SUMMER 2025

THE CMTA REPORT

Charcot-Marie-Tooth Association

cmtausa.org

Celebrating 10 Years of Camp Footprint!

A LONG DISTANCE CYCLIST WITH CMT SHARES HIS TRIP:
VIEW *JEFFREY'S JOURNEY* AT THE 2025 PATIENT & RESEARCH SUMMIT IN SEPTEMBER

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SUMMER 2025

THE CMTA REPORT | SUMMER 2025

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Email CMTA at info@cmtausa.org

FROM THE DESK OF SUE BRUHN, CMTA CEO

COMMUNITY IS EVERYTHING

AT CMTA, it is already shaping up to be a year of incredible progress for our community, and I could not be more inspired by what lies ahead. You make every step forward possible in our mission to accelerate research, improve quality of life, and find a cure for CMT.

At the CMTA, we've started saying something simple that carries a lot of meaning: Community is everything. You may have already seen these words in our communications. You'll see them throughout this issue of The CMTA Report. And you'll see exactly what we mean when we say them.

This issue reflects what happens when people come together with purpose. You'll read about families driving progress through participation in research. You'll meet kids and teens who have found a second home at Camp Footprint. You'll hear from donors who have chosen to support others because they remember what it felt like to need support themselves. And you'll see how researchers, clinicians, parents, and young adults are all part of the same story, a story that is still being written.

Together, we are building something lasting. We are expanding programs like the Patient Support Fund and launching new ones like CMTA's The Roots, designed for parents of young children with CMT. We are funding studies that bring us closer to treatments. We are creating space for every person with CMT to be seen, supported, and connected.

This fall, we will gather in Indianapolis for the 2025 CMTA Patient & Research Summit. There will be science. There will be stories. There will be conversations that matter. But most of all, there will be connection. That connection is what makes everything else possible.

You will see it again and again in these pages. The most powerful force we have is each other. Every step forward, every discovery, every trial, every moment of courage starts with a person or a family who says, "I want to help."

Thank you for being part of this work. Thank you for being part of this community. And thank you for helping us move toward a world without CMT.

Because at CMTA, *Community Is Everything*.

With deep appreciation,

Suzanne L Bruhn

Sue Bruhn, PhD
Chief Executive Officer, Charcot-Marie-Tooth Association

COMMUNITY IN ACTION: CHANGING LIVES

Because no one should face CMT alone.

At CMTA, we believe in science, strategy, and systems. But we also believe in something even more powerful: each other.

When someone in our community is struggling to access care, when the cost of braces or surgery stands between a child and their future, the CMTA community shows up. Through two donor-powered funds: the **Patient Support Fund** and the **emBrace the Future** Fund, we are turning compassion into action, proving, again and again, that community is everything.

ILONA'S STORY

The First Step Toward Mobility Independence

Ilona is a ten-year-old living with CMT. And for the past three years, she's lived with constant pain that even her AFOs couldn't relieve. Her right foot had become so unstable that walking hurt. Her family knew she needed surgery, not just to ease her pain, but to give her back the freedom that CMT was slowly taking away.

They searched for help from Florida to Boston to California. That journey led them to Glenn Pfeffer, MD, one of the world's leading orthopedic surgeons for people with CMT. But like so many families, they hit a wall. The cost of travel, lodging, and recovery made the trip feel impossible. That's where the CMTA Patient Support Fund came in.

Created by a community donor who wanted to remove the financial barriers standing in the way of surgical care, the fund helped cover Ilona's travel and made the surgery possible.

"We're just regular people trying to give our daughter the best chance," Heather wrote. "And because of this fund, she has one."

Ilona became the very first recipient of the CMTA Patient Support Fund. The fund is specifically designed to support families who need help covering travel costs related to corrective foot and ankle surgery for CMT performed by Dr. Pfeffer at Cedars-Sinai Medical Center.

For many in the CMT community, access to this kind of surgical care offers real solutions to CMT's impact and a path toward mobility independence. But for families who live far away and may not have access to surgical expertise in CMT, getting there can be the hardest part. This fund exists to change that.


Ilona post-surgery!

It is not about being first. It is about making sure every family has the opportunity to take the next step toward expert care, greater mobility, and a better quality of life.

TOM'S STORY

A Future Reimagined

Tom duPont knows what CMT takes. He has lived with the disease for years. He also watched it take a devastating toll on his family, including the loss of both his sister and brother, whose lives were shaped and shortened by the challenges of progressive mobility loss.

Tom never forgot what they went through. He also never stopped thinking about the people still facing those same struggles.

In their memory, Tom created the **emBrace the Future Fund**—a CMTA support fund dedicated to helping people with CMT overcome mobility challenges through access to bracing and other assistive devices. His hope is simple and profound: to restore independence, preserve dignity, and offer strength to those living with the everyday realities of CMT.

"It's my hope that this fund will not only provide mobility but also foster strength and hope in those facing the challenges of this disease."

> "My sister and brother inspired this fund, and their memory drives me to help others living with CMT."
>
> *—Tom duPont*

Thanks to Tom's vision and generosity, the fund is already changing lives. This year, its first recipient, a five-year-old newly diagnosed with CMT, received AFOs that made it easier to move, to play, and to feel confident in their own body.

This is what legacy looks like. Not just remembering but reaching forward to make someone else's journey a little easier. **Learn more at cmtausa.org/embracefund**

continued on page 4

HELP US LIFT SOMEONE UP TODAY...

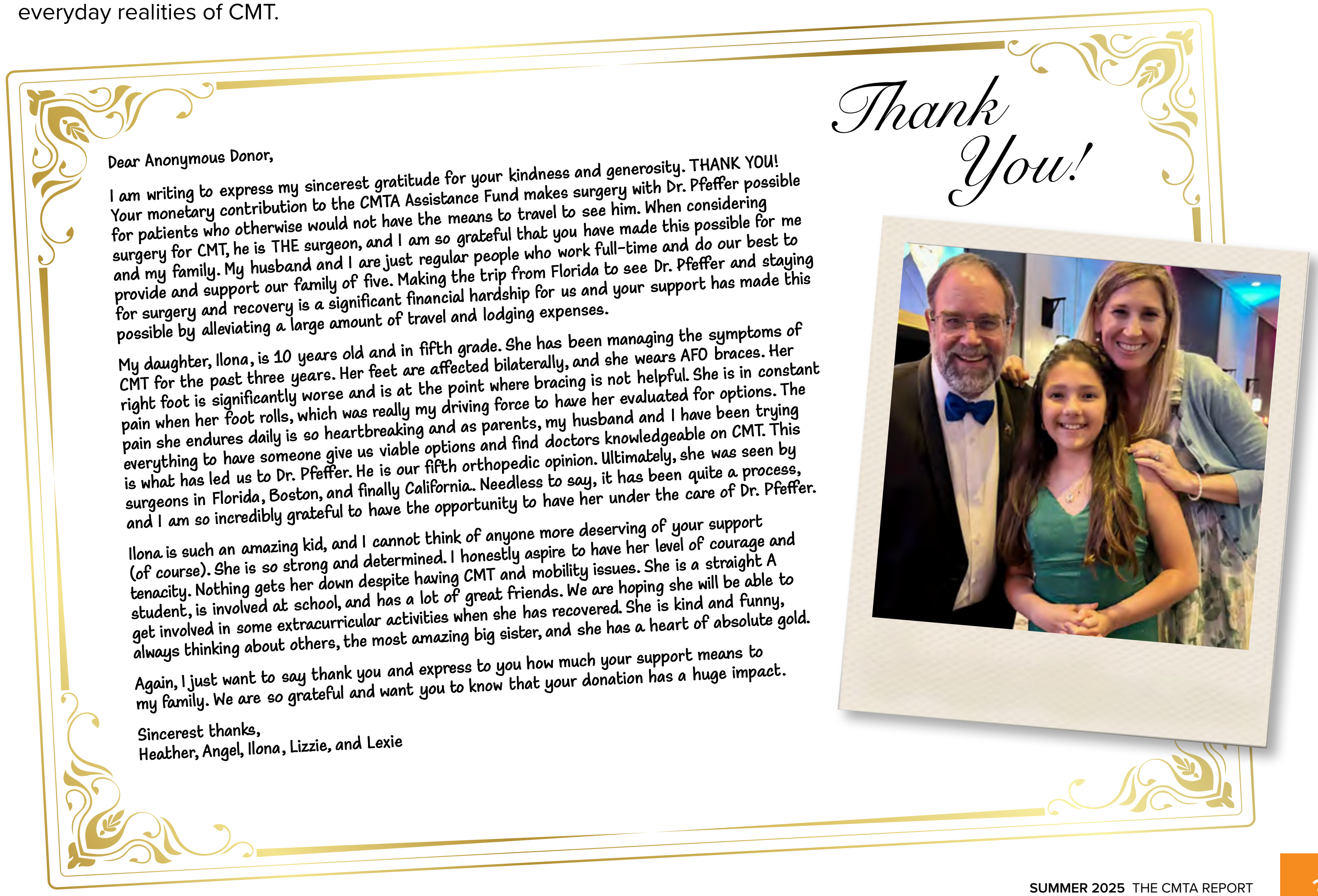
The CMTA Patient Support Fund and emBrace the Future Fund exist because members of this community saw a need and chose to act. They imagined a world where no one has to delay surgery or go without braces because of cost, and they helped make that world possible, one person at a time.

Today, you can be part of that story.

Your support helps someone travel for life-changing care. It helps a child walk safely with the right equipment. It reminds every person with CMT that they are not alone. Donate today at **cmtausa.org/supportpatients**

Together, we're proving that community is everything.

Every gift for these funds goes directly to CMT families. No overhead. Just help, where and when it's needed most.



Thank You!

Dear Anonymous Donor,

I am writing to express my sincerest gratitude for your kindness and generosity. THANK YOU! Your monetary contribution to the CMTA Assistance Fund makes surgery with Dr. Pfeffer possible for patients who otherwise would not have the means to travel to see him. When considering surgery for CMT, he is THE surgeon, and I am so grateful that you have made this possible for me and my family. My husband and I are just regular people who work full-time and do our best to provide and support our family of five. Making the trip from Florida to see Dr. Pfeffer and staying for surgery and recovery is a significant financial hardship for us and your support has made this possible by alleviating a large amount of travel and lodging expenses.

