Partnership and progress for a rare disease community

How Inspire and HNF collaborated to grow the Charcot-Marie-Tooth community and accelerate medical research

by Judy Chandler, MPH, CHES, Partnerships Director, Inspire, Allison Moore, Foundation (HNF), Joy Aldrich, Advocacy Director, HNF, and Courtney Hollett, Executive Director, HNF



Supporting rare disease patients and caregivers

Inspire partners with leading non-profit patient advocacy organizations to provide a safe, private space of connection and support for about 350,000 rare disease patients and caregivers across condition-specific online support communities.

Inspire and non-profit organizations partner to:

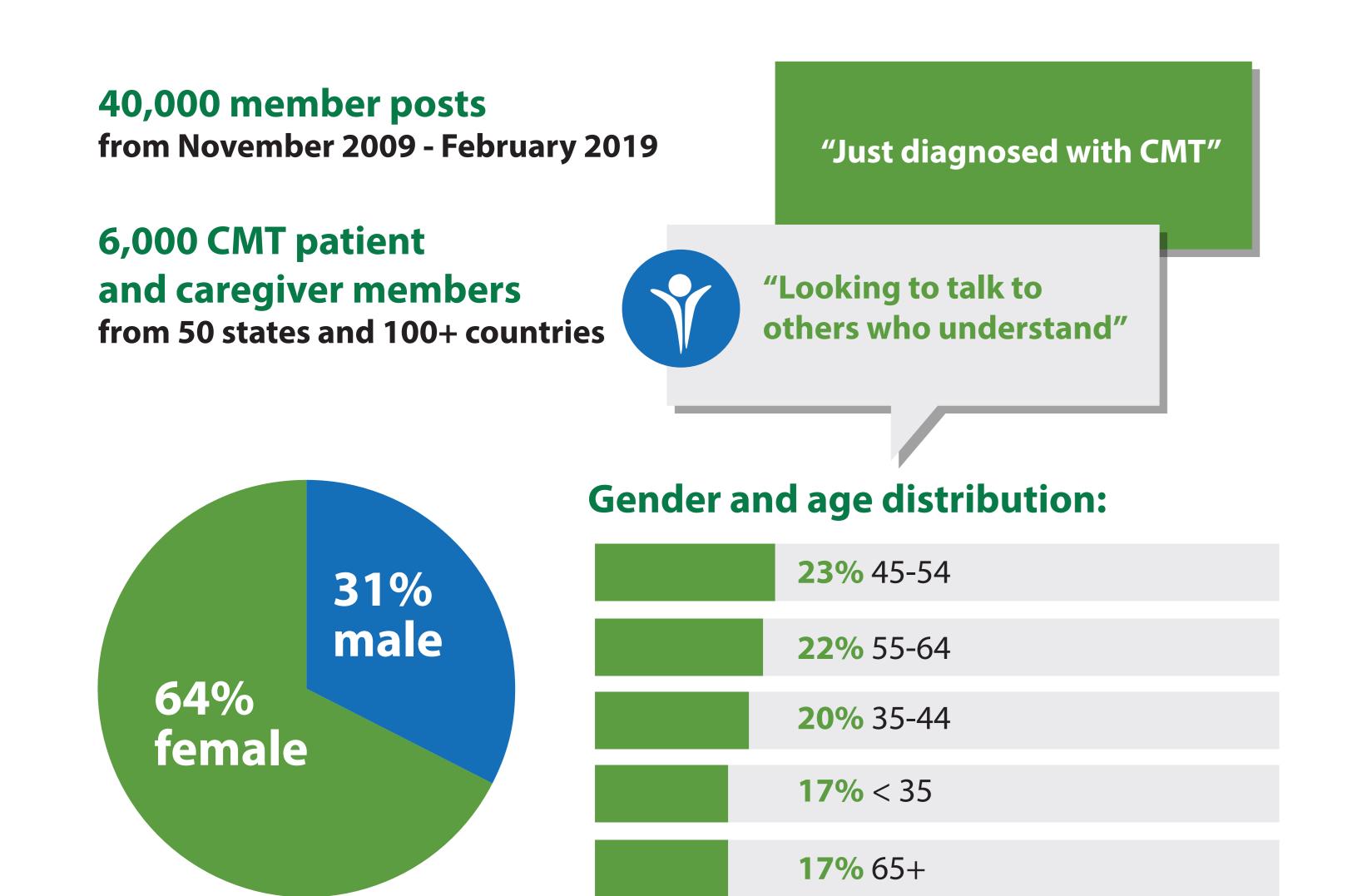
- Connect patients and caregivers with others who understand what they are going through
- Grow and engage with rare disease patients and caregivers
- Collaborate on data and research to help partner organizations better understand the needs of patients and caregivers in their community
- Share events, resources, and other opportunities with community members



