HEREDITARY
NEUROPATHY
FOUNDATION

**SUMMER** 2021

# cmtupdate

## CMT THRIVE!

**September Awareness Month**

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TRIAD

**Growing CMT4A Stem Cells**

Pg. 4

## 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

# CMT UPDATE
# SUMMER 2021

**Allison T. Moore**
Founder and CEO
Hereditary Neuropathy Foundation

As I write this letter, I am astonished by how quickly this summer is going! We are busy with the planning of what we hope will be the most successful CMT Awareness Month this September. We want you to show the world how we THRIVE with CMT! Send in your photos and explanations of how you THRIVE and please ask your friends and family to vote and spread the word that CMT can be a challenge, and we deserve treatments. We want to continue to build on the momentum from our meeting with the FDA and spark more industry interest in committing to the CMT disease market.

In this edition of the *CMT Update*, I'm proud to share the progress HNF is making on addressing the more rare types: GDAP1 and C12orf65. I was lucky enough to meet the Kohler family when they made a visit to the Saporta Lab in Miami to provide skin fibroblasts which will be used to create stem cells to test gene therapy approaches to treating GDAP1 (CMT4A). This project is funded by HNF. You may remember their community helped raise over $48,000 for this research project which included their creative and fun, "Grow or Mow" campaign. We are also collecting critical data about the specific signs, symptoms and progression of these rare types of CMT with what are called natural history studies in our Global Registry of Inherited Neuropathies (GRIN).

For those with CMT1A, the PREMIER Trial - Pivotal Phase 3 Trial of Pharnext's PXT3003 has started. All of the sites and details about the trial can be found **here**.

Patient and Family Support is, and will continue to be a huge part of HNF's mission. Our newest partnership with Pawsitive Pawsibilities, Tabitha Bell's non-profit organization with a mission to raise funds to purchase and train service dogs for children and young adults and to raise awareness about the blessings these dogs bring to their companions in the way of independence and mobility, is going to be so fun and rewarding. We can't wait to follow Reagan on her journey to get a service dog! Other tools we have included in this edition include the Neurotoxic Drug Caution – Chemotherapy and access to two, new HNF Centers of Excellence.

HNF is powered BY patients, FOR patients. We need your support to continue providing our services and programs. There are so many ways you can help! We need sponsors for our Movement is Medicine™ platform. We need runners for the TCS New York Marathon. We need you to choose HNF as your charity with amazon and eBay. And if you are living with CMT, we need your valuable input. Our Global Registry for Inherited Neuropathies (GRIN) patient registry with the newly formed "CMT Medical Journal" can enable anyone, from anywhere, to enroll in natural history studies that are a necessity in the drug discovery paradigm. HNF's intention is to substantially speed the pace of clinical research with an adjunct-innovative approach to support the exceptional talent of the CMT scientific community as well as to support industry. Join **GRIN** today and then express your interest in participating in the "CMT Medical Journal" pilot study by emailing **registrycoordinator@hnf-cure.org**.

We need your help to raise the funding to continue to support this important research. You'll find several ideas about how you can do that in this CMT Update. By supporting these projects, you'll be opening the door to research for other types of CMT. Together, we will continue to THRIVE with CMT while continuing to raise awareness and advocate for treatments for all types of CMT.

Enjoy the rest of your summer. I can't wait to see your photos!

Best,

Allison T. Moore

Allison T. Moore

# SEPTEMBER AWARENESS MONTH

# “CMT THRIVE”

This September HNF wants to help CMT’ers and their families “THRIVE.” Help us to spread awareness and fundraise for CMT research. Show us how you “THRIVE” with CMT!

Anna blows bubbles for respiratory therapy

## 1. “THRIVE” Photo Contest Fundraise with $1 a vote!

Snap a photo showing how you THRIVE with a short description for a chance to have your photo featured on the cover of our *CMT Update* and other prizes!

All photos and descriptions will be uploaded to this website to share with your friends and family for voting.

Votes can be purchased for a $1 donation each. The top three photos will win a $50 Amazon Gift Card. First place will be featured on the cover of our Fall *CMT Update*!

**RULES:**

- Submit your photo to CMTthrive@hnf-cure.org with the subject line: "CMT Thrive Photo"
- Describe your photo and how you THRIVE (limit 100 words)
- Submit your photo as an email attachment under 2MB in file size
- One submission per person
- Include your full name in your email

**QUESTIONS? PLEASE EMAIL:**
CMTthrive@hnf-cure.org

## 2. Band Together for CMT

Let’s continue to THRIVE with Band Together for CMT exercise kits! Each kit includes an exclusive guide book of CMT-specific exercises and three resistance bands. As a special event, our very own Julie Stone will be offering two classes using the bands and demonstrating fun and creative ways to stay strong and THRIVE! Build strength while boosting stamina, flexibility, range of motion and more!

**Julie will offer two classes this September.**

- Friday, September, 17th at 12:00pm PDT/3:00pm EDT
- Sunday, September, 26th at 10:00am PDT/1:00pm EDT

Register today! Click Here!

Get your exclusive guide book of CMT-specific exercises plus three bands with every $25 donation.

### Let’s Band Together for CMT today!

**ORDER YOUR BOOK AND BANDS TODAY!** Click here!

> *“Start where you are. Use what you have. Do what you can.”*
> – Arthur Ashe

## 3. #CMTSelfCare campaign

Share. Like. Tag us! Join HNF on all social media channels to get the word out with our #CMTSelfCare campaign. Got a great **#CMTSelfCare** tip? Tag us and we’ll share!.

## 4. Facebook Frame

Show your support for CMT families and raise awareness across the globe by updating your Facebook profile photo.

Special thanks to Tovah Fine for designing the **#CMTThrive** frame for 2021.

