HEREDITARY NEUROPATHY FOUNDATION

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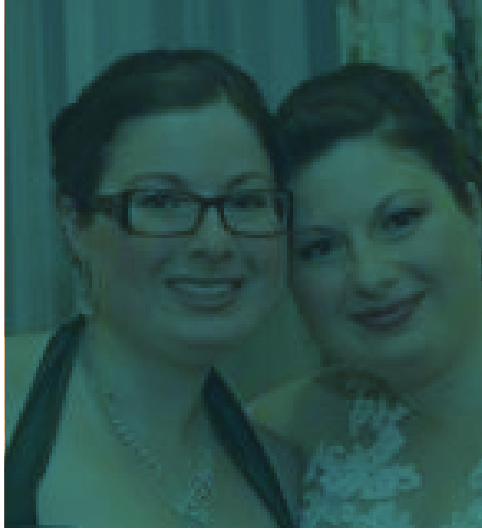

CMTupdate™

Summer 2015

September is Charcot-Marie-Tooth (CMT) Awareness Month

By Estela Lugo, Contributor - HNF

Although we are not ready to say goodbye to summer, September is fast approaching and the Hereditary Neuropathy Foundation (HNF) is once again ready to take on Charcot-Marie-Tooth (CMT) Disease. We are continuing our mission to increase awareness, raise research funds and find a cure for CMT!

How to get involved:

In September, HNF has many ways to participate in our ongoing efforts to find a cure for CMT. Here are a few exciting and fun ways for you to join us in our efforts to raise awareness and funds:

1. Join our #ShowYourHandsCMT social media campaign

We are continuing our popular Text2Give campaign this year with the theme "Show Your Hands". Learn more here. If you or someone you know has CMT, you can show your support by participating:

- Text **CMT** to **501501** to donate $10
- Snap a picture of your hands
- Share on a social media platform **#ShowYourHandsCMT**
- Share with your friends

2. Social Media Awareness with pictures

Upload the #ShowYourHandsCMT banner found on HNF's September Awareness page to update your own Facebook cover photo and/or Twitter page to help spread the word about CMT Awareness Month! Find the Facebook banner here. Find the Twitter banner here.

3. Donate while you dine out with family and friends!

Want to help fund research and build awareness?

Here is your chance to mingle with friends and family while you dine! HNF has set up fundraising events at multiple California Pizza Kitchen locations across the country. Simply visit one of these locations: http://www.hnf-cure.org/california-pizza-kitchen-campaign/ on the designated day and present our flyer to your server. 20% of your bill will be donated to CMT Research.

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.

Inside This Issue

TRIAD
THERAPEUTIC RESEARCH IN ACCELERATED DISCOVERY

HNF developed the Therapeutic Research in Accelerated Discovery (TRIAD) as 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.

If you would like to take it a step further and be the host at your local California Pizza Kitchen location, contact Courtney@hnf-cure.org to find out how.

4. Nominate yourself or a loved one as a CMT Warrior!

HNF has featured a "CMT Weekly Warrior" for the past few months on our social media platforms. This has consisted of a photo and summary to celebrate the brave individuals living with CMT. During the month of September, we want to hear about "your" CMT Warrior.

What makes a CMT Warrior?

A CMT Warrior is any patient or caregiver who inspires, exceeds expectations and pushes through boundaries for the benefit of the CMT community.

If you would like to nominate someone, please submit your story along with a photo to cmtwarrior@hnf-cure.org. Every story will be highlighted throughout the month of September on our website and social media communities.

An HNF panel will review all entries and announce our "CMT Warrior 2015" on October 5, 2015. Please submit your entry by September 30, 2015 for consideration. Our CMT Warrior will be featured in HNF's Fall CMT Update as well as on our website and social media communties. For questions or comments, email us at cmtwarrior@hnf-cure.org.

5. Host an event or start a fundraising-letter writing campaign

- Sponsor a local event in your town (i.e. Spin for a Cure) or participate in a local event and wear your Team CMT shirt to help spread awareness as you raise funds for research. If you are not a Team CMT member, you can join here.
- Send a letter out to family and friends to contribute to CMT research. To download a sample letter, click here.

Here's to another successful September! Thank you to all who help us on our journey to find a cure for CMT!

Any questions or suggestions contact:
Courtney@hnf-cure.org

HNF Sponsors 2015 Peripheral Nerve Society Conference in Canada

By Joy Aldrich, CMT Advocacy Director - HNF

Allison Moore, HNF CEO, and Joy Aldrich, HNF Advocacy Director, represented HNF as sponsors at this important conference, which was held in Chateau Mont Sainte Anne, Canada, from June 27 - July 2. Over 500 scientists, clinicians and CMT industry leaders that work specifically

on nerve diseases presented their current research projects. The last three days, specifically dedicated to CMT research, were filled with over 150 presentations and posters.