Hereditary Neuropathy Foundation

Hereditary Neuropathy Foundation (HNF) is a rare disease advocacy group focused on supporting patients and families with critical information to improve patients and families quality of life and funds research for Charcot-Marie-Tooth (CMT), a progressive neurological disease. HNF works closely with Inspire and industry partners to identify gaps that need to be addressed to advance research and clinical trial development.

Inspire partnered with the Hereditary Neuropathy Foundation in 2009 to launch the CMT online support community



What CMT support community members are talking about



ymptoms



HNF news

9% posts



Assistive devices and technologies



Medications



9% posts



erapies
1% posts

How CMT changed my life



Newly diagnosed
10% posts



8% posts

8% posts

CMT community member feedback

"The people on this site are giving you first hand experiences and you will find that even though we all have the same problem each one is different and yet you still will find yourself saying...hey, that's exactly what happened to me. Eventually you won't feel so alone."

"I can't express my gratitude for the support and help from everyone who has responded to my posts."

Inspire/HNF partnership timeline

2009: CMT support community on Inspire launched

2011: HNF launched blog to share HNF news

2013: CMT community reached 1,000 members

2015: Joy Aldrich becomes HNF's Advocacy Director to focus on strengthening the CMT support community

2016: Inspire's CMT healthcare provider survey helped HNF develop an HCP directory

2016: Brian Loew, CEO presented at HNF's Patient-Centered CMT Summit in NYC

2017: Inspire's research team co-presented with HNF's CEO Allison Moore at the PMRG conference

2017: Inspire's Communications Director, John Novack, presented at HNF's Pain Summit in Cambridge, MA

2017: Joy Aldrich presented during a webinar to other Inspire partners on using social media to drive traffic to the HNF support community

2018: CMT community reached 5,000 members

2018: Inspire worked with HNF to distribute "True Reply," a voice-activated survey, to capture patient reported outcomes

2018: HNF hosted EL-PFDD meeting which included presentation of True Reply survey data

2019: Inspire sponsored HNF's screening "Take A Look At This Heart," a documentary about love, dating, and disability