**Visit HNF's September CMT Awareness Month page**
Click Here!

TRIAD

THERAPEUTIC RESEARCH IN
ACCELERATED DISCOVERY

# Growing CMT4A Stem Cells:

## Kohler Family donates skin samples for GDAP1 Gene Therapy Research Program

On Monday, June 28th Alana Kohler and her family met up with Dr. Mario Saporta at the University of Miami Miller School, moving the needle forward on CMT4A (GDAP1) research.

Alana is the 2nd GDAP1 patient to have her skin fibroblast taken to advance HNF's **TRIAD gene therapy research program**. These fibroblasts will be developed into stem cells under the direction and supervision of Dr. Saporta's expert team. The stem cells will then be used to test a variety of gene therapy approaches that HNF is currently developing in partnership with biotech leaders.

*"We believe Gene Therapy has immeasurable potential in leading the first treatments of CMT and are excited whenever we hit milestones like these!"*

– Robin Kohler

*"It was only via Zoom that I had met the Kohler family until now. I'm so delighted to have finally met them in person! It takes courage and giving people to champion a cause that is so important for a rare disease like GDAP1 (CMT4A)."*

– Allison Moore

By supporting CMT4A Gene Therapy you will be opening the door to research for other forms of CMT.

Join the Kohler's and support HNF's mission. Donate today or contact courtney@hnf-cure.org to learn more.

**DONATE TODAY:** Click Here!

TRIAD
THERAPEUTIC RESEARCH IN
ACCELERATED DISCOVERY

PREMIER

# PHARNEXT PIVOTAL PHASE 3 OF PXT3003 IN CMT1A

## The Premier Trial– More Sites Are Open For Recruitment!

The PREMIER trial is a pivotal Phase 3 clinical study of the Pharnext-sponsored drug, PXT3003, for patients with mild-to-moderate CMT1A 16 to 65 years of age.

The PREMIER study will require approximately 17 months to complete for each individual patient and is divided into the following three periods:

### 1. Screening Period – Up to 35 Days

The purpose of the Screening period is to ensure eligibility for the study. You will visit the study center to take part in the informed consent process. During this time, doctors will explain everything you need to know about the study, provide written information, and answer any questions you may have.

If you choose to continue and provide informed consent, doctors will then perform a number of screening assessments to confirm your eligibility.

### 2. Treatment Period – Up to 15 Months

If you are eligible to take part in the study, you will progress to the Treatment period and be assigned to one of following two treatment groups:

1. PXT3003 taken orally, twice daily for 15 months
2. Placebo taken orally, twice daily for 15 months

Your designated treatment group will be selected at random by a computer. Study subjects will be split evenly across these groups, to ensure a 50% chance of receiving the study drug.

The study is double-blinded, which means that for the entire study, neither you nor the study doctors will know if you are receiving PXT3003 or a placebo. The placebo looks exactly like PXT3003 but does not contain any medicinally active drug.

You will attend the clinic every 3 months during the Treatment period (6 visits in total) and your study team will contact you by telephone at least twice between these visits.

### 3. Safety Follow-Up Period – 30 Days

This period includes a Safety Follow-Up visit, which will take place 30 days after the Treatment period ends. This will be the final visit in the study with the purpose of assessing your ongoing health and well-being.

**YOU CAN PRE-CHECK YOUR ELIGIBILITY BY VISITING THE PREMIER PATIENT RECRUITMENT WEBSITE.** Click here!

**You will also find additional information about the trial such as the nearest clinical trial site.**

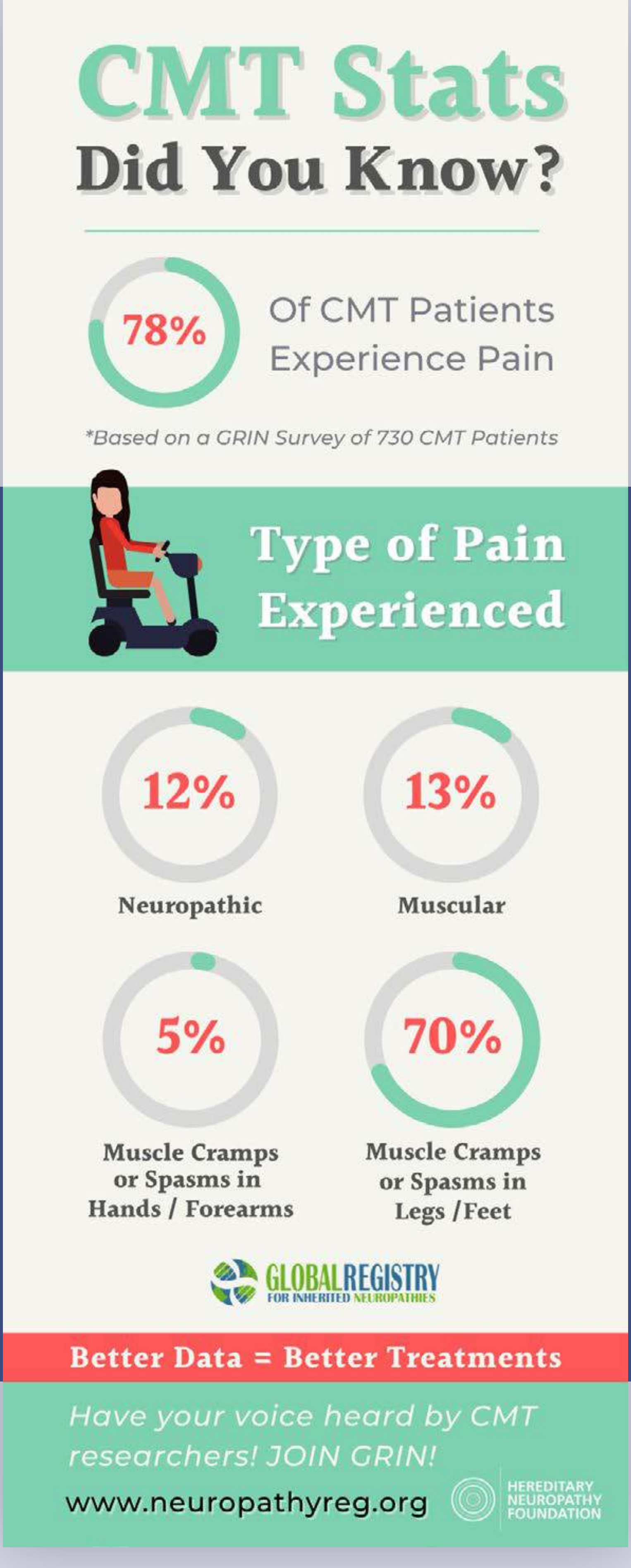
**GLOBALREGISTRY**
**FOR INHERITED NEUROPATHIES**

## As GRIN grows, our CMT voice grows!

We can only gain greater insights on important topics like this from YOU, our CMT COMMUNITY! Simply complete our GRIN surveys and help accelerate vital therapies for Charcot-Marie-Tooth (CMT) and Inherited Neuropathies.