At the conference, HNF connected with their TRIAD funded researchers and were encouraged by the progress of our CMT2 research program. Andrew Grierson, PhD, Sheffield Institute of Translational Neuroscience, presented his work on the zebrafish preclinical model for CMT2A. Rachel Bailey, a postdoctoral research associate - UNC Gene Therapy Center, made a featured presentation, "Development of a Gene Therapy for Giant Axonal Neuropathy (GAN)". We were also able to connect with renown scientists such as Ludo Van Den Bosch and Veronick Benoy, KU Leuven & VIB, Belgium, who are studying the therapeutic potential of HDAC6 inhibitors in different forms of CMT2. These are important contacts as we continue the drug discovery path towards treatments for CMT2.

A conference highlight was the presentation of Michael Sereda's MD, Ph.D and Thomas Prukop, MD from the Max Planck Institute (Göttingen, Germany). They showed

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promising results obtained in their CMT 1A young rat model treated with Pharnext's PXT-3003. This might lead to the initiation of this drug candidate development in children affected with CMT 1A.

As sponsors of the conference, HNF had an information table where we were able to bring more attention to our TRIAD research programs, which emphasize collaboration and support of clinical trials. In addition, we also presented a poster with data on our clinical patient registry, GRIN. Our sponsorship not only increased awareness of our patient advocacy foundation, it opened up discussions with world-class researchers on new approaches to unlocking additional pathways to gene therapy to cure some of the rarer forms of CMT. Also, highlighted was HNF's collaboration with Jackson Labs and the newest endeavor to support a repository of mice with all forms of CMT to the research community. This is very important to scientists that are studying and understanding disease as well as experiments to validate clinical compounds for drug discovery.

It was an incredibly successful and informative week. There is an amazing amount of work being done around the world to find treatments for CMT and related inherited neuropathies!

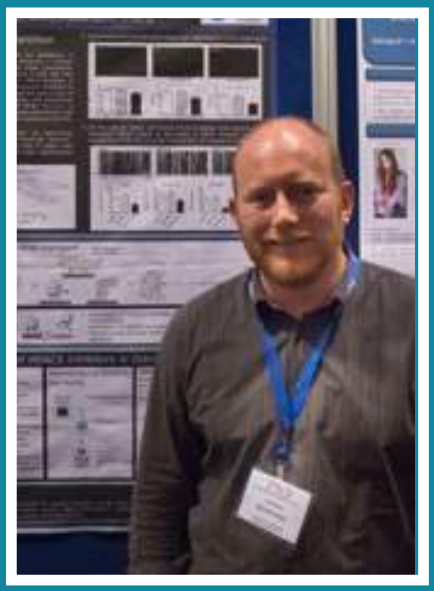

Photo Credit: Jason Butcher

What is gene therapy and how can it be used to treat inherited neuropathies?

By Rachel M. Bailey, Postdoctoral Researcher at UNC School of Medicine, Gene Therapy Center, Gray Lab

Gene therapy is one of the emerging strategies for the treatment of inherited neurological disorders by delivering therapeutic genes directly to a patient's cells instead of using drugs or surgery. In its simplest form, gene therapy provides a healthy copy of a gene to cells that carry a mutated gene that causes disease. A common approach to gene therapy is to engineer viruses found in nature, relying on their innate ability to deliver genes. In one example, a common non-disease-causing virus called AAV is used, in which all the viral genes are replaced with a therapeutic piece of DNA. In essence, scientists use the outer shell of the virus as a molecular delivery vehicle to carry therapeutic genes into a patient's body. After a single dose, AAV is capable of permanently replacing a defective gene within long-lived cells, such as nerve cells, which are key players in many peripheral neuropathies. Scientists have developed many versions of engineered AAV so that AAV can be designed to meet the precise treatment needs of a given disease by delivering a gene therapy to specific cell types with a minimal immune response.

Currently, AAV is being used to treat the inherited neurological disorder, Giant Axonal Neuropathy (GAN), in an ongoing NIH-sponsored Phase I clinical trial (NCT02362438) that began in January 2015 (https://clinicaltrials.gov/ct2/show/NCT02362438). In this trial, GAN patients are receiving a single dose of AAV carrying the human GAN gene with the aim of treating the most severe aspects of GAN, namely motor and sensory neuropathy.

The development of the GAN gene therapy started in the fall of 2008 at

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the University of North Carolina at Chapel Hill in the laboratory of Dr. Steven Gray and was supported almost entirely by Hannah's Hope Fund. Enormous efforts to fund and carry out this research enabled the fast-track of the first GAN therapy from the initial concept, to laboratory experiments, and finally to the clinic in a little over six years. Importantly, the strategy used to treat GAN can be applied to the development of gene therapies for other neurological disorders, such as CMT. Disorders caused by loss-of-function mutations in one gene, such as CMT6, are the simplest to target with gene therapy, as a virus only needs to carry and deliver a healthy copy of one gene to affected cells; however, researchers are developing more complex gene therapy approaches that have the potential to inactivate a mutated gene that is functioning improperly and to introduce a new gene to the body that helps to fight a disease. Overall, the use of gene therapy has salient therapeutic potential for many of the hereditary neuropathies.

