equal parts humility and honesty. I would recommend the organic kind, found in non-processed, real-food stores.

When I used to make up stories about walking funny because of a "sports injury," I felt good about myself for about five minutes. I was trying so hard to be something that I mistakenly thought made me look and seem better. I was trying to be a perfect person who had that perfect kind of person accident on a ski slope somewhere in Aspen.

Perfectionism is actually more about the fear of not being accepted or good enough. People try so hard not to show the things about themselves that in reality make them more likable and relatable to others.

Telling your story does not have to define your forever interactions with people. But, if you are honest with people and tell it (but don't overdo it), it can, and will, make you closer.

2 Tablespoons of Empathy

Try not to make assumptions and be judgmental about someone else's cart. If you knew I had physical limitations and saw my basket filled with overpriced, prepared, specialty-store foods, you might assume I can't cook. Ok, well that's true, as you already know, but you get the point. You may think it has something to do with my physical challenges when in reality it's more a preference than a limitation. Don't worry, my kids' dad cooks, and they are well nourished half of the time.

In order to get what you want from others in a way that feels good for both parties, you need to get out of your own head and also be aware of what others may want and need. We never really know what's going on in other people's lives. If you want people to begin understanding your cues, then start paying attention to theirs. Everyone has stuff.

A Pinch of Humor

Humor is the ultimate dipping sauce for everything. It gives bland items flavor and makes delicious french fries taste even better.

When asking for help with something, like carrying your plate of food for you in a buffet line, make a joke to ease your discomfort. Say for example, "Do you mind helping me carry this plate to my table? I know if I try with my poor balance, it's gonna end up all over that woman in the white dress."

The Secret Sauce

Asking for help takes practice. Try taking very small bites and chew slowly.

You can learn to ask for what you want without feeling like you're a weak person for doing so. In fact, by using the right mix of ingredients, you will feel far more confident, happier, and have higher quality interpersonal relationships.

As a wise person once said, "You can do anything, but not everything." Be that badass woman who is smart and self-reliant enough to ask for help when you need it.





Above:
10th Annual Card Party for
CMT2A

HNF EVENTS recap

COURTNEY HOLLETT, EXECUTIVE DIRECTOR, HNF

10th Annual Card Party for CMT2A

On March 22, 2019 dedicated H.E.L.P. (Help Elliot Live Proud) Fund supporters participated in the 10th Annual Card Party Brunch and Boutique at Broken Sound Country Club, in Boca Raton. Record numbers flocked into the picturesque country club for a day filled with gourmet food, a boutique, raffles, cards and a silent auction.

HNF longtime supporter, Iris Adler, is dedicated to making this event a success year after year, and we are grateful for her dedication. Many new faces attended this year, and new faces equals spreading awareness for Charcot-Marie-Tooth, the No. 1 inherited neuropathy. One regular attendee noted, "I always look forward to this event year after year – and what a great cause". HNF is proud to report that it raised more than \$35,000 to fund research

for CMT2. The Adler family would like to thank all the participants, volunteers and individuals who donated and those who were unable to attend, but with us in spirit! It's thanks to people like you that we WILL find a cure for CMT!

For more information on H.E.L.P. Fund or to support CMT2 research, [click here. www.hnf-cure.org/cmt-2c](http://www.hnf-cure.org/cmt-2c)

Documentary

Love and disability took to the stage and big screen on February 8th at the Fashion Institute of Technology in Manhattan. HNF proudly presented a special evening and screening of the groundbreaking documentary, *Take a Look at This Heart* by director Ben Duffy.

Guests were greeted at the door with roses and snacks, as well as with special demos by MOTUS Made for Movement

and Dreamy Eyes Makeup Artistry by Jessica Ruiz. The night kicked off with welcoming remarks by moderator and HNF Medical Outreach Manager, Estela Lugo, who noted: "Why has society been so scared to talk about disability, love, dating and sexuality? Well, tonight that changes... tonight we are breaking stigmas."

F.I.T. Professors CJ Yeh and Christie Shin provided insight into how they promote inclusive design into their program, Cynda Media lab. Students are challenged to break both cultural and physical barriers through creative solutions and technologies for improved quality of life. An app designed to help individuals living with Parkinson's was presented as a prime example of how creativity and purpose are the ultimate partners for progress. The concept video for Re-Collect was designed by student Emily Frost and can be [viewed here: https://bit.ly/2J6j4e4](https://bit.ly/2J6j4e4)

Comedian and trailblazer Chris Crespo stirred up plenty of edgy laughter with his witty perspective of life and dating with a disability. He joked: "I recently applied to be a male dancer but didn't get the job... I was disappointed because I wanted to ruin at least one bachelorette party... hey! Which one of you sexy ladies parked in the Handicapped spot, cuz I'm gonna need you to move."