**PLEASE JOIN GRIN TODAY!**

Click Here!



# CMT Stats

## Did You Know?

78% Of CMT Patients Experience Pain

**Based on a GRIN Survey of 730 CMT Patients*

## Type of Pain Experienced

12% Neuropathic

13% Muscular

5% Muscle Cramps or Spasms in Hands / Forearms

70% Muscle Cramps or Spasms in Legs / Feet

GLOBALREGISTRY FOR INHERITED NEUROPATHIES

**Better Data = Better Treatments**

*Have your voice heard by CMT researchers! JOIN GRIN!*

www.neuropathyreg.org

HEREDITARY NEUROPATHY FOUNDATION

# Band Together for CMT

## Julie Stone, Movement is Medicine™ Ambassador

This CMT Awareness Month let's THRIVE with Band Together for CMT exercise kits! Each kit includes an exclusive guide book of CMT-specific exercises plus 3 resistance bands.

As a special event, our very own Julie Stone will be offering 2 classes using the bands and demonstrating fun and creative ways to stay strong and THRIVE! Build strength while boosting stamina, flexibility, range of motion and more!

Julie will offer two classes this September DATES.

**Get your exclusive guide book of CMT-specific exercises plus 3 bands with every $25 donation.**

**Let's Band Together for CMT today!**

**ORDER YOUR BOOK AND BANDS TODAY!** Click here!

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## Join us weekly with new Movement is Medicine™ Classes!

Click Here!

Movement is Medicine™

# Become A Sponsor!

## Mission

Movement plays an essential role in physical and mental well-being, especially for people living with disabilities or chronic conditions like Charcot-Marie-Tooth. Our Movement is Medicine™ mission is to foster meaningful connections while virtually providing safe and **adaptive exercise & wellness classes**.

## What Makes MiM™ Unique?

Our Instructors "Get It"! MiM™ Ambassador instructors are experienced, empathetic, and live with disabilities and chronic conditions themselves. They "get it" —there's plenty of adaptive options and **ZERO judgment** in our classes! We offer choices for all ages and types of mobility while encouraging our community to offer their feedback and suggestions for optimal inclusion!

## How Your Sponsorship will help us GROW

We are committed to nurturing this program and with your support, we will:

- Provide regular updates to the Movement is Medicine™ platform & website
- Expand our mission and classes to other disability groups (i.e. Multiple Sclerosis, Cerebral Palsy, Muscular Dystrophy)
- On-board new Ambassadors and Guest Instructors
- Spread the word through increased advertising and marketing
- Increase and expand our selection of weekly classes

## What Sponsoring MiM™ says about YOU!

- You believe in empowering the disability and chronic illness community with **Education, Connection, and Wellness!**
- You believe that providing wellness for a disability or chronic illness requires a **Multifaceted Approach and can be Powerfully Provided** by individuals who can truly relate through shared experiences.
- You support true **DIVERSITY, REPRESENTATION, and INCLUSION** in the wellness community and recognize MiM's™ role in breaking barriers for people with disabilities/chronic illnesses.

**LEARN MORE ABOUT SPONSORSHIP LEVELS:** Click Here!

# "The Power of Pups: Pawsitive Pawsibilities"

HNF is excited to announce our newest partnership with Pawsitive Pawsibities. They have chosen Reagan Warren to receive a service dog trained especially for Reagan's needs. We'll be documenting this process from their first meeting through to doggie delivery and the first months with Reagan at her new school. But first, we'd like to introduce you to the amazing girl who founded Pawsitive Pawsibilities, Tabitha Bell.

### What is the story behind Pawsitive Pawsibilities?

I was first diagnosed with CMT when I was 10 years old. I had already had two orthopedic foot surgeries by that time. After my diagnosis, my health began to decline rapidly so by the time I was 12 years old, I could barely get out of a wheelchair and both of my feet atrophied quickly and dramatically. My doctor at the time told my parents to accept the reality that I'm never going to walk again and they should just buy me a motorized wheelchair. My parents and I knew I could not give up THAT easily, so instead, we looked for a new solution to a mobility aid I could actually use (I couldn't use arm crutches or a walker because I didn't have enough balance to even use those). We had been living near Camp Pendleton in San Diego, CA at the time and had heard about a type of service dog called a "balance and brace service dog." We decided that it would be the best idea to try. If the service dog didn't work, we would have a highly trained pet. I first got Sunny in September, 2012. He was a beautiful, black, two year old German Shepherd. I had just returned home post- complete spinal fusion surgery. I remember after training with Sunny for around 6 months, we finally clicked! I was able to step off a tiny curb while only holding onto Sunny's harness. After that, I went back to my foot surgeon (the same one who told me I would be in a wheelchair the rest of my life) and walked in with Sunny. He was skeptical when I first explained to him how I used Sunny. He then asked to see me walk, barefoot, down a long hospital hallway with only Sunny for balance - something I had never been able to do before. When I walked the length of that hallway without much of a wobble or hesitation, my surgeon was sold! "I need 10 more of these dogs! How much are they?" Service dogs cost around $10,000-$20,000 and are not covered by insurance, therefore most patients who need service dogs can not financially afford these dogs. That's why I started Pawsitive Pawsibilities - to help physically disabled young adults and children gain their independence. Sunny helped me build my strength because I didn't need to be dependent upon anyone. I went from falling almost 10 times a day to only falling once in a blue moon. Sunny gave me my independence and confidence and I wanted to do the same for others.