A Bi-Directional, Translational Model of Resistance-Type Exercise Training in the Management of Charcot-Marie-Tooth (CMT) Disease

A team of government researchers, including Dr. Robert Chetlin (Associate Professor and Clinical Director of Sports Medicine at Mercyhurst University, Research Physiology Contractor in the Division of Safety Research at CDC-NIOSH), have collaborated with Dr. Michael Sereda and Dr. Klaus Nave of the Max Planck Institute for Experimental Medicine (MPI) to successfully secure the CMT1A transgenic rat from MPI and establish a colony in the United States. Presently. Initial funding for this project was supported by the Hereditary Neuropathy Foundation (HNF, New York, NY).

These government investigators, including Dr. Chetlin, are continuing to examine the effects of a validated, evidence-based mechanical loading "resistance-type" exercise protocol on these transgenic CMT1A rats. These unique animals are trained on a specialized machine, called a dynamometer (a machine used to exercise and test force, torque, and power), using stretch-shortening contractions (SSCs; coordinated muscle lengthening and shortening movements used daily in human and animal activities).

The animals' exercise routine is very tightly controlled, including all the training variables, such as the volume (reps and sets), intensity (how hard), duration (time per training session), frequency (number of training sessions per week), and progression (training variable increases) of exercise. The extent to which the animals had beneficially adapted to the exercise training was determined upon completion of the "resistance-type" training protocol. An integrated systems approach was utilized to examine and quantify exercise-induced changes in:

(1) functional performance of muscle, including strength and muscular work;

(2) muscle physiology and quality and individual myofiber histomorphology (microscopic examination of muscle anatomy, function, and pathology);

(3) the nerve coating (myelin), connection between nerve and muscle (neuromuscular junction), and the nerve itself, and; (4) the genes, proteins, and other potential "biomarkers" that may exert control over the way muscles and nerves operate and communicate with each other in response to exercise.

Collectively, these initial findings suggest that SSC-exercise training may be the preferred mode of resistance-type training, given that it enhanced static and dynamic muscle performance and muscle quality. This provides evidence suggesting that when an established fundamental model of CMT1A is mechanically loaded, using a validated, in vivo, high-intensity, "resistance-type" training regimen, a dynamic adaptive response results.

This occurrence may represent a critical direction for establishing an achievable

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A synopsis of the most recent findings, presented at the 2015 Annual Meeting of the American College of Sports Medicine (ACSM), includes:

- SSC-exercise training protocol of 4.5 weeks duration (~6-month human equivalent), employing dynamic SSCs, elicits an adaptive response in transgenic CMT1A rats.

- The training-related adaptive performance and physiological response improved relative isometric force production (static strength), maintenance of normalized muscle mass (a ratio of examined muscle mass to limb length), and a trend for improved static muscle quality (a ratio of isometric force to normalized muscle mass).

- Improved dynamic (SSC) muscle function indicated by an increase in positive work and a trend for increased cyclic force (repeated SSC force); while, additionally, there was an enhancement in dynamic muscle quality (a ratio of SSC force to normalized muscle mass).

- SSC-exercise training reduced PMP22 gene (the gene which codes for myelin production; mutations of this gene may result in CMT1A, Dejerine-Sottas Disease, or Hereditary Neuropathy with liability to Pressure Palsy) expression, which may, ultimately, improve myelin integrity (and function) in these SSC-trained animals.

translational paradigm for increased quality-of-life and independence in human CMT patients. To our knowledge, this is the first study to integrate an established animal model of CMT and investigate the effects of a validated and adaptive, resistance-type exercise training regimen on PMP22 gene expression in muscle for any animal or human CMT population.

Given the course of these experiments, we now intend to refine the exercise exposure to identify the type, quantity, and frequency of training that provides optimal functional benefit to the transgenic CMT1A rats. Additionally, future studies will also examine the possible effects of dietary supplements, and other small-molecule therapies in the transgenic rats, with and without exercise, to determine if the combination of exercise and these other interventions are more effective than any of these approaches used alone.

Disclaimer: The information presented in this article has previously been disseminated to the public at the 2015 and the National Conference of the American College of Sports Medicine. HNF, in part sponsored this research project.

Glossary

Dynamometer - A device for measuring force, torque, or power.

Myofiber histomorphology – The microscopic examination of muscle structure composition and function.

Stretch-Shortening Contractions - Coordinated muscle shortening and muscle lengthening for movements used in daily activities.