Next to the stage was TV producer and writer Rebecca Etchberger, debuting her new mini-film on dating with CMT. Her clever, funny and vulnerable film, *Date-ABLE*, dove into the "inner voice" and insecurities that come along with meeting someone for the first time while managing CMT symptoms. Stay tuned for our online release. You'll want to swipe right for this one!

Finally, it was time for the main event. The opening credits for *"Take a Look at This Heart"* appeared on the screen and, for the next hour and forty minutes, the audience was transported into the lives of 17 people living with disability as they shared their intimate lives and deepest insecurities. It wasn't hard to hear the full array of emotions across the audience ranging from laughter, shock, heartbreak, joy and a few sniffles (us included).

Our panel of ten influential disability advocates soon made their way to the stage to discuss some of the top themes from the film, as well as their personal experiences and perspectives. Panelists included interabled couple and rising YouTube stars Cole Sydnor and Charisma Jamison from their "Roll with Cole" channel. Bernadette Scarduzio and Richard Cole represented the CMT community with insight on their unique challenges with relationships and self worth. Jessica Ruiz spoke about her unconventional break into the world of fashion and makeup, despite not having the use of her arms or hands. Danyiah Manderson shed light on life as a single mother and NYC teacher, while Chris Crespo and girlfriend, Kathryn Jakubik, spoke on unconditional love and instant connection. Paralympic competitor and paraplegic Garrison Redd highlighted the importance of confidence, and broke the audience into laughter as he recalled his college "party days" and

credited his wheelchair as a "chick magnet". Published NYC photographer Sophie Klafter spoke on the depth of relationships between people with disabilities, and the beauty of caring for one another. Dan Garcia, executive producer on the film, described his personal motivation for wanting to be part of the project, along with some of the biggest challenges in seeing it through to completion.

We could not be more honored to have shared the stage and evening with these incredible thought leaders, or more grateful for all of those who attended and supported this event. HNF will continue to raise awareness on this important and universal topic until stigmas and shame around disability and intimacy are eliminated.

To view the full panel session, please [click here:](https://youtu.be/sz74W7YDWMs)
<https://youtu.be/sz74W7YDWMs>

Take a Look at This Heart is now available to download on iTunes or Amazon

Rare Disease Day 2019

On Thursday, February 28, 2019 the HNF team was out on the town spreading awareness for CMT.

Allison Moore, HNF's CEO, presented at PRA Health Sciences, a leading CRO in Rare Diseases on the importance of collaboration to accelerate therapies for CMT! She sported blue GENES in honor of Rare Disease Day!

Courtney Hollett, HNF's Executive Director, attended Rare Disease Day at the National Institute of Health (NIH). Thank you to Inspire for highlighting HNF's CMT Inspire Community and Dr. Mike Shy for representing the The Inherited Neuropathy Consortium (INC) RDCRC.

The atmosphere was electric as hundreds of Rare Disease advocates came together to show their stripes!



Above:
Allison Moore at Rare Disease Day 2019

Left:
Take a Look at This Heart documentary Q&A after the screening.

HNF's CMT Centers of Excellence

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

CALIFORNIA

Cedars-Sinai Medical Center
Los Angeles, CA
Contact: Dana Fine
310-423-8497 Dana.Fine@cshs.org

**Stanford Neuroscience Health Center
Neuromuscular Clinic**
Palo Alto, CA
Contact: Jennifer Fisher
jnfisher@stanford.edu

CONNECTICUT

Hospital for Special Care
New Britain, CT
Contact: Sharon McDermott
860-612-6305

FLORIDA

University of Florida Health
Gainesville, FL
Contact: Tracie Kurtz, RN, CCRP
352-273-8517 tlkurtz@ufl.edu

University of Miami
Miami, FL
Contact: Meri Jaime (for appointments)
305-243-7400 MJaime@med.miami.edu

ILLINOIS

**Ann and Robert H. Lurie Children's Hospital
of Chicago***
225 East Chicago Avenue
Chicago, IL 60611
Contact: 312-227-4471

KANSAS

University of Kansas Medical Center
Kansas City, KS
Contact: Nicole Jenci
913-945-9934 njenci@kumc.edu

MASSACHUSETTS

Brigham and Women's Hospital
Boston, MA
Contact: Kristen Roe
617-525-6763 kroe@partners.org

MICHIGAN

University Of Michigan
Ann Arbor MI
Contact: Keianna Banbury
734-763-2554 kbanbury@med.umich.edu

MINNESOTA

University of Minnesota Health
Maple Grove, MN
For Research Studies:
612-624-7745 CNRU@umn.edu
For Clinic Appointments:
763-898-1080

MISSOURI

St. Louis University Medical Center
St. Louis, MO
Contact: Susan Eller
314-977-4867 ellersc@slu.edu

MU Health Care:

Columbia, MO
Contact: Dr. Raghav Govindarajan
573-882-1515 govindarajanr@health.missouri.edu

NEW JERSEY

Hackensack University Medical Center
Hackensack, NJ
Contact: Annerys Santos
551-996-8100
Annerys.Santos@HackensackMeridian.org

Atlantic Health System*

Morristown, NJ
Contact: Dr. Jahannaz Dastgir
973-971-5700
jahannaz.dastgir@atlantichealth.org

NEW YORK

Columbia University
New York, NY
For clinical appointments: Allan Paras
212-305-0405
For research studies:
212-305-6035 ap3476@cumc.columbia.edu

WASHINGTON

St. Luke's Rehabilitation Institute
Spokane, WA
Contact: Ann Cooper
509-939-8079 coopera@st-lukes.org

* Pediatric Center of Excellence

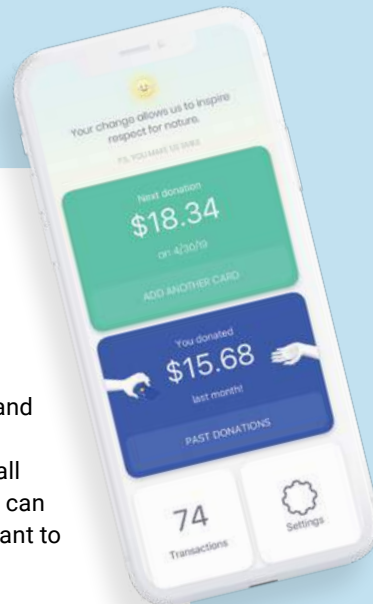
Help Support HNF with the RoundUp app!

HNF has partnered with an app called RoundUp that allows you to round up and donate the change from your credit or debit card purchases to support us – all automatically and without hassle. You can even cap the maximum amount you want to donate in a given month. [CLICK HERE](#).

If you would be willing to support us in this way, simply download the app or use the web version at roundupapp.com. You will be able to create an account and choose us when prompted to select the organization you will support. Also, we would love for you to spread the word to other individuals who may be interested!

Please let us know if I can answer any questions
courtney@hnf-cure.org

Thanks,
Courtney



HEREDITARY
NEUROPATHY
FOUNDATION

401 Park Avenue South
10th Floor
New York, NY 10016
hnf-cure.org

HNF
401 Park Avenue South
10th Floor
New York, NY 10016
hnf-cure.org

Team CMT Event Opportunities

HNF is proud to be a charitable partner for the following three events in 2019. Each event is a Team CMT event. All funds raised will go to HNF's TRIAD program for CMT research.

TD Bank Five Boro Bike Tour

SUNDAY, MAY 5TH

New York City

This is the ninth time that HNF's Team CMT has participated as a charity rider in the 2019 TD Bank Five Boro Bike Tour, America's largest cycling event. Team CMT riders will cover the challenging 42 miles through the five NY boroughs, cycling to increase awareness of CMT and raising thousands more dollars to fund research that will lead to treatments and cures. The 42-mile tour begins in Lower Manhattan and heads north through the heart of Central Park, and continues on to Harlem and the Bronx. Riders then return south along the East River on the FDR Drive crossing into Queens, and then into Brooklyn. Riders take over the Brooklyn-Queens Expressway before tackling the Verrazano-Narrows Bridge and, finally, into the Finish Festival on Staten Island.

Donate to a Team CMT rider here:
hnf.donorpages.com/BIKENEWYORK2019/

2019 Million Dollar Bike Ride

SATURDAY, JUNE 8TH

Philadelphia

HNF's Team CMT will be participating in the Million Dollar Bike Ride (MDBR) for the first time this year. The Penn Medicine Orphan Disease Center (ODC) will host the 6th Annual Million Dollar Bike Ride on Saturday, June 8th to raise money for rare disease research. The MDBR brings more than 500 cyclists and volunteers to Penn's campus to ride either 13, 34, or 72 miles starting in the city, and ranging across the Greater Philadelphia region. The Million Dollar Bike Race has raised over 8 million dollars for rare disease research. HNF is excited to participate in the race, joining other rare disease teams as we raise money to find cures and treatments. All riders are required to raise a minimum of \$250 for Charcot-Marie-Tooth Research.

Contact courtney@hnf-cure.org to join the team.

TCS New York City Marathon

SUNDAY, NOVEMBER 3RD

New York City

We are thrilled to be headed back for the second time to the TCS NYC Marathon. The annual marathon course takes the runners through the five boroughs of New York City. In 2018, the race had more than 52,000 finishers, the largest field event in history. Team CMT participated in the 26.2 mile race through the Big Apple last year and celebrated as HNF's Founder/CEO, Allison Moore crossed the finish line with her friend and HNF Chairman, Joy Kaye.

To join HNF's Team CMT at any of these events, please email courtney@hnf-cure.org



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