### How do you raise money for service dog donations?

Usually through private donations and word of mouth. We have also created two Super Paws 5k walk/run/rolls, and a Sunny's Spring Sing Benefit Concert. We are hoping to put on another **Super Paws 5k walk/run/roll next fall!**

### How has having a trained service dog helped you?

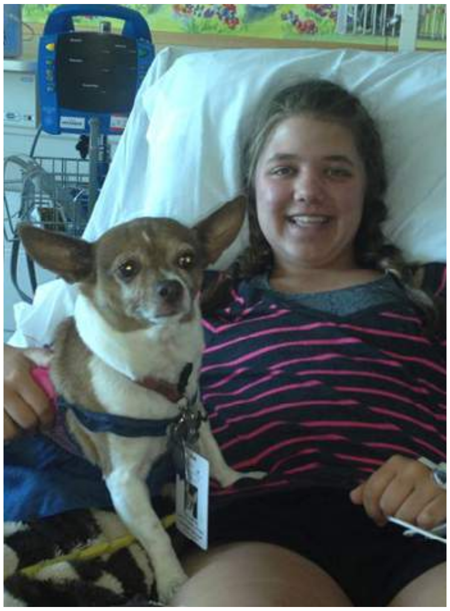
He gave me the independence I needed to start building strength and confidence. Sunny taught himself how to get my papers off the printer and he picked up anything I dropped. My second service dog, Nox, picks items up for me as well and helps me stand up off the ground if I fall. Both dogs have helped as a "counterbalance" to when I'm walking and I teeter to one side.

### How does Pawsitive Pawsibilities select it's breeders? Trainers?

Our first trainer was near where we lived in Utah. He didn't have a preference for breeders, he usually chose dogs that had not been chosen as show dogs to breed, or something happened and the dog was not able to breed. Our second trainer, Mike, we met through a close mutual friend, Larry Jerome, who is also our breeder.

### Why did you decide to help Reagan get a trained service dog? What has your interaction with her been like so far?

I have been wanting to help more younger kids, again. I got connected with HNF through Dr. Pfeffer. Dr. Pfeffer encouraged me to work with a younger person and that's when HNF told me about Reagan. Hearing Reagan's life story was like hearing my own story from when I was her age. I have loved every



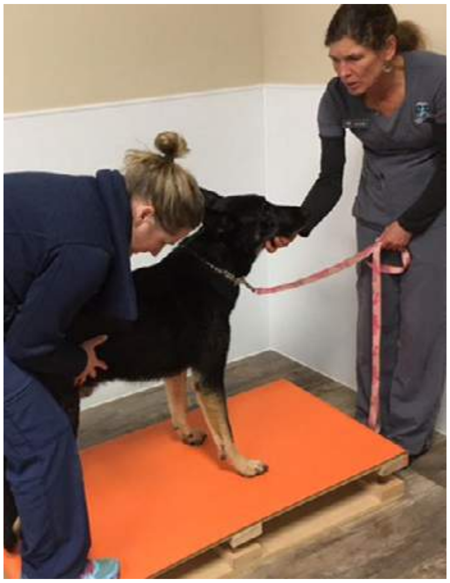
minute of getting to know her and her wonderful mom, Brooke.

**What do you hope Reagan and Atlas will accomplish together?**

I hope Reagan will gain the confidence and independence she deserves to achieve a life she wants and dreams of.

**After the dog is placed with the family do you have any other interaction with them?**

It all depends on the family and how much support and interaction they want and need. We are always here for any questions and support the family might need after the placement.



# Team CMT – 2021

*Congratulations to Brad Floyd for completing the Ironman Tulsa!!*

# **Runners Wanted:** TCS New York City Marathon

## Sunday, November 7th, NYC.

We are thrilled to be headed back for the third time to the TCS NYC Marathon, an exciting annual marathon course taking runners through the five beautiful boroughs of New York City! Team-CMT 2021 slots are filling up fast, with only a handful of remaining spots! Don't miss this prestigious event in one of the most amazing cities in the world!

***TO REGISTER & RUN, EMAIL:** courtney@hnf-cure.org

# **JOIN** Team CMT!

Share the CMT spirit and bring awareness to your next local event. Get your exclusive Team CMT singlet & swag while supporting HNF's expanding research and community programs.

## How Team CMT Started:

In 2007, the Hereditary Neuropathy Foundation (HNF) partnered with Team CMT founder Chris Wodke. Chris lives with CMT and has been a fierce advocate in promoting exercise for the CMT community. So whether members sport their athletic wear to the gym, for a stroll in the park, or at an event, awareness of CMT is being shared!

**JOIN TEAM CMT!** Click Here!

*"It was a wet and nasty day but there is something about representing the CMT Gear that gives you an extra push. Proud to represent the team and anxious to see it grow."*

**Congratulations to Marc Daigle for completing Verizon New York City Triathlon on July 11, 2021.**

**PARTICIPATE IN MARC'S MONTHLY MOVEMENT IS MEDICINE™ CLASS**

"Building Strength"

Click Here!

# Accessible College: NEW CMT-Connect Webinar!

**The transition to college is challenging for all students.** For students with physical disabilities and/or health conditions, navigating the process and ensuring that needs will be met is essential. HNF is proud to announce that we have teamed up with Accessible College to host an informative and interactive webinar on **Thurs. 9/16 @7pm EDT** to help provide families and individuals with an introduction to the skills and tools needed to ensure a successful transition to and through higher education.