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Hot Off the Press

By: Sean Ekins, CSO - HNF

Last year mutations in MFN2 gene were shown to be present in 4.3% pf CMT patients in a recent study (http://onlinelibrary.wiley.com/doi/10.1002/mgg3.106/pdf). Mutations in the mitofusin 2 gene are well known to cause axonal Charcot-Marie-Tooth 2A (CMT2A). Autosomal dominant CMT6 patients have also been shown to have mutations in the mitofusin 2 (MFN2) gene.

MFN2 is involved in mitochondrial (the powerplant of the cell) fusion and is needed to maintain mitochondrial energy metabolism. A recent study by Mourier etc al., (http://www.ncbi.nlm.nih.gov/pubmed/25688136) shows loss of MFN2 results from depletion of the mitochondrial coenzyme Q pool. This can be partially rescued in their experiments by coenzyme Q10 supplementation, although their hypothesis has not as yet been tested clinically in humans. However a clinical trial using coenzyme Q10 in CMT patients has completed according to clinical trials.gov (https://clinicaltrials.gov/ct2/show/results/NCT00541164), the results have not been published.

The coenzyme Q10 analog called Idebenone (http://en.m.wikipedia.org/wiki/Idebenone) has been used for clinical trials with Duchene Muscular Dystrophy (DMD), Friedrich's Ataxia and Lebers Hereditary Optic Neuropathy yet to date it has not been approved by the FDA or European regulators. A recent clinical trial in DMD was published in the Lancet (http://www.ncbi.nlm.nih.gov/pubmed/25907158) and showed Idebenone reduced the loss of respiratory function. It would certainly be interesting to see whether idebenone could be useful in CMT2A and CMT6. For example animal models for CMT2A may be a useful starting point for assessment (e.g. Zebrafish, mice). In the interim perhaps other supplements containing coenzyme Q10 or similar molecules could be tested preclinically. It is important to note that drugs like coenzyme Q10 analogs may be problematic because of their similarity to vitamin K and potential competition with anticoagulants like warfarin. Therefore it is very important to seek medical advice before considering such supplements.

WHAT IT'S LIKE TO LIVE WITH CMT

Visit our website for your free copy of our latest Essential Guide, What It Is Like to Live with CMT. Based on the findings of the renowned Ph.D., Elizabeth Barrett and Dr. Carol Birdsall, the booklet provides a moving and informative account of the daily challenges and triumphs experienced by people with CMT.

GLOBAL REGISTRY FOR INHERITED NEUROPATHIES

Support Hereditary Neuropathy Research by joining the Global Registry for Inherited Neuropathies (GRIN)

Athena Diagnostic is Walking the Path with HNF to a Cure

By: Joy Andal Kaye, Board Chairman - HNF

If you are one of the millions affected by CMT, we know how overwhelming it can be living with a debilitating health condition. An important patient support function of HNF is to work closely with diagnostic companies to eliminate the lack of insurer reimbursement or high cost of testing as a major barrier to accurate diagnosis. In doing this, we hope to alleviate some of the fear, anxiety, and lack of control that CMT families experience when they don't have a definitive diagnosis of their disease.

HNF partner, Athena Diagnostics, is committed to making diagnostic testing more accessible and more affordable. On September 1, 2014, they launched the Athena Alliance Program, which was created to expand patient access to a variety of diagnostic methodologies and testing, especially those for rare and esoteric disorders. Athena's focus is on providing patient centric customer service so that each patient has an individual specialist and a team of dedicated personnel to support them from the time their test is ordered through the delivery and interpretation of test results.

The new tiered financial assistance program is based on income levels, with improved financial assistance available for families up to 600% of the Federal Poverty Level (FPL). This will drastically minimize costs to patients and families should their insurance companies decline coverage of these important tests!

www.athenadiagnostics.com/aap

The path to a cure starts with a differentiated diagnosis. By expanding access to critical testing, we can walk that path together.

HNF Physician Spotlight: Dr. Wayne Berberian
Dakota's Surgical Journey

By Kerin Reilly, Board Member - HNF

Dr. Wayne Berberian is a specialized orthopedic surgeon who repairs complications of the foot and ankle. He is among the rare physicians who have mastered both a caring patient-centered approach and a level of surgical skill that is so advanced; it can be viewed as true artistry.

My daughter, Dakota, has Charcot-Marie-Tooth Disease - CMT Type 1A. Since her diagnosis, we have worked tirelessly to find treatments that could provide her with the most comfortable, pain-free life possible. Last year, we began our search for a surgeon. CMT patients know how difficult it can be to find a surgeon with a stellar reputation and knowledge of CMT. Fortunately, we found Dr. Berberian who exceeded our expectations on every level.

Dakota, like many living with moderate to severe forms of CMT can be helped with surgery. Dakota's case required four surgeries. Each foot consisted of ten procedures broken down to two surgeries of five procedures each. The procedures Dr. Berberian performed were focused on correcting Dakota's forefoot deformity, cavus high arch, ankle laxity and tendon relocation.