My daughter, Ilona, is 10 years old and in fifth grade. She has been managing the symptoms of CMT for the past three years. Her feet are affected bilaterally, and she wears AFO braces. Her right foot is significantly worse and is at the point where bracing is not helpful. She is in constant pain when her foot rolls, which was really my driving force to have her evaluated for options. The pain she endures daily is so heartbreaking and as parents, my husband and I have been trying everything to have someone give us viable options and find doctors knowledgeable on CMT. This is what has led us to Dr. Pfeffer. He is our fifth orthopedic opinion. Ultimately, she was seen by surgeons in Florida, Boston, and finally California. Needless to say, it has been quite a process, and I am so incredibly grateful to have the opportunity to have her under the care of Dr. Pfeffer.

Ilona is such an amazing kid, and I cannot think of anyone more deserving of your support (of course). She is so strong and determined. I honestly aspire to have her level of courage and tenacity. Nothing gets her down despite having CMT and mobility issues. She is a straight A student, is involved at school, and has a lot of great friends. We are hoping she will be able to get involved in some extracurricular activities when she has recovered. She is kind and funny, always thinking about others, the most amazing big sister, and she has a heart of absolute gold.

Again, I just want to say thank you and express to you how much your support means to my family. We are so grateful and want you to know that your donation has a huge impact.

Sincerest thanks,
Heather, Angel, Ilona, Lizzie, and Lexie

CHANGING LIVES

continued from page 3

FUELING THE MISSION

Coldiron Derby Raises Over $80K for CMT Research

The Coldiron Derby fundraiser, hosted each year in Nashville by CMTA Board Member David Coldiron and his wife, Christina, is another reminder of what happens when community meets action.

In May 2025, their annual Coldiron Derby event raised an incredible $80,667, with 168 guests coming together under a festive tent in Nashville's Sylvan Park neighborhood to support CMTA's mission.

Inspired by their daughter Hazel, who lives with CMT, the Coldirons started this event to bring awareness, raise funds, and build hope. From derby hats to heartfelt toasts, the day was more than a party. It was a declaration that CMT families are not alone and that together, we can change the future.

Top left: **The Coldiron Family—Christina, Clara, Hazel and David.** *Top right:* **David Coldiron with Michael Sheridan and Sheldon Helm.** *Middle right:* **Clara, Christina and Hazel Coldiron.** *Bottom right:* **Vishnu Jayamohan, Isabelle Tate and Katerina Tate.** *Bottom left:* **Kelly Terrell.**

CONNECTING THE DOTS

How Distant Cousins Are Driving CMT Gene Discovery

When genetic testing doesn't find an answer, many families are left wondering what comes next. That's where the Distant Cousin Project comes in. Funded by a CMTA community donor, this research initiative is helping scientists discover new CMT genes by analyzing the DNA of people with CMT who are distantly related, often third or fourth cousins. By comparing these more genetically distinct relatives, researchers can pinpoint potential mutated genes with greater accuracy. The project has already led to the discovery of two new CMT subtypes: ITPR3 (CMT1J) and COQ7 (CMT-COQ7).

For Howard Landis, this project isn't just about his own test results. It's about contributing to something bigger. As someone living with an unidentified form of CMT, he's drawn to the Distant Cousin Project's potential to uncover answers that conventional genetic testing thus far hasn't yet revealed.

"None of us are going to find the missing genes alone," Howard says. "But if we can connect the dots, we can help the scientists do what they do best."

The Distant Cousin Project is a powerful reminder that research doesn't begin in a lab. It begins in living rooms, in family trees, in conversations between relatives. It begins with a community willing to act, to share their stories, and to fuel the next big breakthrough.

See page 25 to learn how to participate in the Distant Cousin Project.

POWERED BY THE COMMUNITY

Whether it's accelerating research, funding travel for life-changing foot & ankle surgery, or connecting the genetic dots, the CMT community is not just hoping for progress; you're driving it.

Not just strategy. Not just science. Community.
And the power to act.

ANNOUNCING EIGHT NEW CMTA CENTERS OF EXCELLENCE

CMTA is proud to welcome nine new Centers of Excellence to our growing international network. Each of these clinics brings expert, multidisciplinary care to individuals and families living with CMT, helping us move closer to a world where everyone has access to the care they deserve.
New additions include:

- Laurie Gutmann, MD, Indiana University School of Medicine—*Indianapolis, IN*
- Hani Kushlaf, MD, University of Cincinnati Gardner Neuroscience Institute—*Cincinnati, OH*
- Joshua Smith, DO, University of Pittsburgh Medical Center (UPMC)—*Pittsburgh, PA*
- Amy Harper, MD, Children's Hospital of Richmond—*Richmond, VA*
- Lisa Williams, MD, University of California Davis (UC Davis)—*Sacramento, CA*
- Sarah Breevoort, MD, PhD, Medical University of South Carolina (MUSC)—*Charleston, SC*
- Kathryn Brennan, MD, NHS Greater Glasgow and Clyde—*Glasgow, Scotland*
- Hernan Gonorazky, MD, The Hospital for Sick Children—*Toronto, Canada (INC Site)*

CMTA Centers of Excellence are clinics led by physicians recognized by the CMTA Clinical Expert Board through a rigorous review process. While CMTA does not operate or accredit clinics directly, we award this designation to specialists who demonstrate deep clinical expertise, outstanding patient care, and a strong commitment to the CMT community. Each clinic brings together a care team that may include neurologists, genetic counselors, orthopedic surgeons, and physical therapists, all with experience treating CMT. See page nn to find your Center of Excellence

Together, we're building a stronger and more connected care network, because at CMTA, community is everything.

CELEBRATING TEN YEARS
2025
CMTA
CAMP FOOTPRINT
ONE STEP AT A TIME

CELEBRATING 10 YEARS OF CAMP FOOTPRINT:

A DECADE OF BELONGING, BECOMING, AND THE TRIBE OF THE FUNKY FEET

A HUMBLE BEGINNING

Ten years ago, Camp Footprint began with a simple, radical belief: kids with Charcot-Marie-Tooth disease deserve to feel what every kid should feel at summer camp. Safe. Seen. Celebrated. Free to be themselves without needing to explain.

Since that first summer, more than a camp has grown. A community has taken root. A movement has found its rhythm. Camp Footprint has become a home for hundreds of young people across the country and around the world who, for one week each year, do not have to walk alone.

A VISION REALIZED

Camp Footprint is the vision of Jeana Sweeney, CMTA's Chief Engagement Officer, who believed in her bones that kids with CMT needed more than medical care. They needed connection. They needed joy. They needed a place where they could just be kids.

From day one, Jeana poured everything into making that vision real. She built Camp Footprint from the ground up, shaping every detail with care, passion, and purpose. She knew this had to be more than a camp. It had to be a movement.

And then came Jonah Berger.

A lifelong youth mentor and the son of a legendary camp director, Marty Berger, Jonah brought his heart, humor, and lived experience with CMT to the Camp Footprint story. After meeting Jeana at a CMTA Patient and Family conference in Washington, DC, he knew he had found something special. He joined the mission and eventually stepped into the role of Camp Director—a role he calls the most meaningful of his life.

"I've directed eight camps," Jonah said. "I've never been part of something as special as this. Camp Footprint changes how kids see their disease, their lives, and themselves in the world."

Jonah tells the story of one camper who arrived with his hoodie pulled tight over his head, eyes on the floor, barely speaking. By the end of the week, that same camper stood in front of more than 150 people and said, "Thank you all. This was the best week I've ever had."

For the past decade, Jeana and Jonah have grown Camp Footprint into a movement empowering CMT youth and the future of our community.

Together, Jeana and Jonah helped build something no child with CMT had before. What started as a camp at a single site with 36 Campers in the first year have grown to 2 full-week Camps: Camp West in Oregon in June, and Camp East in Pennsylvania in August, with total attendance of about 160 campers supported by about 160 volunteer counselors and staff.

Camp Footprint is the only sleepaway camp in the United States created specifically for kids with CMT, and it is completely free. Every activity, from rock climbing to adaptive archery, is designed so that no camper is left out.

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CELEBRATING 10 YEARS OF CAMP FOOTPRINT

continued from page 7

If a child needs a one-to-one counselor, they have one. If they need help, they get it. If they need to just belong, they already do. Inclusion isn't just part of Camp. It is the reason Camp exists.

THE CAMP FOOTPRINT FEELING

Ask anyone who has been to Camp Footprint, and they'll tell you. It is not just the dance parties or the slip-n-slide or the zip line that make it unforgettable. It's the feeling. It's the moment a camper realizes they are not the only one. It's the first time someone says, "I get it," and means it. It's the freedom to try something new without fear of judgment. It's the space to fall and be lifted up. It's the space to just be.

"It was the most empowering, inspiring week of my life," said camper Paola. "For the first time, I was in an environment where having CMT was not only normal but was celebrated."

FROM CAMPERS TO LEADERS

Over the last decade, Camp Footprint has done more than create memories. It has shaped identities. Many of today's counselors were once campers themselves. They return not just to give back, but to carry forward the power of what they received. Confidence. Belonging. Pride. Some veteran counselors, like Dr. Katherine Forsey, Laurel Richardson, Riley Williams, Cassidy Miller, and Sarah Gentry, have even become CMTA staff.

"I used to feel so alone," said counselor Maddie. "But now, every step I take carries the strength of this community. I never walk anywhere alone anymore."

> This camp gave my daughter the gift of acceptance, and more importantly, self-acceptance. Picking her up after camp, she said 'Mom, this was the best week of my life. I'm sorry, but it even beat Disneyland.'

FAMILIES FEEL IT TOO

Parents feel it too. One parent wrote, "This camp gave my daughter the gift of acceptance, and more importantly, self-acceptance. Picking her up after camp, she said 'Mom, this was the best week of my life. I'm sorry, but it even beat Disneyland.'"

Every part of Camp Footprint is made possible by people who believe in the power of togetherness. From the volunteers who give their time, to the parents who take a leap of faith, to the counselors who provide campers with one-on-one care when needed, this has always been a community-driven story.

POWERED BY COMMUNITY

Camp Footprint would not exist without the generosity of others. We are deeply grateful to our national and local business sponsors, our biotechnology partners, and especially our CMTA community donors who make it all possible. Every year, your support turns a dream into a real, accessible reality for kids who need it most. You make the travel happen. You make the adaptive gear possible. You make the camp moments real. You make the magic.

A PROMISE FOR THE FUTURE

And after ten years, the story is still being written.

Camp Footprint is not just a program. It is a promise. A promise that no child with CMT will grow up thinking they are the only one. A promise that joy, identity, and connection are not extras, but essentials. A promise that this community will keep showing up, year after year, to make space for kids to be exactly who they are. It is also where the Youth Movement began. Where the Youth Council was born. Where COMPASS first took shape.