How HNF leveraged its relationship with Inspire

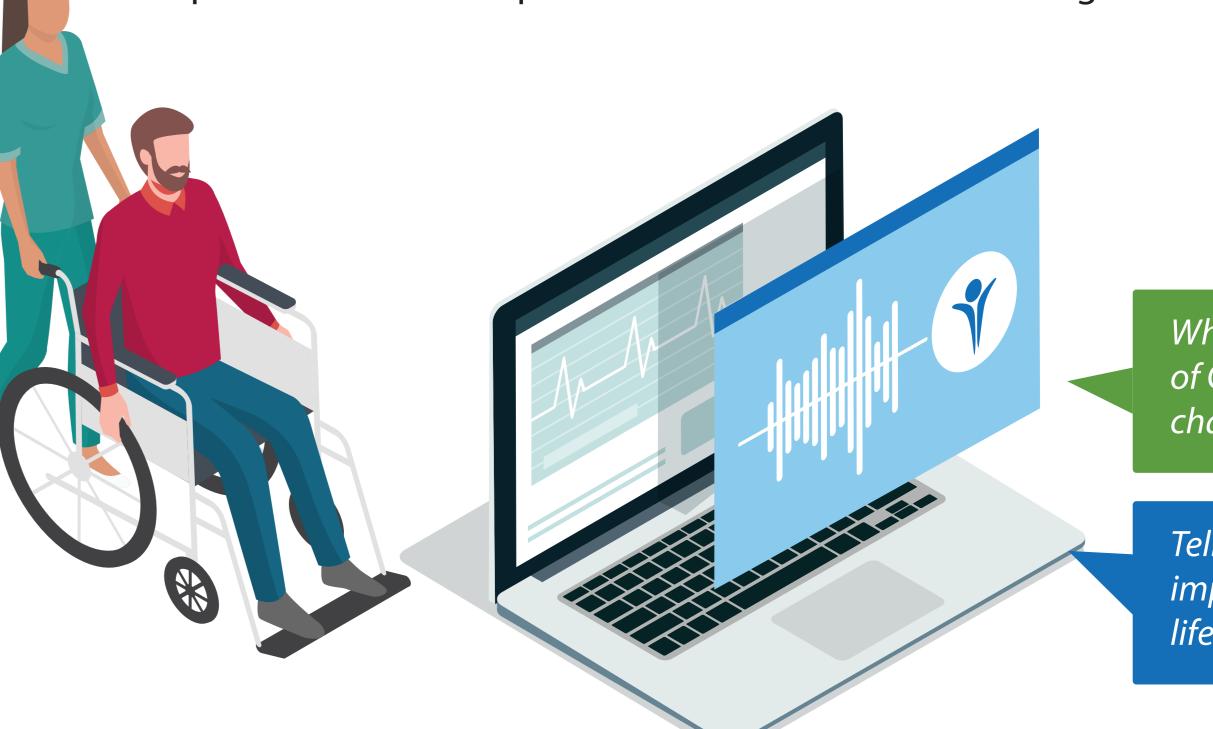
Identifying key trends in the online community

HNF has used data and discussions from the online community to identify trends, themes, and gaps that need to be addressed among the CMT patient community. For example, HNF took on several initiatives to better understand pain - a recurring topic - including the "Patient-Centered CMT and HNPP Pain Summit" with partial support from PCORI's Eugene Washington PCORI Engagement Award and a PRO study with an industry partner. Inspire member discussions helped researchers understand pain as a major CMT symptom as well as the type of pain and how to develop related questions and answers that would be most relevant to the CMT community. **Today, pain is recognized as a very important endpoint in CMT treatment and outcome measures in clinical trials.**

HNF also used data and content from Inspire to develop questions around the psychosocial components of CMT for its Natural History Study, **Externally-led Patient Focused Drug Development (EL-PFDD) meeting**, and Patient-Centered Behavioral Health Summit.

Capturing the CMT patient voice

The Externally-led Patient Focused Drug Development (EL-PFDD) meeting offered the opportunity for patients and families to inform the FDA, drug developers and other key stakeholders, on the true burdens of living with CMT and how patients view the benefits and risks of CMT treatments. In preparation for the meeting, HNF collaborated with Inspire to use voice-activated technology (VAT) to capture CMT community members' own words. VAT survey questions included: "What is the one symptom of CMT that you would change if you could?" and "Tell us how this symptom impacts your quality of life." A representative sample of the data was presented at the EL-PFDD meeting in a video montage.



What is the one symptom of CMT that you would change if you could?

Tell us how this symptom impacts your quality of

Recruiting volunteers

Through posts, HNF cultivated Inspire members who have become volunteers, including an HNF board member who has been very active in fundraising. His sister recently joined HNF's Board and raised \$60,000+ for CMT research.

Partnership and progress for a rare disease community

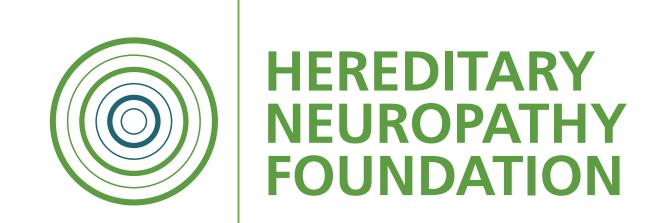
How Inspire and the Hereditary Neuropathy Foundation collaborated to grow the CMT patient community and accelerate research

By Judy Chandler, MPH, CHES, Partnerships Director, Inspire; Allison Moore, Founder/CEO, Hereditary Neuropathy Foundation (HNF); Joy Aldrich, Advocacy Director, HNF; Courtney Hollett, Executive Director, HNF