In addition to twelve hospital affiliations, Dr. Berberian is one of a number of top physicians who own and operate Vanguard Surgical Center in Maywood, New Jersey. Vanguard offers top quality surgical care through an innovative team approach and is a model for hospitals and surgical centers to aspire to.

As our family approached Dakota's first surgery, we prepared for a difficult year. Her recovery has been challenging but the support from Dr. Berberian has made the last six months significantly easier. Selecting the right surgeon is critical. For us, that surgeon is Dr. Wayne Berberian. I have conveyed to Dakota in the moments when she is struggling with the painful recovery, that she is changing her path in life, and remind her how brave she is to endure this long process. Her outlook is positive and she cannot wait to begin college this fall with a brand new pair of feet! Dakota is taking charge of her CMT and Dr. Berberian has given her the ability to take the 'first step' into her future!

Q&A: Rachel and Laurel Levine

By Courtney Hollett, Fundraising Coordinator - HNF

Tell us about yourselves: Where do you live? What are your favorite interests?

Rachel: Currently, I live in Beverly, MA, north of Boston, but I grew up in Houma, Louisiana, just south of New Orleans. I work for Mass General Hospital in the Department of Dermatology as a Patient Service Coordinator. I used to be a Stage Manager in the performing arts for 10 years, but changed career paths a couple of years ago. I wanted a career less demanding of my time as I discovered there is more to life than just working. I love to read romance novels, swim, go to the beach, learn new things, and I'm very crafty. I always have a new craft project to fill my time.

Laurel: I live in Wakefield, MA, just north of Boston. I moved to Massachusetts for a job offer, which I received after I finished Grad School. They took a chance on this girl from Houma, Louisiana, and I think that they haven't regretted it, as I am still here over 7 years later. I currently work for an IT Solutions and Staffing agency, as a Delivery Specialist (Account Manager). I specialize in making placements for accounts that run special staffing programs. I have to admit that I really like my job - it meets all of my physical needs as well as my personal goals for success. Some of my interest include being outdoors, reading books and listening to music. The thing I like most though is traveling as well as spending time with my family.

When you both were diagnosed with CMT? What type do you have?

Rachel: I was unofficially diagnosed with CMT when I was 20, but was officially diagnosed last year, 2014. I have CMT 1A.

Laurel: That's a complicated question. The simple answer is that we have CMT 1A. But, we had a 25 year journey that involved several stages of self-loathing and discovery, that then lead to my genetic testing where my neurologist defined my disease as CMT 1A. I was 25.

Do other members of you family have CMT?

Rachel: Yes, my paternal grandmother (Rita Levine), father (Jerry Levine), and also his brother, my uncle (Norman Levine). I'm not aware if there is anyone else in our family who has been diagnosed with CMT.

Laurel: Our late father had CMT, and so did his mother. Our father's only brother also has CMT.

What are the challenges you face with CMT?

Rachel: I am very lucky to have a mild case in comparison to many others, but it does still come with its struggles. In general, my muscles are very weak and weakening every year. The joints in my hands and feet are fused, so this makes mobility more of a challenge. The muscles at the bottom of my feet have depleted, causing a high arch, weak ankles, and difficulty walking at times. Walking barefoot is a challenge anywhere except my own home. Good, expensive shoes are a must! My muscles fatigue faster than others, so when I exercise, I have to be careful not to overdo it, or else I will really pay for it for a few days. Given the weak muscles in my hands, it makes extending my fingers for long periods of times difficult. Writing for long periods of time is also troublesome. My job requires me to be on the computer all day, so cramping in my hands is a given. When I climb stairs, I have to force myself to use my left leg, as it is weaker than my right leg. If I don't pay attention where I'm going, I will trip and that's not fun for anyone involved.

Laurel: I find that my twin and I have mainly, the same symptoms but differ in severity, and pronouncement. I have numbness in my feet and hands, although that is more how the doctors describe it. I say it is more like, opposite phantom limb syndrome. I obviously know they are there, but my awareness of them has altered since my preteens. It's as if I can feel everything and nothing at the same time. I have deformities in my feet and slightly in my hands, although not enough to impede primary function. My feet have severe high arches, so no more high heels for this 30-year old. My toes have minimal mobility as well, and my feet hurt pretty much most of the time. I also have these electric shock feelings on random points in my body at least a few times a week. I have restless legs at night, and my muscles in my legs are very tight and weak. Same with my arms as well. And, in general, I have weak muscles and am very easy to fatigue. I have difficulty walking up stairs, and a high gate to my walk. I also have tight neck and shoulder muscles, which cause a lot of stress aches. I have foot drop as well, which has caused more than a few forward facing tumbles. I have a hard time getting up off the floor as well, so falling kind of sucks! HINT: I must always look for an escape route

off the ground. My joints and tendons are sore and in pain at times. I also grind my teeth at night, and wake up with severe cramping in my legs from time to time. I also have cold hands and feet and bad circulation throughout my body.

How has CMT changed your life?