Because when you grow up at Camp Footprint, you do not just find your people. You find your voice. And once you are part of the Tribe of the Funky Feet, you carry it with you wherever you go.

Thanks to the generosity of our donors and the incredible dedication of our volunteers, Camp remains free to every camper, ensuring that cost is never a barrier to connection, and belonging.

SUMMER 2025 CAMP FOOTPRINT WEST

A PLACE WHERE THEY BELONG:

WHY SUSAN SAVAGE CHOSE CAMP FOOTPRINT AS HER LEGACY

At 73, Susan Savage has lived through decades of CMT in a world that rarely made space for her. Bullied, misdiagnosed, and once told she wouldn't live past 21, she spent her childhood navigating misunderstanding and exclusion. Nowhere was that more painful than at camp.

"I thought I was doing OK," Susan said. "I had friends. I was walking. But camp changed that. I was forced to do things I physically couldn't do, and when I said I couldn't, they didn't believe me. There was no understanding. Just shame."

Those experiences left lasting emotional scars. Camp wasn't a safe place. It was a place of ridicule, physical harm, and erasure.

When Susan learned about Camp Footprint, something shifted. For the first time, she saw a place built for kids like her. A place where "can't" is met with compassion. A place where kids with CMT are seen, supported, and celebrated. A place where they do not have to explain themselves because everyone already understands.

She called it a "wonderland."

"It's not just about games and activities," she said. "It's psychological. It's healing. These kids get to grow up believing in themselves."

Camp Footprint is now celebrating its tenth year. For a decade, it has given children with CMT a place to connect, grow, and just be themselves. For Susan, it represents everything childhood should be. And everything hers was not.

Though she never had children of her own, Susan often speaks of protecting "our kids"—the next generation growing up with CMT. Her decision to support Camp Footprint through her estate plans is rooted in love, shaped by pain, and grounded in a clear vision of what today's youth deserve.

"This isn't about charity," Susan said. "It's about clarity. I know what it means to need something like Camp Footprint. I want these kids to feel what I didn't."

For Susan, the choice was also practical. Camp Footprint is made possible by a network of volunteers, staff, and donors who believe in the power of community. It takes all of us to keep the doors open and the experience accessible to every child.

Thanks to Susan's commitment, Camp Footprint will continue to grow and welcome each camper into a space where they are not alone. Her legacy is more than a gift. It is a promise that every child with CMT deserves to feel safe, included, and proud of who they are.

Want to leave your legacy like Susan did?

Learn how you can support the future of CMTA through your estate plans: cmtausa.org/legacy

CMTA's Community Program for Parents of Young Children with CMT

CMTA is proud to launch **The Roots**, a new initiative created for parents and caregivers of young children with CMT, from birth through 9 years old. It's a space to connect with others who understand the unique challenges and emotions that come with parenting a young child with CMT.

Whether you're navigating early signs, adjusting to a new diagnosis, or simply looking for reassurance and shared experience, **The Roots** offers a supportive peer-to-peer environment grounded in community and understanding.

To stay connected, parents are also invited to join our **CMTA Parents' Facebook group**, where families come together to share stories, ask questions, and support one another through every stage of the journey.

The Roots reflects CMTA's commitment to serving every part of the CMT community, starting with the youngest members and the people who care for them. Because at CMTA, community is everything.

Learn more and connect with other parents at cmtausa.org/the-roots

KNOW THAT YOUR DONATIONS ARE BEING USED WISELY.

Charity Navigator awarded us its highest 4-star rating and a Perfect 100 score based on our exceeding industry standards and outperforming other organizations that do similar work. It's a distinction enjoyed by less than 1 percent of the nonprofits rated by Charity Navigator.

TOGETHER, WE CURE CMT: YOUR GIFT DRIVES RESEARCH FORWARD

○ $50 ○ $100 ○ $250 ○ $500 ○ $1000 ○ Other: $__________

○ Please charge my credit card. ○ Visa ○ MasterCard ○ American Express ○ Make my gift a recurring Monthly Donation.

○ Check enclosed, payable to the Charcot-Marie-Tooth Association.

○ I am interested in learning more on how to make a major gift or a multi-year pledge. ○ I would like information on donating securities.

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Your donation is tax-deductible to the fullest extent allowed by law. Tax ID: 22-2480896.
Mail this form to: CMTA, PO Box 105, Glenolden, PA 19036

DONATE ONLINE AT: cmtausa.org/donate

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CMTA Charcot-Marie-Tooth Association | 2025 Camp Footprint – Celebrating Ten Years – One Step at a Time

EVEN THOUGH CAMP ONLY LASTS 1 WEEK, THE COMMUNITY LASTS FOREVER.

cmtausa.org/supportcampfootprint

CMTA Charcot-Marie-Tooth Association

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MORE THAN A MEETING: HOW THE CMTA COMMUNITY SHOWED UP IN SAN DIEGO

In the spring of 2025, a remarkable sequence of events unfolded in San Diego. Part scientific milestone, part community celebration, and all heart. What began as a behind-the-scenes opportunity for CMTA community members to visit CMTA's Strategy To Accelerate Research (CMTA-STAR) Alliance Partner Actio Biosciences (Actio) turned into something bigger. Something deeply human. Something unmistakably CMTA.

A Visit to Remember

Laurie Cook still remembers the moment her husband, Steven, a journalist with CMT2C, received the invitation. Actio was hosting a CMTA Community Day, and for Steven, Schenectady, NY's Daily Gazette editor, it would be his first time visiting a research facility focused on his CMT2C. What his family experienced in San Diego left a lasting impression.

Actio's researchers welcomed the Cook family and other CMTA guests with warmth and generosity. They hosted a tour, a Q&A session, and a social hour. For families living with CMT2C, it was a powerful reminder that progress isn't just measured in milestones. It is also measured in moments of connection and in the strength of the community.

In his follow-up article, Steven wrote, "It was a powerful thing to see so many others in person dealing with CMT, to be able to speak freely about our experiences, and to hear from researchers and pharmaceutical representatives directly about the efforts underway."

Reflecting on the day, Laurie shared what it meant to her as both a partner and a parent.

"As a parent of three children with CMT2C, I've struggled with helplessness for years. Visiting Actio gave me something I haven't felt in a long time—hope. The scientists weren't just kind. They saw us. They answered every question with care and stayed long after the tour ended to talk with us. We were treated not like outsiders, but like part of something."

A Branch That Builds Belonging


Kennie Trout

That same spirit of connection is what drives Kennie Trout, the longtime leader of the CMTA San Diego Branch. For more than a decade, Kennie has helped build a community where no one feels like a stranger. He is a Branch Leader, a CMTA Walk 4 CMT organizer, and a Camp Footprint counselor. But more than that, he is a connector.

"I've never been a leader," Kennie said. "I just want to help people get together and make things happen. I've had the most fun with CMTA community members, and I want people to feel that same sense of community."

That authenticity shows in everything he does, from helping people get rides to events to making sure new faces feel seen and welcome. Under Kennie's leadership, the San Diego Branch has become more than a meetup group. It is a local anchor in the CMTA's nationwide Branch network, proving that real community grows when people show up for each other.


Actio Biosciences welcomed CMTA community members, and joined the San Diego branch to Walk 4 CMT.

Walking the Walk In San Diego

The connection between Actio Biosciences and the CMT community came full circle later that week at the CMTA San Diego Walk 4 CMT. Actio did not just sign on as a sponsor. They showed up. Employees walked alongside participants, cheered them on, and stood shoulder to shoulder with families affected by CMT.

Their presence sent a powerful message. It showed how deeply they value the community they are working to help. We are grateful for their commitment to stand with the people at the heart of this work.

For the Cook family, for Kennie Trout, and for everyone who joined the Walk, the day was about more than fundraising. It was about belonging. It was about being seen, supported, and surrounded by others who understand.

It was community in action.

Find Your Place. Walk With Us.

From CMTA Community Days to CMTA Branch meetings to CMTA Walk 4 CMT events, this is what it looks like when a community shows up. Every step taken. Every story shared. Every new connection formed. It all adds up to something bigger than any one person.

It adds up to progress. To belong. To hope.

Because at CMTA, community is not just part of the mission. Community is everything.

Start something powerful in your own backyard. Launch a Walk 4 CMT. Connect with your local CMTA Branch or start your own. Be the reason someone else feels seen.

Finding Her Footing: Christina's Walk 4 CMT

For CMTA COMPASS member Christina Johnson, CMTA's Sarasota Walk 4 CMT was more than a fundraiser. It was a reunion. A reminder. A homecoming.

Christina and her dad both live with CMT1B. For them, the walk wasn't just about raising awareness. It was about being present. For each other. For their community. For the growing network of families, friends, and young adults who live with CMT and choose to face it together.

Christina didn't walk alone. At her side were her dad, her fiancé, her sisters, and their partners, her whole support system walking with her, every step of the way. The day became a reminder that while CMT brings real challenges, no one has to face them alone.

> *"This community reminds me that I don't have to walk this road alone. Together with my funky foot tribe and so many others, I keep moving forward."*

Another highlight? Reuniting with friends she met through the CMTA COMPASS Young Adult program and at Camp Footprint the previous summer. She is a shining example of how programs like Camp Footprint are working to grow our next generation of community leaders. She also made new connections with fellow walkers—people who, like her, know the power of being surrounded by those who truly get it.

"Hugs, laughter, stories, even the quiet moments," Christina said. "It just felt like home."

Whether someone was newly diagnosed, a longtime advocate, a parent, a partner, or a young adult with CMT, the Sarasota Walk brought people together. And in that togetherness, Christina found something lasting: *Belonging.*

Find your Community—Join Compass today!

CMTA FILMS PRESENTS: JONAH BERGER

A longtime CMTA staff member, youth program leader, and tireless advocate, Jonah Berger, our National Youth Programs Manager, has shaped countless lives through Camp Footprint and beyond.

Now, in this short film from CMTA Films, Jonah shares his story in his own words. It is a story about growing up with CMT, finding strength in identity, and learning to lead, not in spite of difference, but through it.

With his signature honesty and heart, Jonah reflects on the challenges and joys of living with CMT and on the lifelong search for belonging that so many in our community know well.

CMTA Films Presents: Jonah Berger is not just about one person's journey. It is a tribute to the power of community, visibility, and voice. It is a reminder that when we share our stories, we create space for others to do the same.

At CMTA, community is everything. And Jonah has helped build that community from the inside out.