Inspire partners with leading non-profit advocacy organizations to provide a safe, private space of connection and support for about 350,000 rare disease patients and caregivers across condition-specific online support communities worldwide. Inspire and non-profit advocacy organizations partner to:

- Connect patients and caregivers with others who understand what they are going through
- Grow and engage with rare disease patients and caregivers
- Collaborate on data and research to help partner organizations better understand the needs of patients and caregivers in their
- community as well as addressing gaps that may be hindering patient care and therapy development • Share events, resources, and other opportunities with community members
- Incorporate expert advice with resources to support patients and caregivers



The Hereditary Neuropathy Foundation (HNF) is a leading rare disease advocacy and research non-profit with a mission 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. 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. HNF works closely with Inspire and other stakeholders to identify gaps that need to be addressed to advance research, create positive outcomes and treatments for CMT.

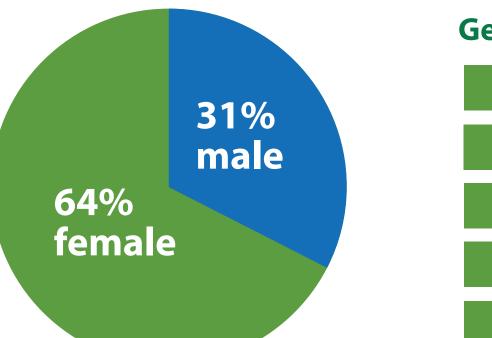
Background

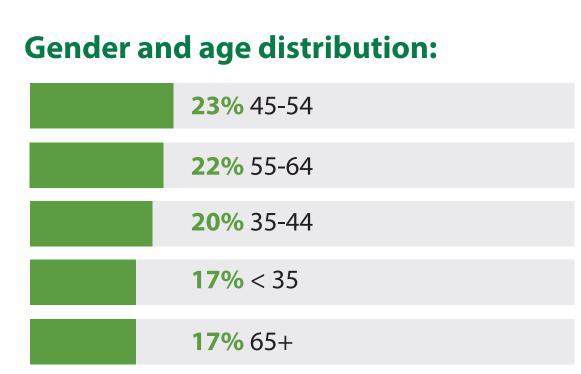
What is Charcot-Marie Tooth?

CMT is the most common inherited peripheral neuropathy affecting 1 in 2,500 patients. Currently there is no cure for CMT. Patients suffer from a variety of classic symptoms, including foot and hand deformities, pain, fatigue, foot drop, and muscle wasting and weakness. In some patients these pathologies can be debilitating, causing serious mobility issues, and in some cases, early death.

Inspire partnered with the Hereditary Neuropathy Foundation in 2009 to launch the CMT online support community

6,000 CMT patient and caregiver members from 50 states and 100+ countries





40,000 member posts from November 2009 - February 2019

What CMT support community members are talking about











Medications
9% posts

Exercise and therapies
11% posts

How CMT changed my life
8% posts





Newly diagnosed
10% posts

Research
8% posts



March 2009 - May 2019: Approximately 1,860 CMT Community members mentioned pain in 4,250 posts

Subject: Tired of the pain!!



Member post: "I just don't know how to deal with the pain...I don't tell my family because I feel like I'm always complaining...But the constant pain is killing me."



Member reply: "I was told that the pain was in my head until someone finally took me seriously and I had nerve conduction testing, and some other testing done, which really showed it is 'not in my head'. I think that is a big issue with doctors and nurses who do not know much about CMT."

Methods and Results

Inspire and HNF Collaborate to Support **CMT Research and Educational Initiatives**

Using social media networks to capture the patient voice from organic patient and caregiver discussions, or user-generated-content (UGC), is a new concept that is gaining recognition among scientists, industry, and regulators. These patient-reported outcome measures (PROs) offer real world data (RWD) that HNF incorporated into research initiatives and clinical trial design. (1)

Incorporating Pain as a Novel Research and Clinical Outcome Measure

From 2009-2017, approximately 1,100 CMT Community members mentioned pain 2,600+ times. Using pain-related conversations and resulting data from the CMT support community, HNF developed initiatives to understand the potential link between pain and CMT, including a patient-centered pain summit and a patient-reported IRB outcomes study through their patient registry, Global Registry for Inherited Neuropathies (GRIN). Pain is now a clinically relevant endpoint in CMT treatment and key outcome measure in CMT clinical trials.