Annie Tulkin is the Founder and Director of Accessible College, as well as an author and public speaker. She is an expert and coach in the area of college transition for students with physical disabilities and health conditions. Annie found her passion for working with students with disabilities when she was the Associate Director of the Academic Resource Center at Georgetown University. While at Georgetown, Annie discovered that many of the students she worked with had not received transition support to prepare them for the transition to college with a physical disability and/or health condition.

**Accessible College's** services help to bridge the gaps, empowering students and families to effectively work together to create positive educational experiences for students and support college success.

> *"We understand the unique needs of students with physical disabilities and health conditions and the challenges that may arise as they plan for their transition to college. Because colleges and universities provide varying levels of support and accommodations for students with disabilities and health conditions, Accessible College's services help to bridge the gaps, empowering students and families to effectively work together to create positive educational experiences for students and support college success."*
>
> – **Annie Tulkin**
> Founder & Director

## Services for students include assistance with:

- Needs
- College Accommodations
- Medication management
- Healthcare management
- Hiring and managing a Personal Care Attendant (PCA)
- Continuity of care (including transfer of care)
- Self-advocacy skills
- Disability disclosure
- Disability documentation review and development
- Communicating with the college's Disability Support Office
- Disability specific questions for college tours and tour debriefs
- Study skills and time management

## Services for parents include assistance with:

- Understanding the role of the parent in the college accommodations process
- Identifying your questions for college tours
- Supporting your student in developing independent living skills
- Learning the difference between high school and college accommodations
- Hiring and managing a Personal Care Attendant (PCA)

If you are a high school student living with CMT and/or parent of one, you won't want to miss this valuable presentation and open discussion!

**REGISTER FOR FREE HERE WITH A CLICK!**

# Find Others Near You!

Here's how you do it:

**Step 1:**

Join the HNF CMT Inspire community.

**CLICK HERE!**

**Step 2:**

When you're in the HNF CMT community (HNF logo in the banner), on the far right, click on "Find Members."

**Step 3:**

On the left side of the page, you can enter and search by zip code, state, country, age or gender.

**Step 4:**

Send them a message to introduce yourself and make a plan to meet in person.

# Are you active on Ebay?

The Hereditary Neuropathy Foundation can earn research dollars when you choose us as your charity.

**Selling for charity is simple!**

**Step 1:**

After you complete your item to sell there is an option “Donate a percentage of your sale to the charity of your choice and we'll give you a credit on basic selling fees for sold items.” Click on the search field and **select the Hereditary Neuropathy Foundation as your charity**.

**Step 2:**

**Sell & Ship**

Sell and ship to your customer as usual. No additional steps or paperwork required.

**Step 3:**

**Receive a Donation Receipt**

Within a few weeks, and after the buyer has paid, PayPal Giving Fund will automatically send the donation to the Hereditary Neuropathy Foundation and send you a donation receipt. eBay will also credit you an equal percentage of your fees.

# Earn Research Dollars While You Shop!

To start donating, it’s super easy! Please click the link below to designate the Hereditary Neuropathy Foundation as your charity. Everytime you shop on Amazon, a portion will go towards the Hereditary Neuropathy Foundation and the many families living with CMT.

**CLICK HERE!**

# Dear Lainie,

I feel so lucky to have finally found your site. I, too am 50 - well 52 on Saturday. I have CMT and have been in AFOs - custom for 6 years. I am moderate in severity and I have not found a doctor who truly understands CMT much less what it is like being a vain woman with this crazy disability.  I need help with everything (new AFOs, shoes, love the skinny jeans idea, etc). This might sound crazy but I am not worried about the cost anymore, I just want to feel normal. Any suggestions you can give me I would be so thankful. I have a wedding coming up, my son graduates from high school this year and so many other exciting things to do this summer that are stressing me out. I'm not only worried about what I'm gonna wear, but also how I'll manage my nerves around the anticipation on having to stand for long periods of time. Thank you so much for your site.

– Lee-Ann from Nashville

. . . . . . . . . . .

### Hi Lee-Ann:

I am so happy you found **Trend-Able** and took the time to reach out. You do not "sound crazy" at all; Many of us living with CMT can relate! It is not vain to want to look and feel like yourself and to be able to wear clothing and shoes that help to express your own sense of style and personality.

Congratulations on your son's graduation and upcoming wedding, etc. It's both exciting and anxiety provoking to be able to partake in these type of celebrations again. I can definitely help with the what to wear part and give you some tips to help reduce some of the stress around attending outdoor events when you have CMT.

Check out **this post** on what to wear to a summer wedding with AFO'S. You'll see several examples of pull-on style jumpsuits and maxi-dresses that are not only easy to get on and off, they're also comfortable and stylish to wear to any summer celebration. You can pair them with a cute pair of **flat AFO friendly sandals**, or even a **cute pair of wide width sneakers**.

When it comes to managing your anxiety around attending summer weddings and other outdoor events, it helps to be proactive and be as prepared as possible for managing the heat, terrain, and other unknowns. Here are some **do's and don't's for attending a summer party when you have a disability** that I hope will help! Now, go and enjoy!

– **Lainie**

Lainie Ishbia, MSW
Disability Speaker & Blogger
Trend-ABLE

# NEUROTOXIC DRUG CAUTION

**CORINNE WEINSTEIN,** CLINICAL ONCOLOGY PHARMACIST AT CANCER CENTERS OF COLORADO-GOOD SAMARITAN, DENVER, COLORADO

## Chemotherapy (Taxanes/Vinca Alkaloids/Platinum Drugs)

While many different types of chemotherapy can cause neuropathy, certain types have an especially high risk. These include taxanes, vinca alkaloids, and platinum based agents.

| Taxanes | Vinca Alkaloids | Platinum-Based Agents |
|---|---|---|
| • Paclitaxel (Taxol®), | • Vincristine | • Carboplatin |
| • Nab-paclitaxel (Abraxane®) | • Vinblastine | • Cisplatin |
| • Docetaxel (Taxotere®) | • Vinorelbine (Navelbine®) | • Oxaliplatin |

### HOW DO THESE AGENTS CAUSE/INDUCE NEUROPATHY?

#### Taxanes

※ Taxanes are used to treat a variety of cancers including breast, ovarian, lung, bladder, prostate, and other solid tumors. Taxanes act as “microtubule-stabilizing agents”, interfering with the normal assembly/disassembly of microtubules (Microtubules are the 'highway' or 'railway' systems of the peripheral nerve, allowing nutrients to reach from the cell core (at the spinal cord) to the peripheral location in the hands or feet). This loss of stability stops mitosis and prevents division of the cell. While effective in killing rapidly dividing tumor cells, the same action is thought to affect cells in healthy peripheral nerves. Because microtubules act as platforms to transport nutrients, neurotransmitters, and mitochondria from the cell body to the periphery, this causes changes to the physiology of the peripheral nerve.