Watch the film and hear Jonah's story at cmtausa.org/news/cmta-films-presents-jonah-berger/

CMTA
Charcot-Marie-Tooth Association
INDIANAPOLIS

2025 CMTA PATIENT & RESEARCH SUMMIT

Join Us!

THE CMTA PATIENT & RESEARCH SUMMIT is back, and this year we are gathering in Indianapolis, Indiana for a weekend of community connection, CMT education, and updates from CMTA's Strategy to Accelerate Research (CMTA-STAR) program.

Just minutes from the Indianapolis airport, the Summit brings together individuals living with CMT, caregivers, clinicians, and researchers from across the country. Friday night includes a welcome reception and screening of Jeffrey's Journey, a documentary about CMT community member Jeffrey McElfresh *(see page 17 to read about Jeffrey's Journey)*

Saturday will focus on living well with CMT, featuring expert-led sessions on bracing, physical therapy, adaptive tools, and a surgical panel chaired by iconic foot and ankle surgeon Glenn Pfeffer, MD.

New this year, Small Group Table Topics will offer focused conversations on mental wellness, parenting, and navigating a new diagnosis.

Saturday also brings plenty of opportunities to connect over meals, breaks, and the evening's dinner reception. A special Dralla Day Youth Outing will give kids and teens a chance to explore Indianapolis and bond with their peers.

Sunday morning begins with keynote speaker Dustin Turner, followed by research-focused conversations led by leading experts, including Joshua Burns, PhD, from St. Jude, with CMTA-STAR Alliance Partners Actio Biosciences, Applied Therapeutics, and NMD Pharma. We will also hear from John Svaren, PhD, Chair of the STAR Advisory Board, and Dr. Katherine Forsey on the progress of CMTA-funded research.

Space is limited, and discounted hotel rooms at the Embassy Suites Plainfield (Indianapolis Airport) are available until August 4.

Questions? **Contact Laurel Richardson, CMTA Director of Community Outreach, at laurel@cmtausa.org**

SEPTEMBER 5–7, 2025

REGISTER AT summit.cmtausa.org

SUMMIT WELCOME
FRIDAY, SEPTEMBER 5, 2025

3:00 PM – 5:30 PM
EARLY BIRD REGISTRATION AND CHECK-IN
Start your Summit weekend stress-free by checking in early. Swing by the registration table to pick up your badge and welcome materials.

5:30 PM – 7:30 PM
"MEET & GREET" HAPPY HOUR
Lobby Bar and Restaurant Area
Join fellow attendees, CMTA staff, and community members for a casual welcome gathering in the Lobby Bar and Restaurant Area. Whether it's your first Summit or your fifth, this is a perfect chance to say hello, swap stories, and settle in for the weekend ahead.

7:30 PM – 9:00 PM
MOVIE SCREENING: *JEFFREY'S JOURNEY*
Location: Hendricks C
Jeffrey's Journey is a full-length documentary that aired on PBS, featuring Jeffrey McElfresh, a long-distance cyclist with CMT.
Discussion and Q & A with Jeffrey will follow.
See story on page 17.

SUMMIT SATURDAY, SEPTEMBER 6, 2025

Presented by
APPLIED THERAPEUTICS

8:00 AM – 9:00 AM
REGISTRATION CHECK-IN AND BREAKFAST
We welcome attendees to check in at the registration table during this time, then join us in the ballroom for a full plated breakfast, coffee, and refreshments.

9:00 AM – 9:20 AM
WELCOME ADDRESS
- ***Speaker: Sue Bruhn, PhD***

Chief Executive Officer, Sue Bruhn, PhD, will give the opening address which will include CMTA highlights, the Summit flow of the day, and much more.

9:25 AM – 10:10 AM
IU HEALTH CMT CLINIC TEAM
- ***Speakers: Laurie Gutmann, MD and her CMT clinic team***

During this session, you will have the opportunity to meet the CMTA Center of Excellence team from IU Health. This panel discussion will be led by CMT Clinic Director, Dr. Laurie Gutmann. This will be an interactive, open Q&A panel discussion.

10:15 AM – 10:50 AM
CMT AND EXERCISE
This session focuses on the most important exercises people with CMT can do daily to help manage CMT. Mary Schmidt, DO, a physiatrist at the CMTA Center of Excellence at the University of Michigan, shares best practices for physical activity in people with CMT. Published studies provide evidence that exercise significantly benefits how people with CMT feel and function, and this session will help you to get started or build on your current practice.

10:50 AM – 11:20 AM
BREAK
Take a moment to enjoy the exhibit hall, have a snack, join a mini workout session led by Julie Stone, and mingle with other attendees.

11:20 AM – 12:00 PM
HELP FOR TODAY: GADGETS AND ASSISTIVE TECHNOLOGY
Speaker: Ashley McLeroy, MSME
This session focuses on the best gadgets, adaptive tools, and assistive technology devices that can make activities of daily living easier for people with CMT.

12:05 PM – 12:45 PM
CMT SURGERY PANEL
Speakers:
- ***Session Chair: Glenn Pfeffer, MD, Cedars-Sinai Medical Center***
- ***Paul Talusan, MD, University of Michigan***
- ***Tyler Gonzalez, MD, MBA,*** *Prisma Health*

This session is a panel discussion led by renowned foot and ankle orthopedic surgeon Dr. Glenn Pfeffer. The discussion will focus on the best surgical options, practices, and outcomes for CMT foot reconstruction.

12:45 PM – 1:45 PM
LUNCH
Take time to enjoy lunch, the exhibit hall, join a mini workout with Julie Stone, and mingle.

1:45 PM – 2:30 PM
BRACING SESSION
- ***Speaker: David Meisner, BSc (HK), CPO, MBA and local orthotist TBD***

This panel discussion on bracing and AFOs for CMT patients will be led by orthotist David Misener. The panel will share information on cutting-edge bracing options for drop-foot and balance issues.

2:35 PM – 3:00 PM
YOUR STORY, YOUR MOJO, YOUR MOVE
- ***Speaker: Jeana Sweeney*** *CMTA Chief Engagement & Gift Officer*

In this session, you will hear from Jeana Sweeney, a CMT community member, parent of a CMT patient, and a passionate and dedicated CMTA staff member. Hear her story and find yours while learning how we can all make a difference.

3:15 PM – 4:30 PM
SMALL GROUP TABLE TALKS
You are invited to join one of three small groups to discuss some of the challenges of living with CMT, such as 1) being newly diagnosed and wanting to learn what's next in your CMT journey, 2) parents wanting to chat with other parents to learn tips on how best to support your child with CMT, and 3) the mental health side of living with CMT.

1. Newly Diagnosed
Facilitated By: TBD

2. Parents to Parents
Facilitated By: TBD

3. Mental Health Wellness
Facilitated by: Elizabeth Misener, PhD, LMSW

ALSO ON SATURDAY

9:15 AM – 3:15 PM
DRALLA DAY YOUTH OUTING
Led by Jonah Berger
(Registration is required to attend) Jonah Berger and chaperones will take CMT youth ages 8-18 out for a day full of fun and connection.

5:00 PM – 6:30 PM
COMPASS YOUNG ADULTS HAPPY HOUR *(ages 19-31)*
(Registration is required to attend)

6:30 PM – 8:30 PM
SUMMIT DINNER RECEPTION
(Registration is required to attend)
Join us for dinner to unwind, connect, and celebrate community with CMTA Summit attendees, researchers, doctors, and CMTA staff.

SUMMIT STAR SUNDAY, SEPTEMBER 7, 2025

8:30 AM – 9:00 AM
BREAKFAST AND KEYNOTE ADDRESS
- ***Keynote Speaker: Dustin Turner***
  Please join us for a full plated breakfast, coffee, and refreshments. The keynote address will be given during this time.

9:05 AM – 9:25 AM
RESEARCH ROUNDUP: CMTA-STAR IN SUMMARY
- ***Speaker: Katherine Forsey, PhD*** *CMTA Chief Research Officer*
  There is so much going on in CMT research and treatment development right now that we need a roundup to share it all with you. This session will be a comprehensive summary across the projects CMTA is funding, thanks entirely to the support of the CMT community.

9:30 AM – 10:00 AM
TREATMENT APPROACHES FOR CMT
Speakers:
- ***Session Chair: John Svaren, PhD*** *University of Wisconsin*
- ***Jordan Verplank, PhD*** *Uniformed Services University*

There are several different types of treatments in development for CMT at the moment. This session will explain the different treatment approaches and deep dive into a couple of examples in development in Biotech and Academic Labs.

10:05 AM – 10:30 AM
STAGES OF DRUG DEVELOPMENT
Speaker:
- ***Sue Bruhn, PhD,*** *CMTA Chief Executive Officer*

This session focuses on the different phases of drug development from conception to bringing a drug to market, and how CMTA works with patients to accelerate progress toward treatments.

10:30 AM – 11:00 AM
BREAK
Take a moment to enjoy the exhibit hall, have a snack, join a mini workout with Julie Stone, and mingle with other attendees.

11:00 AM – 11:20 AM
CLINICAL TRIAL DEVELOPMENT IN CMT
Speaker:
- ***Josh Burns, PhD*** *St. Jude's Children's Research Hospital*

Dr. Burns summarizes the most up-to-date research perspective on how we can measure change over time in patients with CMT, and the importance of studying different outcome measures, biomarkers, and Natural History to accelerate the development of new treatments.

11:25 AM – 12:10 PM
EXCITING PROGRESS IN CLINICAL TRIALS FOR CMT
Speakers:
- ***Session Chair / Panel Moderator: Josh Burns, PhD,*** *St. Jude's Children's Research Hospital*
- ***Daniel Brennan,*** *NMD Pharma*
- ***Evan Bailey, MD ,*** *Applied Therapeutics*
- ***Michael Imperiale,*** *Actio Biosciences*

2025 is a pivotal year for clinical trials in CMT, with five active trials in the US as we go to press and more on the horizon. You will hear a discussion of clinical development for CMT with CMTA-STAR Alliance Partners NMD Pharma, Applied Therapeutics, and Actio Biosciences.

12:15 PM – 12:25 PM
PATIENTS AS PARTNERS IN RESEARCH
- ***Speaker: Katherine Forsey, PhD*** *CMTA Chief Research Officer*
  CMT research and clinical trials cannot happen without you. In this session, learn how you can become a Patient as Partner in Research and participate in the critical work of furthering the development of treatments for CMT. Your voice shapes the future of CMT research, and we cannot wait to hear from you.