Understanding Psycho-Social Components of CMT

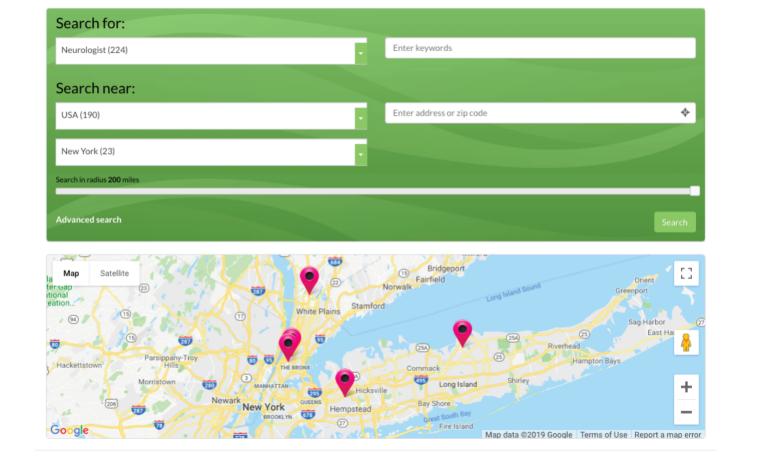
HNF leveraged data and content from the CMT support community about the psycho-social components of the disease to develop questions for a Natural History Study and to plan an FDA Externally-led Patient Focused Drug Development (EL-PFDD) Meeting and a Patient-Centered Behavioral Health Summit.

Capturing the Patient Voice

HNF and Inspire collaborated to use voice-activated technology to capture the CMT patient experience. Results were shared at the EL-PFDD Meeting to help HNF describe the true burden of living with CMT and how patients view the risks and benefits of CMT treatments to inform the FDA, drug developers, and other key stakeholders.

Developed CMT Healthcare Provider Directory

In 2016, Inspire collaborated with HNF to design and analyze a healthcare provider (HCP) survey in the CMT support community. The survey received 225 responses and resulted in a national directory now over 1,500 providers across many disciplines who understand how to manage patients with CMT.



Inspire/HNF Partnership Timeline

2009: Launched CMT Support Community on Inspire; patient discussions about pain indicate this is a serious issue for those with CMT







2016: Brian Loew, CEO presented at HNF's Patient-Centered CMT **Summit in NYC**

2017: Inspire's research team co-presented with HNF's CEO Allison **Moore at the PMRG conference**

2017: HNF launched a collaborative Patient Reported Outcomes (PRO) study with one of their industry partners to gain additional insight into the burden of the disease from the patient perspective as well as to help develop the phase II clinical trial design

2017: Inspire's Communications Director, John Novack, presented at HNF's Pain Summit in Cambridge, MA

2017: Joy Aldrich presented during a webinar to other Inspire partners on using social media to drive traffic to the HNF support community

2018: CMT community reached 5,000 members

2018: Inspire worked with HNF to distribute "True Reply," a voiceactivated survey, to capture patient reported outcomes

2018: HNF hosted Externally-Led Patient-Focused Drug Development Meeting which included presentation of True Reply survey data

2019: Inspire and HNF present poster at Rare Disease Day at the **National Institutes of Health**

2019: Inspire sponsored HNF's screening "Take A Look At This Heart," a documentary about love, dating, and disability

Inspire and HNF were uniquely positioned to create a successful, innovative partnership beyond the critical work of peer-to-peer support for patients and families affected by CMT. By leveraging PRO data as well as Inspire's research services, HNF developed a number of patient-driven research and educational initiatives that have improved CMT patients' quality of life and expanded the scientific understanding of this rare disease. As a result of several of these efforts driven by RWD, pain is now a clinically relevant endpoint in CMT treatment and key outcome measure in CMT clinical trials.

Subject: Excited to be here..