※ Taxane induced peripheral neuropathy (TIPN) presents most often in a sensory fashion with common symptoms including: sensory disturbance, loss of temperature/vibration sensation, and pain in the extremities (mostly hands and feet).

※ Unfortunately, TIPN is extremely common, affecting 80%-97% of patients, often requiring treatment delays and/or dose reductions. Risk increases with higher cumulative doses, combination therapy, and longer durations of treatment. While TIPN can occur with all taxanes, it has been reported that paclitaxel may pose a more significant risk compared to docetaxel.

#### Vinca Alkaloids

※ Vinca alkaloids are drugs developed from the Madagascar periwinkle plant. They are used to treat various tumors, such as Hodgkin and non–Hodgkin lymphoma, testicular cancer and non–small cell lung cancer. Vinca alkaloids inhibit the assembly of microtubules, promoting their disassembly and inducing cell death. While effective in killing tumor cells, they also disrupt the integrity of axonal microtubules, thus contributing to the signs/symptoms of neuropathy.

※ Vinca alkaloid neuropathy often presents as sensorimotor neuropathy (affecting both sensory and motor neurons). Symptoms will include numbness/tingling, muscle weakness, foot drop, impaired balance, and loss of sensory/vibration sensitivity. Autonomic symptoms can also occur including constipation, urinary incontinence and hypotension. While this risk occurs with all vinca alkaloids, vincristine seems to pose the highest risk.

※ Neuropathy symptoms can occur with as little as one dose. There are numerous case reports of patients with CMT1A (many of which were unaware of this diagnosis as they were asymptomatic) presenting with years of progression after just one dose of vincristine, thus revealing their CMT diagnosis.

#### Platinum Based Agents

※ **Platinum based chemotherapy treats a wide variety of cancers including head and neck, ovarian, bladder, and testicular. Platinum derivatives bind to DNA and ultimately inhibits the cancer cell’s transcription and translation, leading to cell death. The exact mechanism of platinum based neuropathy isn’t completely understood but is thought that cell death of dorsal root ganglion neurons (a cluster of neurons in the dorsal root of the spine), is largely contributory.**

※ **Symptoms are predominantly sensory in nature and include sensory disturbance, loss of temperature/vibration sensation, and pain in the extremities. Oxaliplatin can also cause cold insensitivity while cisplatin can cause damage to the inner ear (impairing hearing and balance).**

※ **Prolonged treatment and higher cumulative doses increase the risk of neuropathy from these compounds. Of note, carboplatin seems to have the lowest risk of neurotoxicity.**

### Does chemotherapy have an added risk in patients with CMT?

- YES! Preexisting conditions such as CMT, chemotherapy can severely exacerbate signs and symptoms of CMT and accelerate progression of the disease, leading to further disability. Even asymptomatic cases of CMT can be unmasked by the neurotoxic effects of these chemotherapies (most often with vincristine), with as little as one dose. Other preexisting risk factors include diabetes, advanced age, smoking, and increased alcohol consumption.
- While chemotherapy induced peripheral neuropathy (CIPN) is often reversible once the neurotoxic agent is stopped, this is not always the case for patients with CMT. This further emphasizes the need to alert oncologists to your diagnosis.

### What should I do if I need chemotherapy?

- Tell your doctor about your CMT! Unfortunately, most oncologists are not familiar with CMT and other inherited neuropathies. Informing them of your preexisting neuropathy will help them choose agents that are less neurotoxic.
- Unfortunately, sometimes a decision between lifesaving chemotherapy and the progression of CMT symptoms will need to take place. Again this warrants a conversation with your oncologist. If a neurotoxic chemotherapy is needed, your oncologist may be able to start with a lower dose, shorten the duration of chemotherapy, and closely monitor any signs/symptoms or worsening neuropathy.

### Research

- Currently, no treatments exist for chemotherapy induced peripheral neuropathy (CIPN). However, research is being done to help mitigate these debilitating side effects. Luckily for CMT patients, this research has the ability to cross over into the CMT population, and poses the potential for treatment across both populations.
  - HDAC6 inhibitors, for instance, have shown promising preclinical results in CIPN and are currently being studied in CMT.

# ASK THE EXPERT

Do you ever wish you could have direct access to a neurologist for your CMT questions? Now you can! HNF continues to feature real questions from CMT patients across many topics. Submit your questions from our site to the Directors of the HNF Centers of Excellence, Dr. Jafar Kafaie for St. Louis University School of Medicine, St. Louis, MO, and Dr. Florian Thomas for Hackensack University Medical Center & Hackensack Meridian School of Medicine, Hackensack, NJ

**Q: Is 80 mg of lipitor a good choice for lowering your cholesterol when you have CMT1A?**

**A: Dr. Florian Thomas:**

Statins are classified as having minor or uncertain neurotoxic properties. **Click Here**.

As such their use in CMT must be an individualized decision between the patient & their PCP that considers the risk of taking them vs the risk of not taking them.