12:25 PM – 12:30 PM
SUMMIT CLOSING REMARKS
Speaker: Gilles Bouchard, CMTA Board Chair

INNERVATORS HONOR ROLL

According to the dictionary, an innervator is a nerve stimulator. CMTA INNERVATORS, however, are action-oriented game-changers that sustain CMTA with monthly gifts throughout the year. Life-changing initiatives like Camp Footprint, treatment-focused research, and invaluable education initiatives depend on steady, reliable support from committed and reliable donors. Add your name to the INNERVATORS Honor Roll by visiting cmtausa.org/cmta-innervators and joining today!

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BECOME AN INNERVATOR TODAY!

FINDING MEANING IN MOTION

CYCLIST WITH CMT SHARES HIS JOURNEY

Jeffrey McElfresh is a solo cyclist. A storyteller. A person with CMT and a deep love for the road.

In Jeffrey's Journey, a feature-length documentary that aired on PBS, Jeffrey invites us along for a 10-day ride from Pittsburgh to Cincinnati, following the Ohio River through the Appalachian region. The film moves not just through miles, but also through memory, fear, resilience, and the quiet kind of courage that builds over time.

He camps. He climbs. He adapts. “Each year, walking becomes more difficult,” he says. “But when I'm on two wheels, well, that's a different story.”

CMT affects his balance, grip, and endurance. And still, he rides. His gear includes sandals with clips to compensate for lost foot sensation. He plans each route carefully, adjusting as needed. Starting and stopping are hard. But he keeps going.

“People ask how my disability affects my riding,” he says. “The real change is in my riding style. Speed is not my focus. I'm not competitive. I'm in no rush. I don't have to be first. I just have to get there.”

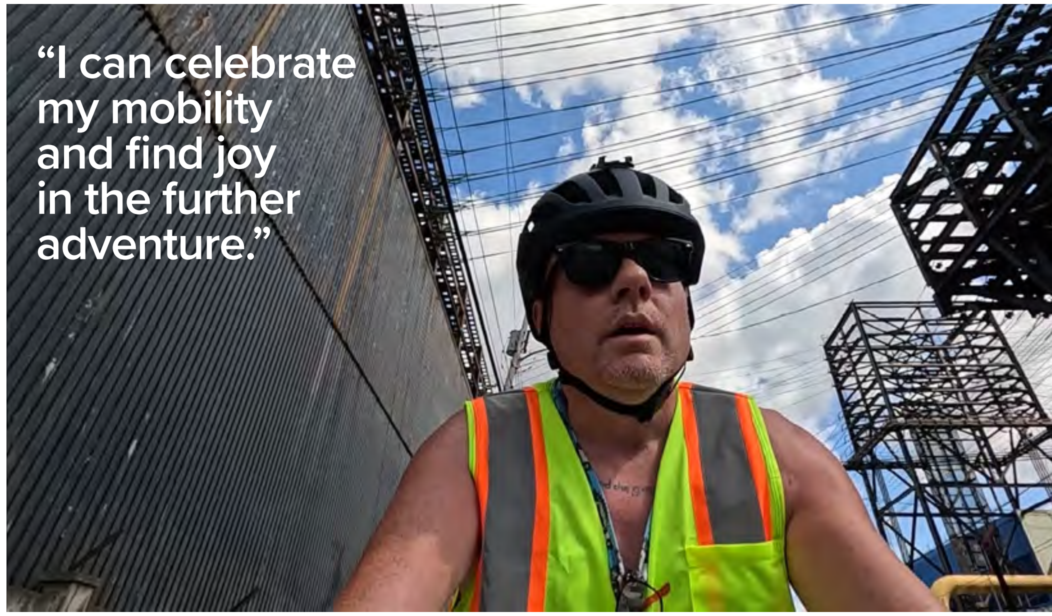
As he rides, Jeffrey reflects on the Appalachian towns he passes, shaped by shifting industries and the people who built them. He shares roadside history, environmental realities, and small moments of connection. He honors the past in places like Ripley, Ohio, a vital stop on the Underground Railroad. And he names the present for what it is. “There's much work to be done,” he says, pedaling on.

The road gives him more than motion. It offers meaning. “It was a peace of mind that I carried with me all the time,” he says. “And once I found it within me, I learned I could access it almost anywhere.”

Along the way, we meet Tracy, his partner and, in Jeffrey's words, his comic relief. She supports him even when the bike tours make her nervous. We see the toll of progressive weakness and the quiet acts of independence he fights to keep. We feel his solitude, his humor, and his freedom. This is not a film about arrival. It is a film about presence.

Jeffrey's journey is not only his own. It is a reminder that the road forward is shaped by those who ride beside us, whether in person, in spirit, or in community.

Join us at the 2025 CMTA Summit for a screening of Jeffrey's Journey, followed by a live Q&A with Jeffrey and the film's producer/director, Richard Wonderling, on Friday night, September 5 in Indianapolis.



> “I can celebrate my mobility and find joy in the further adventure.”

Jeffrey's Journey **aired on PBS and will be screened at the 2025 CMTA Summit. Come watch it with us!**

Friday, Sept. 5, 7:30pm-9pm

Jeffrey is riding his bicycle the 150 miles from Dayton, Ohio, to attend the Summit. He invites everyone to be a part of his further adventure by following along on Facebook or Instagram @juicymelt.

POWERED BY THE PEOPLE: CMTA GOES WHERE THE SCIENCE GOES

For four days in Edinburgh, Scotland, the CMTA team walked through the halls of the 2025 Annual Meeting of the Peripheral Nerve Society (PNS), representing a force far greater than just science. We were there for you. For every person living with CMT. For every family member seeking answers. For every donor fueling progress.

CMTA Chief Research Officer Katherine Forsey, PhD, and CMTA CEO Sue Bruhn, PhD, reviewed hours of presentations, visited every CMTA-funded poster, and met with researchers and biotech teams from around the world. At every turn, they found the same truth: this community is powering real progress in CMT research. And that progress is being recognized on a global stage.

Setting the Stage: Understanding CMT, Natural History, and Biomarkers

The laboratory of Michael Shy, MD, Chair of CMTA's Clinical Expert Board, as well as others, shared critical updates on natural history studies and emerging biomarkers. These are essential tools for understanding how CMT progresses and how it can be measured in clinical trials. Other data from the meeting came from the lab of Dr. David Hermann, also on the STAR Advisory board, who shared a key update from the CMTA-funded ACT-CMT natural history study for CMT1A. This critical study documented changes over three years in the biomarker calf fat fraction as measured by non-invasive MRI imaging. Calf muscle fat fraction MRI is a new biomarker under development for researchers to measure disease progression and accelerate the assessment of new therapies in clinical trials.

Building New Models and Developing Treatments

Standout presentations from members of the FIERCELab at Hasselt University in Belgium, led by Esther Wolfs, PhD, showed how stem cells derived from teeth donated by individuals with CMT through a CMTA-funded project are being used to model CMT in the lab. "They're doing some amazing research with those cells and identifying new targets for treatments," said Dr. Forsey. A CMTA-funded project run by the Timmerman Lab in Antwerp, Belgium, highlighted progress in 3D human cell models, a useful tool for testing therapeutics. Additional sessions presented early-stage findings for CMT2A and CMT2D and expanded research on HNPP and CMT1A.

CMTA Alliance Partner Applied Therapeutics shared new data from the two-year time point of their trial of govorestat (AT-007) in patients with CMT-SORD, the most advanced therapeutic in development for CMT. They detailed how MRI calf-fat fraction imaging showed changes with govorestat treatment for some patients, and assessment with the CMT Health Index (a comprehensive measure of how patients feel and function) continued to look promising.

Spotlight on Innovation: AI in CMT Research

Artificial intelligence (AI) emerged as a major theme throughout the week. Researchers demonstrated how AI is being used to analyze MRI scans, identify blood-based biomarkers, and measure how people with CMT feel and function using wearable sensors. These tools aim to improve precision and speed in tracking disease progression. CMTA-funded projects in Miami, FL, and London, UK, highlighted progress in AI-assisted gene discovery, including research using the GENESIS genomic research platform and database led by CMTA-STAR Advisory Board member Stephan Züchner, MD, PhD. *(See Dr. Forsey's Under the Microscope column in the Summer 2024 issue of The CMTA Report for more on AI in CMT.)*

Posters Tell the Story: The Genetic Landscape and Beyond

The poster exhibition brought a year of progress into focus. The CMTA is proud to share that, of the 157 posters presented at PNS 2025 focusing on CMT and related neuropathies, 24 were funded by the CMTA, including four oral presentations. Dr. Forsey presented CMTA's poster, *The Genetic Landscape of Charcot-Marie-Tooth Disease*, sharing Kenneth Raymond's comprehensive review of all known CMT genes. The analysis identified 135 genes linked to 169 genetically defined subtypes and visualizes CMT's complex classification system. It also highlights the disconnect between genetic subtypes and clinical prevalence (number of people with each subtype), reinforcing how much of the landscape is still being charted. *(See story on page 23).*

A Shared Mission, A United Community

The PNS conference drew almost 1,500 researchers, along with dozens of biotech companies, from 64 different countries. The CMTA team held many individual and partner meetings across the four days to discuss current and future projects and clinical trials. They also visited every CMTA-supported poster and CMT-related platform presentation, connected with the investigators behind the work, and explored avenues for future research. "It's amazing to see in one place just how much research the CMT community is powering through their donations," said Dr. Forsey.

To Learn More About CMTA's Research, Visit cmtausa.org/our-research/

CMTA'S STAR ADVISORS CONVENE IN EDINBURGH

CMT France President Alexandre Hoyau (left) joined CMTA CEO Dr. Sue Bruhn at the CMTA-STAR Advisory Board Meeting in Edinburgh in May 2025. Alexandre attended as an invited guest of CMTA.

The day before the PNS conference, CMTA hosted an annual in-person meeting of its world-leading STAR Advisory Board and invited guests from the CMT research and biotech community. The much-anticipated meeting was chaired by CMTA's Chief Research Officer, Dr. Katherine Forsey, and opened by Scientific Advisory Board Chair, Prof. John Svaren. We use this meeting to identify knowledge gaps, guide future research strategies, assess the funding of new research projects, and facilitate global collaboration to advance CMT treatment development. The themes of the 2025 meeting were axon degeneration (which can appear in all types of CMT), delivery and targeting of therapeutics to the peripheral nervous system (a challenge for all drug developers), and collaboration between researchers and biotech companies to accelerate drug development and FDA approvals. The day allowed us to focus on what is important in CMT research and how the broad and differentiated approach of CMTA could have the greatest impact as we drive progress to accelerate new treatments and ensure our ecosystem is in the best shape possible for clinical trials.