Rachel: I wouldn't say CMT has changed my life as it has always been a part of me. My life has continued on regardless of CMT. You learn to adapt and continue living. Knowing about the disease, however, makes you feel less alone in the world. It also validates all the issues you've had physically and you now know that you are not "normal." You still try to lead as normal a life as possible, but you just have to sometimes be more creative. CMT makes you aware of your limitations, but you also can't limit yourself from life experiences. You count your blessings and you can't feel lacking because you can't climb the highest mountain. Instead, you climb a smaller mountain that is more achievable for you.

Laurel: Well, to be fair, I have been living with CMT my whole life, so I really have no basis for comparison as to whether something is more challenging than what others face. However, it is pretty clear that having CMT has forced me to structure my life in ways that most people would never think about. Exercising is difficult, but necessary. I try to swim and do elliptical when I can. I work in a sedentary job sitting at a computer all day. I don't really have the luxury of living spontaneously. I truly think about my future and what I need to do to maintain all the time. I felt this intense need to keep quiet about my CMT for several years, because of fears of public scrutiny. However, talking about this disease and how it affects me has been in some ways therapeutic, and allowed me to find some happiness in my uncertain future.

How do you lean on each other for support?

Rachel: As an identical twin, you automatically have a relationship with another human being that cannot compare to others. The easiest way I can explain this relationship is that I breathe better when I'm with my twin. The cliché is true when I say "she completes' me." Given this relationship, I've never had to worry about having someone to help me through the tough times. We are not ones to talk or complain about our health issues. She already knows what I'm going through, so talking has never been a major help to offer support. We help each other with the physical challenges when needed. Most importantly, we laugh as much as possible. Happiness is how we offer support for each other.

Laurel: Having my twin to go through this life with has been the greatest blessing. We don't of course enjoy watching each other go through difficult things; however, what doesn't kill us makes us stronger. We sometimes exercise together, and remind each other to stretch and to not overdo it. Having this disease has brought us closer in ways that I don't think we naturally would have without it. Easy times are not what really defines a relationship, it's hard times that do that. Could we do it alone? The answer is yes. But we don't have to, thank God! I truly believe that things happen for a reason, and I was meant to enter this world with my twin.

What advice can you give to others that are affected with CMT?

Rachel: Don't let it get you down. You still have a lot of life to live and count your blessings. Push yourself to your limits. Trust that you will adapt. Don't allow CMT to be the excuse for why you didn't go for your dreams.

Laurel: Well, I truly try to live every day like it's the last, so I would suggest letting go of the small things, and just LIVE. Get out there, find solutions on how you can do something and just get it done. I know that CMT makes things seem harder to achieve, but really, there is always a way, just use your imagination. As for what may be the most difficult piece of advice, find a CMT buddy. I know that may be challenging, but put yourself out there. If you have someone to go through CMT with, whether it's a family member, or a fellow CMT patient, it can make many of the mountains seem like mole hills, and truly improve your outlook on life.

What's a favorite quote of that you think of often?

Rachel: There are many famous quotes from Walt Disney that I have always felt describe my outlook on life. I always feel he is talking to me when I read his quotes:

"All our dreams can come true, if we have the courage to pursue them."

Laurel: I know that this may sound slightly morbid, but ever since I heard this quote, it makes me think so much about my relationship with my twin and how grateful I am to have her.

"If ever there is tomorrow when we're not together… there is something you must always remember. You are braver than you believe, stronger than you seem, and smarter than you think. But the most important thing is, even if we're apart… I'll always be with you."

Do you want your story featured in the CMT Update? Please email courtney@hnf-cure.org

Introducting HNF's Health Care Providers Directory

By Tina Tockarshewsky, HCP Project Director - HNF

The Hereditary Neuropathy Foundation (HNF) has a new initiative aimed at identifying expert Health Care Providers (HCP) for the Charcot-Marie-Tooth (CMT) community. We are creating an innovative online HCP Directory that will enable people with CMT and their family members to find clinicians familiar with treating CMT in their local communities. The Directory will include a range of health care professionals who treat the whole person with CMT— neurologists, physical therapists, podiatrists, and more. We are looking forward to seeing this directory grow and expand over time as the community has a chance to learn about it and interact with it.

Clinicians who join the Directory will have access to additional tools to help care for their patients. The Directory will link them to standard of care guidelines, leading research, and cutting edge information regarding the CMT community. As HNF builds out its CMT health care professional education and accreditation coursework, in the future health care providers who have completed these courses will be identified with a special logo on the directory to help patients find providers with this additional accreditation. In addition to linking clinicians and the CMT patient community with one another, we will be asking our HCP Directory professionals to encourage their patients to join Global Registry for Inherited Neuropathies (GRIN) so they can collaborate in the research process by accelerating therapy development for the CMT community.