Member post: "I just want to say that I am thrilled to have found this web site...I have felt very alone (with the CMT, not in life). Over the next few days I will be reading through the information here and look forward to reading your stories." -CMT Community Member

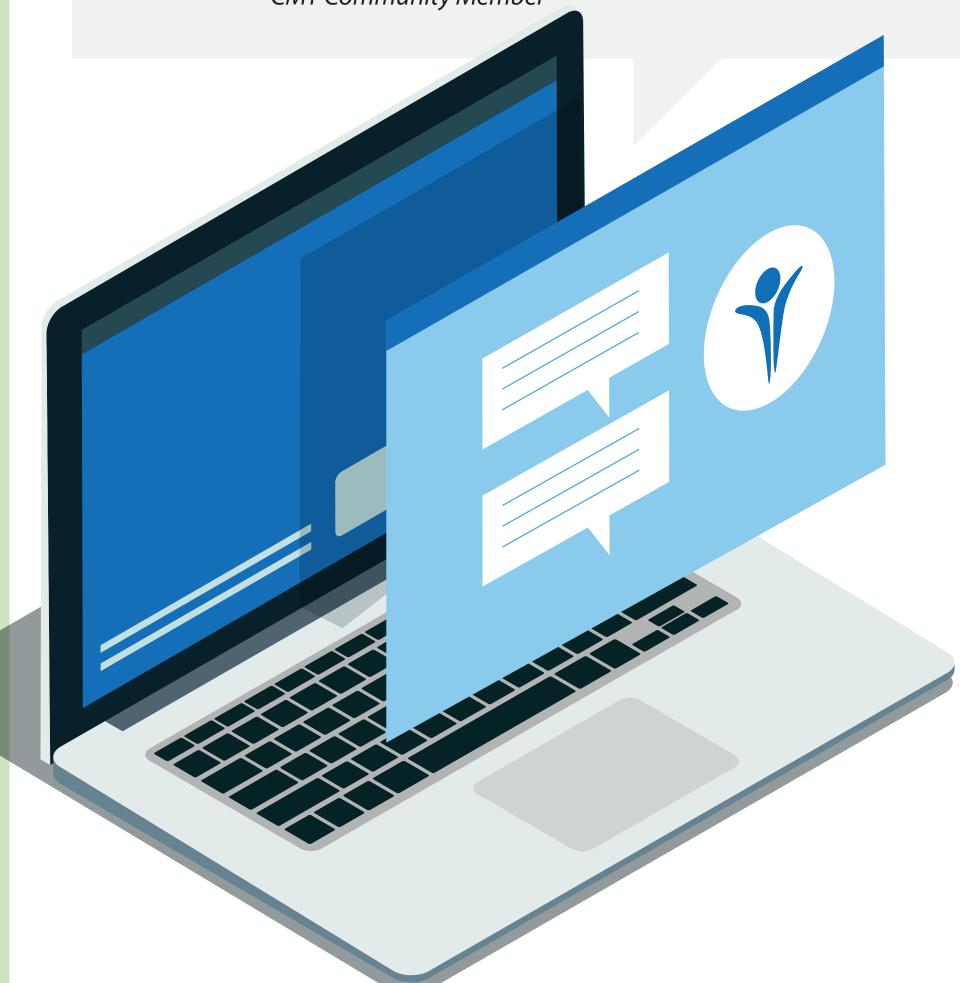


Member reply: "The people on this site are giving you first hand experiences and you will find that even though we all have the same problem each one is different and yet you still find yourself saying...hey, that's exactly what happened to me. Eventually you won't feel so alone." -CMT Community Member

Subject: OUCH!



Member post: "Man, I am hurting today, and it's getting me down...I hate days like this. I'm sore but have no pain killers left and can't get Doc appointment for weeks. I feel like crying cause I'm housebound due to the severity of my condition and where I live. I'm only 20. I should be Working. Partying. Socializing. I should be doing anything other than lying in bed, drinking tea and fearing tomorrow." -CMT Community Member



1. McDonald L, Malcolm B, Ramagopalan S, Syrad H. Real-world data and the patient perspective: the PROmise of social media? BMC Medicine. 2019;17(1):11.