CLOSING THE LOOP

By the end of the week, the message was clear: the CMTA community is driving unprecedented momentum in CMT research. Five clinical trials are active, and more are coming. AI tools are speeding discovery. New models are being built from donated cells. And every one of these milestones traces back to the same source: You.

You showed up. You funded the work. You made it possible. And because of that, we'll keep moving forward. Together.

CMTA-STAR Meeting At-a-Glance:

44 CMT Specialists In Person
8 CMT Specialists Via Zoom
5 Biotech Companies
10 Presentations Plus Q&A
3 Facilitated Discussions
8 CMT Patients
6 CMT Parents

CMTA-STAR: KATHERINE'S KORNER

Katherine's Korner explores the heart of CMTA's Strategy To Accelerate Research (CMTA-STAR) and its impact on advancing treatments for CMT. Led by Katherine Forsey, PhD, CMTA's Chief Research Officer, CMTA-STAR brings together world-renowned scientists, clinicians, industry leaders, and the CMT community to drive progress in CMT research. Since launching in 2008, CMTA-STAR has invested more than $30 million to support groundbreaking discoveries that bring us closer to effective therapies and, ultimately, a cure.

In this edition, we highlight the latest CMTA-STAR-powered projects, from genetic therapies and new treatment strategies to promising tools for tracking CMT progression. We also feature current opportunities to get involved in research that's changing the future of CMT.

PARTICIPATE IN ONGOING RESEARCH

SYNAPSE-CMT: A Phase II Clinical Trial

CMTA-STAR Alliance Partner NMD Pharma is enrolling adults with genetically confirmed CMT1 or CMT2 in the SYNAPSE-CMT Phase II trial. This study is testing whether a new investigational drug, NMD670, can help improve communication between nerves and muscles. This trial his still actively recruiting additional community members with either CMT1 or CMT2 who would be willing to test this new potential treatment.

Participants will complete walking and mobility assessments over the course of the study. If you're ready to take part in research that could move the entire field forward, this is your opportunity.

Learn more about eligibility and locations at cmtausa.org/synapse

ACT-CMT: Join the Effort to Accelerate CMT1A Trials

The ACT-CMT study is enrolling adults with genetically confirmed CMT1A to help researchers measure how CMT progresses over time. The goal? To speed up the path to clinical trials that lead to real treatments.

If you're between 18 and 65 and ready to be part of something bigger, this is your chance to move the needle for the entire CMT1A community. The study includes two visits, about a year apart, at one of four medical centers in the U.S. Qualified travel expenses are reimbursed.

Learn more about how you can get involved at cmtausa.org/pap

CMTA-INC ALLIANCE RESEARCH STUDIES

Through the CMTA-INC Alliance, CMTA supports research that's shaping the future of CMT from infancy through adulthood. Four active studies are focused on understanding how CMT progresses, how to measure it at every age, and how to unlock the next breakthroughs in treatment and gene discovery.

CMTA Charcot-Marie-Tooth Association

+ Inherited Neuropathy Consortium

Natural History Study (INC 6601)

Join a long-term study that tracks how CMT progresses over time in people with any type of CMT, with special focus on CMT1B, CMT2A, CMT4A, and CMT4C. Annual visits include neurological exams, nerve tests, and a quick health questionnaire. Results help build the baselines that future treatments will be compared against. All adults and kids with CMT are welcome.

Learn more at cmtausa.org/pap

Gene Discovery in CMT2 (INC 6602)

Scientists are using next-generation sequencing to uncover new disease-causing and modifier genes for CMT2. If you have CMT2 without a genetic diagnosis, or CMT1A and want to help discover genetic modifiers—this one-time blood study is for you. Your DNA could lead to the next big breakthrough.

Learn more at cmtausa.org/pap

CMTPedS Study (INC 6603)

This study is enrolling families of children under 21 with CMT1, CMT2, or CMT4. The goal is to refine a scale that helps researchers and clinicians measure how CMT progresses in kids and teens.

Learn more at cmtausa.org/cmtpeds

CMTInfS Study (INC 6611)

This study invites families of children ages 0–4, with or without CMT, to help develop a new tool for measuring early signs of CMT in infants and toddlers. It's a step toward better diagnosis and treatment planning at the youngest ages.

Learn more at cmtausa.org/infant

CMTA-STAR PROJECT UPDATES

Finding Hidden Answers Through Long-Read Sequencing

CMTA is investing $210,000 in a new research project that aims to uncover the hidden genetic causes of CMT. Led by Andrea Cortese, MD, PhD, at University College London, this CMTA-STAR study uses long-read sequencing technology to detect complex mutations that standard tests might miss.

Right now, up to half of the people with axonal forms of CMT still don't have a confirmed genetic diagnosis. This study is designed to help more people get the answers they deserve and expand access to future research opportunities.

Learn more at cmtausa.org/news/cmta-invests-long-read-sequencing

CMTA Invests in CRISPR Research for CMT2A

CMTA is investing $300,000 in a new gene editing project at Gladstone Institutes to explore a CRISPR-based treatment for CMT2A. Led by CMTA-STAR Advisory Board Member Bruce Conklin, MD, this research is testing whether CRISPR can silence the mutated copy of the MFN2 gene while leaving the healthy copy untouched.

CMT2A is the most common axonal form of CMT and is caused by mutations in the MFN2 gene. With more than 200 different known mutations, finding a one-by-one solution would be slow and costly. This study aims to change that by developing a gene editing approach that could work across many different mutations, bringing the possibility of a single therapy to more people, faster.

Learn more by visiting cmtausa.org/news/cmt2a-multi-crispr

New Imaging Study Targets Cellular Defects in CMT2A

CMTA is investing $90,860 in a research project at UC San Diego to study how mutations in the MFN2 gene drive progression in CMT2A. Led by Uri Manor, PhD, the study uses high-resolution imaging and artificial intelligence to track how cells behave in CMT2A, with the goal of identifying new therapeutic targets.

By watching how key cell structures move inside neurons from people with CMT2A, this project will help researchers pinpoint what's going wrong and how potential treatments might help. The team will test their approach in both patient-derived cells and a specialized animal model with an MFN2 mutation found in people with CMT2A.

This cutting-edge research could lead to faster screening of drug candidates for CMT2A and may also help unlock insights for other axonal forms of CMT.

Learn more by visiting cmtausa.org/news/cmt2a-ai-imaging

CMT2F Study Needs Just a Few More Patients

With CMTA support, an international study on CMT2F researchers are looking for just a few more participants to stay fully enrolled through the follow-up phase. This study, led by CMTA-STAR Clinical Expert Board Chairperson Michael Shy, MD, is tracking how CMT2F progresses and helping define the best ways to measure those changes for future clinical trials.

So far, 25 participants have joined across sites in the U.S., UK, and Italy. The team is gathering critical data through clinical assessments, MRIs, and blood and skin samples.

Learn more by visiting cmtausa.org/news/cmt2f-full-recruitment

CMT CLINICAL TRIAL UPDATES

Research doesn't pause once a study begins. Across the CMTA-STAR Alliance and beyond, trial sponsors are sharing important updates as they move treatments for CMT through the clinical pipeline. While these trials may not be actively enrolling, every step forward brings us closer to answers.

Applied Therapeutics Presents Two-Year Data on CMT SORD

CMTA-STAR Alliance Partner Applied Therapeutics shared new 24-month results from its Phase III INSPIRE trial for CMT SORD at the annual meeting of the Peripheral Nerve Society, held in Edinburgh, Scotland, in May. The data showed continued reduction in blood sorbitol levels, improvements in MRI muscle-fat fraction. The company plans to discuss these findings with the FDA to discuss next steps,

Learn more by visiting cmtausa.org/news/cmt-sord-pns2025

Actio Biosciences Doses First Participant in Phase I CMT2C Trial

CMTA-STAR Alliance Partner Actio Biosciences has dosed its first healthy volunteer in the Phase I trial of its investigational drug for CMT2C, ABS-0871. This phase will assess safety, tolerability, and how the drug moves through the body. ABS-0871 received orphan drug designation (ODD) and rare pediatric disease designation (RPDD) from the FDA, marking a big milestone for CMT2C.

Learn more by visiting cmtausa.org/news/actio-phaseI

Augustine Therapeutics Begins Phase I Clinical Trial

Augustine Therapeutics has dosed the first healthy volunteer in its Phase I trial of AGT-100216, its investigational drug for CMT. The study is assessing safety, tolerability, and how the drug moves through the body. This is an important step in advancing a potential treatment for CMT.

Learn more by visiting cmtausa.org/news/augustine-hdac6-phase1

continued on page 22

KATHERINE'S CORNER

continued from page 21

SCIENCE THAT MOVES US FORWARD

In addition to clinical trials and CMTA-STAR-funded projects, researchers and advisors across the CMTA-STAR network are making important strides in the lab, in clinics, and in published literature. Here are just a few recent highlights from the global effort to understand, diagnose, and treat CMT.

Professor Mary Reilly Awarded MBE for CMT Leadership

CMTA-STAR Clinical Expert Board Co-Chair Professor Mary Reilly, MD, has been appointed Member of the Order of the British Empire (MBE) for her outstanding contributions to CMT research and clinical care. A global leader in inherited neuropathies, Prof. Reilly co-founded several key UK-based centers advancing neuromuscular disease research. Her work with CMTA-STAR and the Inherited Neuropathy Consortium (INC) has helped accelerate clinical trials and gene discovery worldwide. CMTA applauds Prof. Reilly's recognition and her ongoing impact on our community.

Learn more by visiting cmtausa.org/news/reillymbe

New Study Shows MRI Could Be A Biomarker for CMT1A Progression

In a study published by CMTA-STAR Clinical Expert Board Co-Chair Professor Mary Reilly, MBE, researchers found that MRI scans of muscle fat accumulation can forecast disease progression in CMT1A. In a four-year study of 20 adults, MRI fat measurements taken just 12 months after the first scan predicted worsening symptoms during the study. Early data suggest MRI may provide a faster and more reliable way to monitor treatment effects, helping accelerate the drug development process.

Learn more by visiting cmtausa.org/news/cmt1a-mri-biomarker-study

New Research Identifies A Promising Treatment Target for CMT1B

CMTA-STAR Advisory Board member Maurizio D'Antonio, PhD, and colleagues have published research in *Brain* showing that boosting the activity of an important transcription factor that helps Schwann cells (the key cells for myelination) clear misfolded proteins and protect myelin in CMT1B. Their findings suggest this approach could benefit not only CMT1B but also other demyelinating forms like CMT1A and CMT1E. CMTA-STAR's support made this discovery possible, placing this important transcription factor on the radar as a compelling target for future therapies.