To launch this exciting initiative, this past spring HNF participated in the Neurological Rare Disease Special Report that was published as a supplement to Neurology Reviews and is now available as a digital version under the "Education" tab on our website. HNF developed a two-paged advertorial describing the diagnosis and treatment for CMT (p. 8-9). The Neurological Rare Disease Special Report was mailed to over 24,000 clinicians, including 20,000 neurologists and 4,000 primary care physicians in the U.S. In addition, this special report will also be distributed from the Neurology Reviews exhibit booth at over 15 major industry conferences.

One such conference where we recently shared news of the Directory was the recent biennial meeting of the Peripheral Nerve Society in Quebec, Canada, which was attended by investigators and clinicians from around the world. HNF and the Directory initiative were extremely well received, with professionals from a range of countries inquiring about participation and expressing their eagerness to share news of the initiative with their patients.

Our goal is to continue to raise disease awareness, inform clinicians of how to recognize CMT and accurately diagnose it using new and improving diagnostic tools, and finally inform them of potential future treatment options currently under investigation. We will continue to highlight CMT through similar mechanisms and peer reviewed scientific publications.

For Medical Professionals:
http://www.hnf-cure.org/cmtprovider/

For Patients:
http://www.hnf-cure.org/provider-directory/

Update on Michael Watkins – Team CMT Member

By Courtney Hollett, Fundraising Coordinator - HNF

Michael Watkins completed the Leadville Trail 100 MTB race on August 15, 2015 in Leadville, CO. The trail race is widely considered one of the toughest mountain biking races in the country. Michael has been logging 200 - 225 miles on the bike each week to prepare for the big day.

Michael has a wonderful support system with his family and friends. He received a surprise from his brothers in early July to accompany him on the most difficult portions of the trail to test if he was physically able to take on the course and how his body would react and cope with the altitude. In late June, he was fitted for two custom-made low AFO's that he wore during the race to help him with the steeper rocky sections that he will most likely have to walk. These are being made at no cost to him courtesy of generous friend of his family who is a PT/Clinical Electrophysiologist.

Throughout training, Michael stayed motivated by thinking "sometimes we just have to work a little harder than most to get what we want and the harder you work at something the greater the feeling you have when it's over". That helped him throughout his training runs and took him to the finish line. Congratulations on your tremendous accomplishment!

Click here to support Michael!

Chrome Diva's First Annual Motorcycle Scavenger Hunt

By Courtney Hollett, Fundraising Coordinator - HNF

On Saturday July 25, 2015, Debi and Tom Houliares hosted the first annual Chrome Diva's Motorcycle Scavenger Hunt in Webster, New York to raise awareness and funds for CMT Type 6. The Houliares' son Zachary has CMT Type 6 and the Houliares are working tirelessly to find a cure for their son. The flawless event was put on with the help of the Rochester Chome Diva's. Riders cruised the streets and were on the hunt for items to complete a scavenger hunt. The top three teams were awarded cash prizes. After a day of riding, guests enjoyed a BBQ along with live music from local bands. Guests took a chance on raffle baskets, a silent auction and two roundtrip tickets on Southwest Airlines anywhere in the Continental United States. Thank you to all our riders and dinner guests who came out to support Zach's Team - we are happy to report they raised over $14,000!

To learn more about Zach's Team visit http://www.hnf-cure.org/zachsteam/

10th Annual HNF Golf Outing: Dick Sharpe Memorial Event

By Courtney Hollett, Fundraising Coordinator - HNF

On Thursday, September 17, 2015, golfers and dinner guests will come together to not only commemorate 10 years of success in raising funds for Charcot-Marie-Tooth (CMT) but also celebrate the life and work of our dear friend, Dick Sharpe. In addition, we are excited about our new venue, Plandome Country Club, in Manhasset, New York. Although it's hard to believe 10 years has passed, HNF is thriving on the path for treatments and one day a cure for CMT. All of this is possible from the consistent support of golfers and guests that have come out year after year to support the cause.

Please join us for golf as we highlight the past 10 years! Not a golfer? Join us for dinner and take a chance on the plentiful raffle and silent auction items.

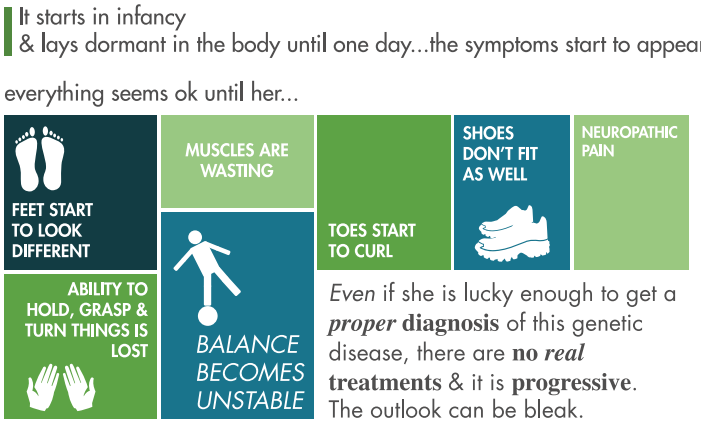
Register at: http://weblink.donorperfect.com/2015GOLF