**HAVE A QUESTION?**
www.hnf-cure.org/ask-the-expert

# New Center of Excellence: Oregon Neurology

**MICHAEL BALM**, MD, OREGON NEUROLOGY

**Q: TELL US ABOUT YOURSELF:**

I am Mayo Clinic Neurology trained, with an additional fellowship (also at Mayo) in Neurophysiology. I have American Board of Psychiatry and Neurology added qualifications in Clinical Neurophysiology, and have been in private practice for 25 years. I am married and enjoy outdoor activities as well as local, regional, national, and international travel.

**Q: WHY IS CMT YOUR PASSION?**

It is an opportunity to use my experience and expertise to provide care and support to this specific group of individuals, with goals of symptom management, disease education, lifestyle enhancement, and whenever available, access to clinical research opportunities.

**Q: TELL US WHAT PATIENTS WILL EXPERIENCE WHEN THEY COME TO YOUR CENTER?**

- Excellent supportive staff to guide them through every step of the clinic experience
- Compassionate care and education, with time for all patient questions/ concerns to be addressed
- In house physical, occupational and speech therapy
- Coordination of care with local physiatrists, orthopedists, orthotists, and other specialists
- Referrals to social services and behavioral health as well

**Q: WHAT DO YOU LOVE MOST ABOUT YOUR PRACTICE?**

The people and the excellent team at our clinic, and the interesting individuals we meet as clients.

**Q: HOW CAN PATIENTS MAKE AN APPOINTMENT AT YOUR CENTER?**

Either referral from an existing provider, or by calling our clinic directly. Necessary information can be submitted through our website, once contacted by phone.

1 Hayden Bridge Way
Springfield, OR 97477
Phone: 541-868-9430
Fax: 541-868-9450

*Currently recruiting CMT1A patients for the Premier Trial **Click here!**

# New Center of Excellence:
# Rush University, Chicago IL

**RYAN JACOBSON MD**

**Q: TELL US ABOUT YOURSELF:**

I am a neuromuscular specialist at Rush University in Chicago. I completed my neurology and neuromuscular training at the University of Michigan in Ann Arbor, Michigan. In addition to my clinical practice, I am very involved in the education of residents and fellows here at Rush.

**Q: WHY IS CMT YOUR PASSION?**

I really enjoy working with people with CMT. They tend to be engaged, motivated, and knowledgeable. Each individual is different and we learn a great deal from our patients in this field. There is also a lot of ongoing research with the possibility of exciting new treatments in the near future. The basic science of these diseases is also fascinating.

**Q: TELL US WHAT PATIENTS WILL EXPERIENCE WHEN THEY COME TO YOUR CENTER?**

Rush University is a large, urban academic medical center but we take a very personalized approach to make sure every person is heard and their unique situation appreciated. We are responsive to every patient’s needs and questions. We also strongly believe in multidisciplinary care for CMT, and engage a wide range of therapists and experts in this process.

**Q: WHAT DO YOU LOVE MOST ABOUT YOUR PRACTICE?**

Our practice here at Rush gives us the opportunity to partner with patients to make a positive impact on their lives.

**Q: HOW CAN PATIENTS MAKE AN APPOINTMENT AT YOUR CENTER?**

Our number for scheduling is 312-942-5936.

# 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 patients and families with inherited neuropathies.

## ARIZONA

**Barrow Neurological Institute**
240 W. Thomas Rd., Suite 400
Phoenix, AZ 85013
602.406.6262 (O), 602.406.2565 (Fax)
**Contact:** Coordinator: Aide Raya
602.406.8144 (O)
Email: Aide.Raya@dignityhealth.org

## ARKANSAS

**Arkansas Children's Hospital**
* Pediatric Center of Excellence
1 Children's Way
Little Rock, AR 72202-3591
**Contact:** Dr. Aravindhan Veerapandiyan
Assistant Professor of Pediatric Neurology
Director Comprehensive Neuromuscular Program
Email: aveerapandiyan@uams.edu
Primary Care Appointments:
501-213-1883
Specialty Care Appointments:
501-819-3520

## CALIFORNIA

**Cedars-Sinai Medical Center**
127 S. San Vicente Blvd.
Advanced Health Sciences Pavilion, A6600
Los Angeles, CA 90048
**Contact:** Tara Jones
CMT Clinic line: (310-423-4268)
Email: tara.jones@cshs.org

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

**UCLA Department of Neurology**
300 Medical Plaza, Suite B200
Los Angeles, CA 90095
**Contact:** Dr. Halabi Anasheh
Telephone: 310-794-1195 (note: request to be scheduled with Dr. Halabi)
Fax: 310-794-7491

## COLORADO

**Children's Hospital Colorado**
* Pediatric Center of Excellence
13123 E 16th Ave,
Aurora, CO 80045
**Contact:** Alison Ballard: 720-777-8723
Clinic Schedulers: 720-777-2806

## CONNECTICUT

**Hospital for Special Care**
Charles H. Kaman Neuromuscular Center
2150 Corbin Avenue
New Britain, CT 06053
**Contact:** Angel Preece, RN:
860-612-6305; Fax: 860-612-6304
Email: APreece@hfsc.org

## FLORIDA

**University of Florida (UF) Health**
3009 Williston Road
Gainesville, FL 32608
**Contact:** Melissa Hines: 352-294-5000
E-Mail: mhines@ufl.edu
University of Miami
Professional Arts Center (PAC)
1150 NW 14th Street, 6th Floor
Miami, FL 33136
**Contact:** Roberto Fernadez
305-243-7400
E-Mail: r.fernandez5@med.miami.edu

**University of Miami**
Professional Arts Center (PAC)
1150 NW 14th Street, 6th Floor
Miami, FL 33136
**Contact**: Roberto Fernadez: 305-243-7400
E-Mail: r.fernandez5@med.miami.edu

**AdventHealth Neurology at Winter Park**
1573 W Fairbanks, Ste 210
Winter Park, FL 32789
Contact: Nivedita Jerath MD, MS
407- 303-6729
E-Mail: Orl.neuromuscular.medicine@adventhealth.com