Learn more by visiting cmtausa.org/news/cmt1b-xbp1-target

New Review Highlights Key Advances in CMT Diagnosis And Care

CMTA STAR Advisory Board member Davide Pareyson, MD, released an expert review titled *What's New in 2025* detailing updates in CMT diagnosis and symptom management. The review covers refined diagnostic techniques, improved multidisciplinary care approaches, and emerging research trends that are shaping the way individuals with CMT are diagnosed and supported. Dr. Pareyson's review reinforces the importance of ongoing investment in accurate diagnosis and comprehensive care.

Learn more by visiting cmtausa.org/news/cmt-2025-update-davide-pareyson

GET INVOLVED: Participate and Progress

Every study featured in Katherine's Korner needs one essential ingredient to succeed: you. By joining CMTA Patients as Partners in Research, you help accelerate CMT research and bring hope to everyone living with CMT.

If you're looking for a way to make a real difference in the future of CMT treatments, participating in a clinical study is one of the most powerful things you can do. It's important to know that each study is different, with eligibility based on factors like age, genetic subtype, current health, or your ability to safely complete certain assessments. These criteria are carefully designed to protect participants and ensure researchers gather the most accurate and meaningful data. And remember, not qualifying for one study doesn't mean you won't qualify for others, or for the treatment when it becomes available. As research moves forward, we'll keep you informed through email updates so you never miss an opportunity to be part of the progress.

Ready to take the next step?
At CMTA, community is everything, and progress begins with participation.

Visit cmtausa.org/pap to register today.

PATIENTS as PARTNERS in RESEARCH
Charcot-Marie-Tooth Association

TOGETHER, WE DRIVE PROGRESS

CMTA-STAR's impact is made possible by the generosity of our community. Every research breakthrough, clinical trial, and scientific discovery is fueled by donors who believe in a future without CMT. Your support accelerates research, accelerates treatment development, and brings hope to everyone living with CMT. Donate today at cmtausa.org/supportstar to support CMTA-STAR and help drive the next breakthrough.

POWER IN THE PATTERNS: CMTA PRESENTS THE FULL GENETIC PICTURE OF CMT AT PNS 2025

At the 2025 Annual Meeting of the Peripheral Nerve Society (PNS) in Edinburgh, Scotland, this past May, the CMTA presented a new analysis of the CMT genetic landscape. The scientific poster, presented by CMTA Chief Research Officer Katherine Forsey, PhD, showcased a comprehensive review of every published CMT gene to date.

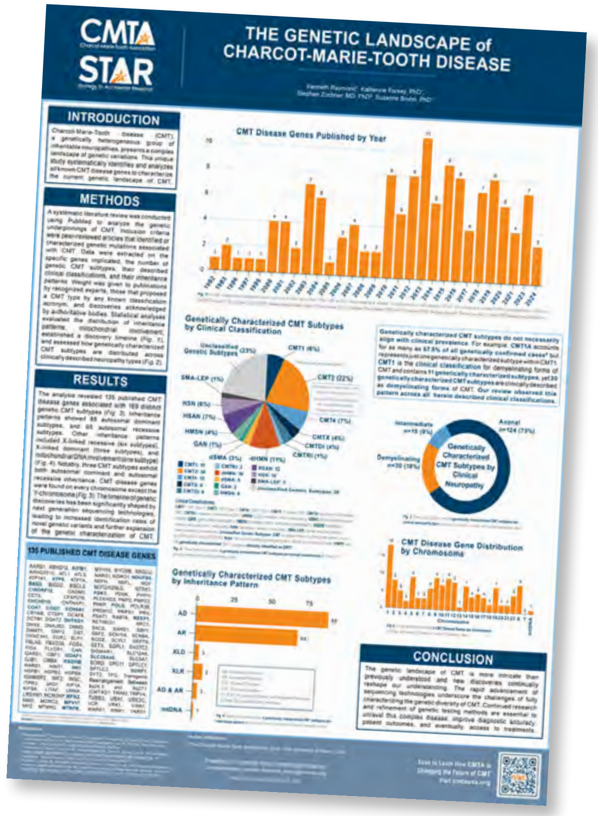
The findings are striking: 135 published CMT genes across 169 distinct genetic subtypes. Inheritance patterns include 88 autosomal dominant, 67 autosomal recessive, six X-linked recessive, three X-linked dominant, and one mitochondrial subtype. But the story doesn't end with numbers.

The study revealed that clinical labels like "CMT1" or "CMT2" don't always reflect the full complexity of genetic reality. For instance, CMT1A is just one of 11 subtypes under the clinical label CMT1, yet it accounts for more than two-thirds of all genetically confirmed cases. And while CMT1 is defined by demyelination, demyelinating subtypes also appear in other categories as well. In fact, 30 subtypes across the spectrum show demyelinating features. This pattern appears across all of CMT.

"This analysis shows how far we've come in understanding the genetic diversity of CMT," said Dr. Forsey. "But it also reminds us how far we still have to go."

This work isn't just a catalog. It's a map of what we know, what we've yet to discover, and what the CMT community needs next. And it's powered by the same force behind every step forward in this field: **You.**

View the poster, and read the full story at cmtausa.org/news/genetic-landscape-pns2025/



CMTA STAR

THE GENETIC LANDSCAPE of CHARCOT-MARIE-TOOTH DISEASE

INTRODUCTION

Charcot-Marie-Tooth disease (CMT), a genetically heterogeneous group of inheritable neuropathies, presents a complex landscape of genetic variations. This unique study systematically identifies and analyzes all known CMT disease genes to characterize the current genetic landscape of CMT.

METHODS

A systematic literature review was conducted using PubMed to analyze the genetic underpinnings of CMT. Inclusion criteria were peer-reviewed articles that identified or characterized genetic mutations associated with CMT. Data were extracted on the specific genes implicated, the number of genetic CMT subtypes, their described clinical classifications, and their inheritance patterns. Weight was given to publications by recognized experts, those that proposed a CMT type by any known classification acronym, and discoveries acknowledged by authoritative bodies. Statistical analyses evaluated the distribution of inheritance patterns, mitochondrial involvement, established a discovery timeline (Fig. 1), and assessed how genetically characterized CMT subtypes are distributed across clinically described neuropathy types (Fig. 2).

RESULTS

The analysis revealed 135 published CMT disease genes associated with 169 distinct genetic CMT subtypes (Fig. 3). Inheritance patterns showed 88 autosomal dominant subtypes, and 65 autosomal recessive subtypes. Other inheritance patterns included X-linked recessive (six subtypes), X-linked dominant (three subtypes), and mitochondrial DNA involvement (one subtype) (Fig. 4). Notably, three CMT subtypes exhibit both autosomal dominant and autosomal recessive inheritance. CMT disease genes were found on every chromosome except the Y-chromosome (Fig. 5). The timeline of genetic discoveries has been significantly shaped by next generation sequencing technologies, leading to increased identification rates of novel genetic variants and further expansion of the genetic characterization of CMT.

135 PUBLISHED CMT DISEASE GENES

Genetically characterized CMT subtypes do not necessarily align with clinical prevalence. For example, CMT1A accounts for as many as 67.5% of all genetically confirmed cases* but represents just one genetically characterized subtype within CMT1. CMT1 is the clinical classification for demyelinating forms of CMT and contains 11 genetically characterized subtypes, yet 30 genetically characterized CMT subtypes are clinically described as demyelinating forms of CMT. Our review observed this pattern across all herein described clinical classifications.

CONCLUSION

The genetic landscape of CMT is more intricate than previously understood and new discoveries continually reshape our understanding. The rapid advancement of sequencing technologies underscore the challenges of fully characterizing the genetic diversity of CMT. Continued research and refinement of genetic testing methods are essential to unravel this complex disease, improve diagnostic accuracy, patient outcomes, and eventually access to treatments.

CAN WE FIX THE CODE?

BY KATHERINE FORSEY, PhD, *Chief Research Officer, CMTA*

A LOOK AT GENETIC THERAPIES FOR CMT

Current treatments for Charcot-Marie-Tooth disease (CMT) focus mainly on managing symptoms. But new advances in genetic therapies are changing the conversation and offering real hope for the future. CMTA is investing heavily in research that targets the root cause of CMT, the genetic mutations that drive the disease.

I'm putting these therapies under the microscope because they're going to come up more and more as research advances toward clinical trials. It's important for all of us to stay informed, so let's dive in.

What Are Genetic Therapies?

Genetic therapies aim to correct or modify the underlying mutations that cause diseases like CMT. These therapies can work in several ways, including repairing, replacing, turning off, or adjusting how much of a gene is expressed. Different genetic subtypes need different approaches, depending on whether there is too much or too little of the product of the affected gene, and treatments can work like the dimmer switch on a light in your living room.

You might hear "gene therapy" and "genetic therapies" used interchangeably, but there's a difference:

- **Gene therapy,** which can also be called gene replacement therapy, refers specifically to inserting a healthy copy of a gene into cells to replace or compensate for a faulty one.
- **Genetic therapy** is a broader category. It includes gene therapy, but also includes tools like gene editing, gene silencing, and RNA-based treatments, where genes are specifically modified but not replaced.

Types of Genetic Therapies

Through CMTA's Strategy To Accelerate Research (CMTA-STAR), we're supporting work on a wide range of genetic therapy approaches:

- **Gene replacement therapy:** Delivers a working copy of a gene. CMTA is supporting work on this approach for CMT4C at the University of Texas Southwestern (UTSW) with researchers Xin Chen, MD, and CMTA-STAR Advisory Board member Steven Gray, PhD.

Other Genetic Therapy approaches:

- **CRISPR/Cas9:** Molecular scissors to precisely cut and fix faulty DNA. We're funding CRISPR-based studies at Gladstone Institutes with CMTA-STAR Advisory Board member Bruce Conklin, MD, for multiple types of CMT.
- **Antisense Oligonucleotides (ASOs):** Synthetic molecules that bind to RNA and block or modify how a gene is read. We're supporting ASO development at the University of Miami with project CMTA-STAR Advisory Board member Mario Saporta, MD, PhD.
- **Small Interfering RNA (siRNA):** Silences faulty genes by interfering with the RNA before it can make disease-causing proteins.
- **shRNA (short hairpin RNA):** Long-acting RNA therapy that can silence gene expression
- **Base editing and prime editing:** Precise, next-generation tools for rewriting individual DNA letters without cutting the DNA strand.