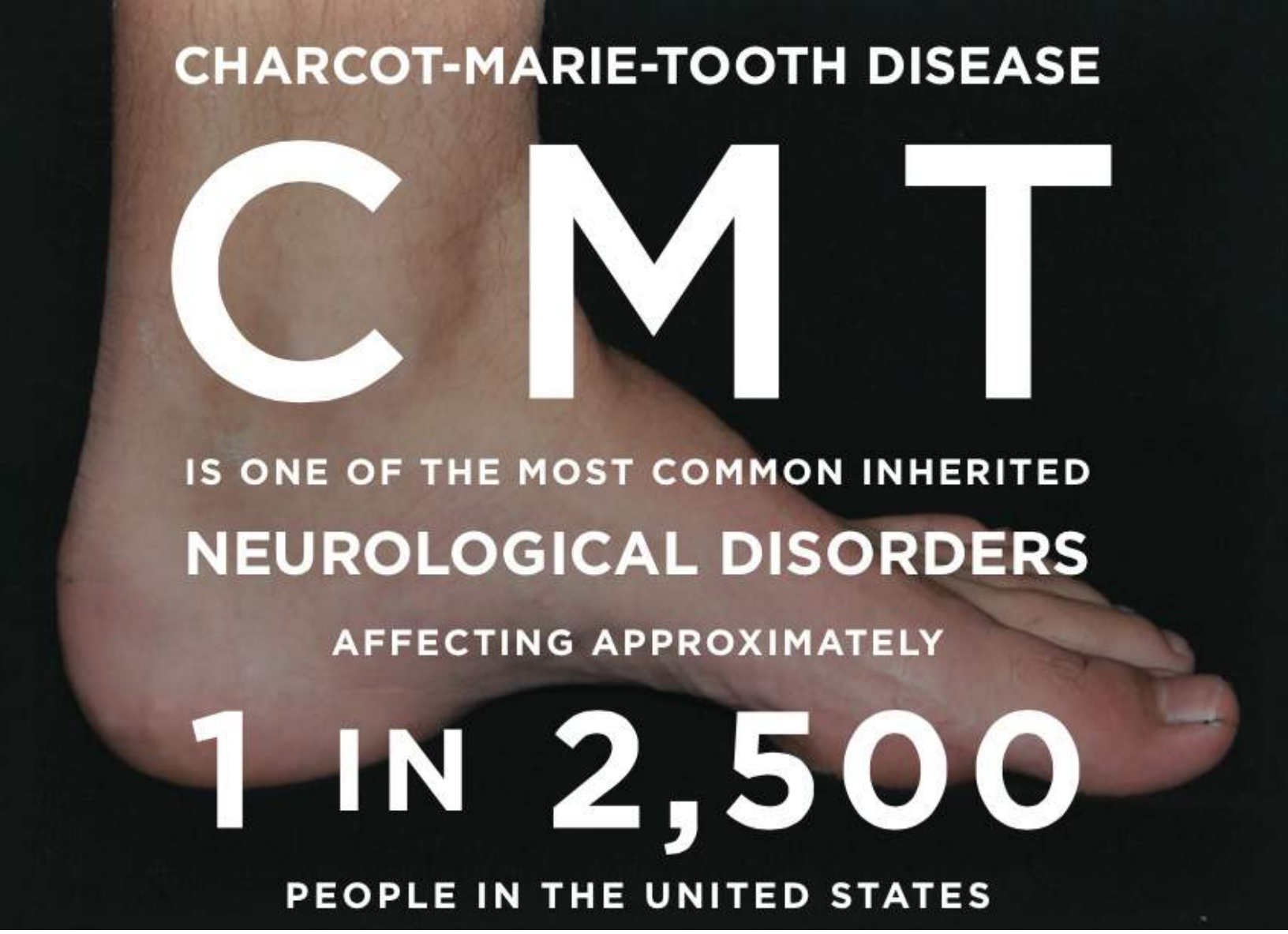
Upcoming Events – Save the Date

Dick Sharpe Memorial Golf Outing
Thursday, September 17, 2015
Plandome Country Club
Manhasset, New York

Champions for Charity
Thursday, December 3 - Sunday, December 6, 2015
Manhasset, New York

H.E.L.P. Fund Brunch, Boutique & Card Party
Friday, January 29, 2016
Broken Sound Country Club
Boca Raton, Florida





Why does it take the average patient 2 years to receive a diagnosis?

Through educational resources and genetic testing, the HNF helps physicians identify, diagnose and treat CMT patients.

Join our provider directory and help us spread awareness and get access to treatment options for CMT patients.

HEREDITARY NEUROPATHY FOUNDATION

Find out more at hnf-cure.org/cmtprovider