## ILLINOIS

**Ann and Robert H. Lurie Children's Hospital of Chicago**
* Pediatric Center of Excellence
225 East Chicago Avenue
Chicago, IL 60611
**Contact:** Dr. Vamshi Rao
Call 1-800-KIDS-DOC for an appointment.
If there is already a diagnosis of CMT, call 312-227-4471

**Rush University**
600 S. Paulina Street
Chicago, IL 60612
**Contact:** Ryan Jacobson MD
Rush University Call Center: 312-942-5936
Appointments: 312-942-5936

## KANSAS

**University of Kansas Medical Center**
Landon Center on Aging
3599 Rainbow Boulevard
Mailstop 2012
Kansas City, KS 66160
**Contact:** Andrew Heim: 913-945-9926
E-Mail: aheim2@kumc.edu

## MASSACHUSETTS

**Brigham and Women's Hospital**
Department of Neurology
75 Francis St, Tower 5th Floor
Boston, MA 02115
**Contact:** Kristen Roe: 617-525-6763
E-Mail: kroe@partners.org

## MICHIGAN

**University of Michigan**
2205 Commonwealth Blvd
Ann Arbor MI 48105
**Contact:** Arija Jarvenpaa
734-763-2554 for appointments
Email: jarveari@med.umich.edu
Website: https://www.uofmhealth.org/

**St. Louis University Medical Center**
Department of Neurology
1438 South Grand Boulevard
St. Louis, MO 63104
**Contact:** Research: Molly Labrier
For Appointments: 314-977-6177
E-Mail: molly.labrier@health.slu.edu

**MU Health Care:**
Department of Neurology
1020 Hitt Street
Columbia, MO 65212
**Contact:** Dr. Karim Salame and Dr. Vovant Jones
Phone: 573-882-1515

## MINNESOTA

**M Health Fairview Clinics & Surgery Center – Maple Grove**
Neurology Clinic
14500 99th Avenue N
Maple Grove, MN 55369
For Research Studies: 612-624-7745
E-Mail: CNRU@umn.edu
For Clinic Appointments
Phone: 763-898-1080

## NEW JERSEY

**Goryeb Children's Hospital**
* Pediatric Center of Excellence
55 Madison Avenue
Morristown, NJ 07960
**Contact:** Jahannaz Dastgir: 973-971-5700
Email: jahannaz.dastgir@atlantichealth.org

**Hackensack University Medical Center**
Neuroscience Institute
360 Essex Street
3rd floor, Suite 303
Hackensack, NJ 07601
**Contact:** Dr. Florian Thomas
551-996-8100
Email: Annerys.Santos@HMHN.org

## NORTH CAROLINA

**University of North Carolina**
Dr. Rebecca Traub
194 Finley Golf Course Road, Suite 200
Chapel Hill, NC 27517
**Contact:** For clinical appointments
984-974-4401
Referral Fax: (984) 974-2285

**Atrium Health Neurosciences Institute-Charlotte**
Dr. Urvi Desai
1010 Edgehill Road North
Charlotte, NC 28207
**Contact:** For clinical appointments:
704-446-1900
Referral Fax: Ashley Clyburn
704-355-5650

## NEW YORK

**Columbia University**
Department of Neurology
710 West 168th Street
New York, NY 10032
**Contact:** For clinical appointments
212-305-0405
For research studies
212-305-6035, ask for Allan Paras
Email: ap3476@cumc.columbia.edu

## TEXAS

**Austin Neuromuscular Center**
3901 Medical Parkway, Ste. 300
Austin, TX 78756
**Contact:** Yessar Hussain, MD
512-920-0140
Website: www.austinneuromuscle.com/contact/

## OREGON

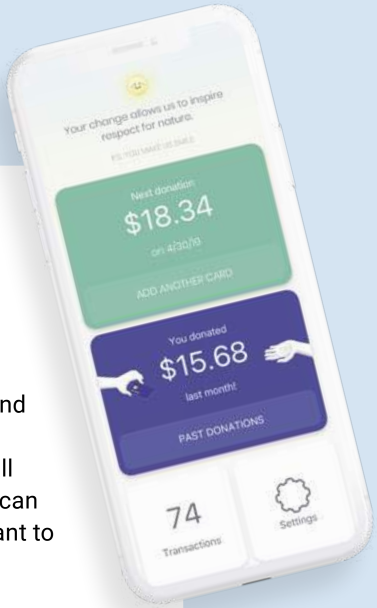
**Oregon Neurology**
Michael Balm, MD
1 Hayden Bridge Way
Springfield, OR 97477
Contact: Jody Nichols, 541-868-9430
Fax: 541-868-9450

## WASHINGTON

**St. Luke's Rehabilitation Institute**
715 South Cowley Street, Suite 210
Spokane, WA 99202
**Contact:** Dianna Russell, 509-939-8079
Email: Dianna.Russell@providence.org

**University of Washington Medical Center**
Dr. Nassim Rad
959 NE Pacific Street
Seattle, Washington 98195
CMT Scheduler Phone Number:
206.598.0058

* Pediatric Center of Excellence



Your change allows us to inspire respect for nature.

## 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**

1641 3rd Avenue
#28K
New York, NY 10128
**hnf-cure.org**

HNF
1641 3rd Avenue
#28K
New York, NY 10128
hnf-cure.org

# Events

## Get Involved and Join us at an Upcoming HNF Event

### TD Bank Five Boro Bike Tour

*Sunday, August 22nd*

Thank you for supporting HNF during the past 10 TD Bank Five Boro Bike Tours in New York City!

Team-CMT is now calling for new or return riders to join us for our 2021 tour; a unique and fun 40 mile, traffic-free ride through all five boroughs.

***To register & RIDE, email: courtney@hnf-cure.org**

### Chicago Golf Event

*October 4, 2021*

**Learn more: Click Here!**

### Jaxson Crusaders Clay Shoot

*October 16, 2021*

**Learn more: Click Here!**

### TCS New York City Marathon

*November 7, 2021*

**Learn more: Click Here!**

PHAR NEXT

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