Our CMTA-STAR investments support multiple genetic therapy programs across the spectrum from the most common type, CMT1A, to rarer subtypes like CMT4C or CMT4J. These approaches have the most potential for impact because they may directly address the underlying genetics driving CMT.

The Big Hurdle: Getting to the Peripheral Nerves

One of the biggest technical challenges in treating CMT is delivery. Most genetic therapies need to reach the peripheral nerves, which are long, delicate structures protected by something called the blood-nerve barrier. The blood-nerve barrier provides important protection to nerves by keeping harmful substances out. But that same barrier can also provide a challenge and needs to be bypassed for the delivery of therapeutic treatments to the nerve.

CMTA-funded researchers are exploring multiple delivery strategies, including viral vectors, nanoparticles, extracellular vesicles, and direct injection, to get therapies where they need to go.

What's in the Preclinical Pipeline?

Thanks to our CMTA-STAR Alliance Partners, several CMT genetic therapies are moving closer to clinical trials:

- **CMT1A:** Novartis is developing an ASO, Armatus Bio is using siRNA with an AAV9 vector, Nervosave Therapeutics is exploring shRNA via local injection, and Sarepta is also working on a gene therapy approach. All aim to reduce the overexpression of the PMP22 gene. More than 200 CMTA community members helped shape one of these programs through our Patients as Partners in Research platform.
- **CMTX1 (aka CMT1X or CMTX):** CMTA-STAR Advisory Board member Kleopas Kleopa, MD, has created a gene therapy that replaces the faulty GJB1 gene that has shown success in animal models.
- **CMT2A and CMT1B:** CRISPR-based therapies are showing encouraging signs in early research.
- **CMT4C:** CMTA, the University of Texas Southwestern, and the CMT4C community are working together on Project Foresee to bring an AAV-based gene therapy into an investigator-led clinical trial.
- **CMT4J:** CMTA is supporting Elpida Therapeutics in their work to bring gene therapy to CMT 4J patients by supporting the 4J Natural History study as the company prepares for the clinical trial in parallel.

If these strategies succeed in these types of CMT, they can be adapted for other subtypes. Once we solve the delivery challenge, the technology becomes more widely applicable.

Looking Ahead

Genetic therapies represent a transformational step forward. They don't just manage symptoms; they aim to modify the disease itself.

While obstacles remain, particularly around delivery, the CMTA is committed to driving this science forward. We're hopeful that within the next 5 to 10 years, several of these therapies could move into clinical trials and eventually reach patients.

Katherine Forsey, PhD, is a trained biologist in the UK, earning her PhD in reproductive biology and IVF. As the CMTA's Chief Research Officer, she leads CMTA-STAR and oversees the CMTA-STAR Advisory Board, a team of over 30 world-renowned CMT experts who evaluate research projects and help shape CMTA's scientific strategy. Under her leadership, CMTA-STAR is managing more than 50 active research projects across academic labs and industry collaborations.

How You Can Help

This research doesn't move forward without the community.

- **Donate to CMTA-STAR** to fuel this work: **cmtausa.org/supportstar**
- **Join Patients as Partners in Research** to guide therapy development and be the first to hear about trial opportunities: **cmtausa.org/pap**

Together, we're changing what's possible for people with CMT.

THE TIES THAT FIND:

COMMUNITY CONNECTIONS POWERING NEW CMT GENES

Every CMT gene discovery starts with a question. For individuals with undiagnosed CMT, that question is often: Why haven't we found the gene yet?

CMTA's response: Together with you, we're accelerating genetic discovery, and the Distant Cousin Project is a powerful example.

Funded by a CMTA community donor and led by CMTA's Strategy To Accelerate Research (CMTA-STAR) Advisory Board member Stephan Züchner, MD, PhD, at the University of Miami, this research study focuses on individuals with CMT who have received negative genetic test results. Its aim is to identify new CMT genes by comparing the DNA of people with CMT and their distant relatives who are also affected.

Why distant cousins? Because they share far less DNA than close relatives, making it easier for researchers to spot shared mutations that could point to a genetic cause.

This approach is already producing results. Through participation in the Distant Cousin Project, researchers have identified mutations in two new genes: ITPR3 (CMT1J) and COQ7 (CMT-COQ7). These discoveries were only possible because families took the initiative to connect, share their information, and fuel discovery with their participation.

The study is limited to ten families and is currently enrolling. To be eligible, participants must meet these criteria:

- A confirmed clinical diagnosis of CMT
- A previous negative result from genetic testing
- A distant relative (such as a third or fourth cousin) with CMT and similar test results

All samples are analyzed using GENESIS, a collaborative genomic research platform and database developed by Dr. Züchner and his team. GENESIS is the same platform behind dozens of rare disease gene discoveries, including the groundbreaking CMT-SORD discovery.

This work is only possible because of the community's support, from the donor who made the project a reality to the families willing to help connect the dots. If you think you might qualify, or if you're ready to help advance CMT gene discovery, visit cmtausa.org/distantcousin to learn more about this CMTA Patients as Partners in Research opportunity.

SURGICAL EXPERTISE, COMMUNITY IMPACT:
DR. BOPHA CHREA NAMED 2025 CMTA-INC FELLOW

The CMTA-INC Alliance has named Bopha Chrea, MD, as the 2025 recipient of the Inherited Neuropathies Fellowship. A board-certified orthopedic surgeon at the University of Iowa, Dr. Chrea is the first surgeon to receive this long-running fellowship, a milestone that reflects the growing need for surgical care for people with CMT.

Foot and ankle deformities are among the most common and life-altering challenges in CMT, yet decisions around surgery often rely on limited evidence. Through her fellowship, Dr. Chrea is studying how foot structure, mobility, and function evolve over time in people with CMT. Her work aims to identify which patients benefit most from surgery and which could respond well to orthotic and bracing interventions.

> Through her fellowship, Dr. Chrea is studying how foot structure, mobility, and function evolve over time in people with CMT.

The CMTA-INC Alliance Fellowship program supports early-career physician-investigators through structured mentorship, clinical exposure, and research time. Directed by Professor Mary M. Reilly, MBE, MD, at University College London, and supported by an international selection panel including CMTA's Chief Research Officer Katherine Forsey, PhD, the program builds long-term research capacity to improve care across the CMT community.

CMTA's Dr. Katherine Forsey meets with Dr. Bopha Chrea, the 2025 CMTA-INC Inherited Neuropathy Fellow, at PNS 2025 to discuss Dr. Chrea's poster presentations on corrective foot and ankle surgery in CMT.

At this year's Peripheral Nerve Society (PNS) meeting, Dr. Chrea presented three posters on her work, one of many ways her fellowship is already translating into real-world impact. Her research isn't happening in isolation. It's grounded in care, shaped by lived experience, and already making a difference in people's lives.

Read the full story at cmtausa.org/news/cmta-inc-2025-fellowship

> "Dr. Chrea did the surgery on my foot and ankle. Awesome doc, surgeon, and person. Really cool that such an accomplished surgeon also really cares."
>
> —**Ronald Rush,** *CMT community member*

got grants?

Do you have a connection to a foundation, corporation, or other grant opportunity that might fit a CMTA program?

Tell me about it, and I'll take it from there!

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CONGRATULATIONS

Dr. Richard Finkel, CMTA-STAR Advisory Board member and a pediatric CMT specialist who sees patients at St. Jude Children's Research Hospital, was named to the TIME100 Health 2025 list.

He plays a key role in CMTA's Strategy To Accelerate Research (CMTA-STAR), sharing his gene therapy expertise to help advance treatment development for CMT. Dr. Finkel has recently received the CMTA Center of Excellence designation, and clinic info is coming soon.

Dr. Finkel was recognized by Time Magazine for leading the first documented in utero treatment for spinal muscular atrophy (SMA). This groundbreaking work could reshape how we treat neuromuscular diseases before birth.

INTERESTED IN STARTING A BRANCH IN YOUR AREA?

Contact CMTA Community Outreach Coordinator Riley Williams at riley@cmtausa.org

CMTA CENTERS OF EXCELLENCE

One of the many ways we implement OUR mission by sponsoring patient-focused, multi-disciplinary Centers of Excellence CMT clinics. World-renowned CMT care specialists and researchers staff each CMTA Center of Excellence, ensuring those living with CMT receive only the best comprehensive care for themselves and their loved ones. Newly added COEs are shaded.

CMTA Centers of Excellence affiliated with the Inherited Neuropathy Consortium (INC), marked below with an asterisk, go further by collecting and recording genetic, biological, and other data from individuals with CMT as part of CMTA-funded research. For more information, visit cmtausa.org/coe.

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The SAB provides scientific input for ongoing and proposed projects, the TEB evaluates the translational quality of ongoing and proposed projects, and the CEB provides expert guidance and support to CMTA's STAR Alliance Partners regarding clinical trial planning and delivery.

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WHAT IS **CMT?**

Named after the three doctors who first described it in 1886: Charcot (shar-coh), Marie, and Tooth, Charcot-Marie-Tooth disease (CMT) is an inheritable peripheral neuropathy that includes many motor and/or sensory neuropathies, axonopathies, myelinopathies, and neuronopathies.

Due to the effects on the nerves, people with CMT suffer lifelong progressive muscle weakness and atrophy of the arms and legs, and/or progressive sensory loss; and CMT can affect other parts of the body. There is no treatment or cure for this debilitating and often overlooked disease.

CMT leads to problems with balance, walking, and hand use. CMT can cause foot drop, chronic nerve pain, chronic muscle and joint pain, abnormal reflexes, fatigue, tremors, sleep apnea, hearing loss, breathing difficulties, and much more.

Early signs of CMT can be toe-walking, especially in children; frequent trips and falls, frequent ankle sprains, and difficulty with handwriting, tying shoes, or buttoning a shirt.

Visit CMTA's What is CMT webpage today: cmtausa.org/cmt

The Charcot-Marie-Tooth Association is a community-led, community-driven 501(c)(3) nonprofit organization with a mission to support the development of new treatments for CMT, to improve the quality of life for people with CMT, and, ultimately, to find a cure. As the leading global philanthropic funder of CMT research, CMTA unites the community with clinicians and industry experts to accelerate the advancement of treatments, with investments of nearly $30 million since 2008.

Walk 4 CMT and Cycle 4 CMT are annual community-based nationwide fundraising events for the Charcot-Marie-Tooth Association that have continued for more than a decade. These community events bring together the CMT community to raise awareness about Charcot-Marie-Tooth disease and generate funds for research and patient programs.

Learn more and get involved today at **cmtausa.org/4cmt**