

Eugene Washington PCORI Engagement Award Lay Conference Summary

Hereditary Neuropathy Foundation Patient-Centered CMT/HNPP Pain Summit, November 3, 2017

Background

On November 3, 2017, nearly 100 participants gathered at the Samberg Conference Center on the Massachusetts Institute of Technology (MIT) campus in Cambridge, MA for HNF's Patient-Centered Charcot-Marie-Tooth (CMT) / Hereditary Neuropathy Pressure Palsies (HNPP) Pain Summit. People with hereditary neuropathies and their family members, caregivers, clinicians, researchers, funding agencies, payors, leading pain experts and pharma industry came together to get an understanding of chronic pain in the CMT/HNPP community, including the impact of chronic pain on patients' quality of life.

Conference Summary

The prevalence of pain in our community became apparent during HNF's 2016 Patient-Centered CMT Summit. To validate this issue, HNF used its Global Registry for Inherited Neuropathies (GRIN) and the Inspire CMT Online Support and Discussion Community to poll patients about the one word they would use to describe their disease: PAIN was the number one response. As a result, it was decided that the 2017 Summit would focus on addressing the gaps in pain management for hereditary neuropathy patients. Patients were involved throughout the program's development, from serving on the planning committee to speaking and even facilitating in interactive workshops and polling that contributed to presentations. Patients from other chronic pain diseases were also featured to share learnings from other disease groups as well. Having patients interact with other chronic pain patients from within/outside of their disease community promoted a sense of empowerment for those attending and gave attendees insights into new approaches for managing their own personal disease experience by learning about the experiences of others.

The Summit began with a welcome from HNF Founder and CEO, Allison Moore, and a report from Robert Moore (Allison's husband) on a voice survey about pain that was conducted by using True Reply phone polling. This unique survey tool gave patients the opportunity to have their voice heard by simply calling the toll-free number and answering a few questions when prompted. Data regarding descriptions of pain symptoms (stabbing, shooting, aching, pressure), descriptions of how the pain impacts quality of life ("My pain keeps me from doing the things I love"), and currently used methods of pain management (fentanyl patch, gabapentin, marijuana, yoga, mindfulness) was recorded.

Pamela Katz Ressler, RN, MS, HNB-BC and founder of Stress Resources in Concord, MA, then presented the Keynote Address, "Chronic Pain and the Importance of Social Networks," describing how pain can cause isolation and loneliness, and that online communities (including HNF's Inspire community with 4,800 CMT/HNPP patients and caregivers) help to facilitate gathering and sharing information, as well as connecting with others, which reduces suffering and isolation.

John Novack, Director of Communications at Inspire, conducted a live-time text chronic pain poll with Summit participants to further explore chronic pain within the CMT/HNPP community. The questions were drawn from a pain assessment tool used by the VA/military for chronic pain patients. We learned:

- 64% of audience had chronic pain that was a combination of neuropathic, muscle and skeletal/bone pain
- 61% of audience had a combination of achy/sore, sharp/stabbing, and tingling/electrical pain
- The majority of respondents said their chronic pain rated 4-6 on a pain scale (with 10 being worst/completely debilitating pain)
- Most reported moderate to severe interference with general activity, mood, and stress levels.
- Most reported moderate to severe impacts on sleep as well as both personal and professional life interactions.

Clifford J. Woolf, MD, PhD, an internationally-renown pain researcher as well as a Harvard professor and researcher at Children's Hospital Boston, gave a preview on his new, potentially groundbreaking, research approach for studying painful chemotherapy-induced peripheral neuropathies and the implications his learnings might have for other types of painful neuropathies.

The rest day included:

- Dr. Florian Thomas, director of HNF's Center of Excellence (COE) at Hackensack University Medical Center, presented an overview: "CMT/HNPP 101."
- Dr. Jafar Kafaie, director of HNF's COE at St. Louis University, gave an overview of the types of chronic pain and the current standards of care for treating neuropathic and musculoskeletal pain.
- Dr. James Nussbaum, clinical and research director of ProHealth & Fitness PT OT, presented his case for the importance of physical and occupational therapy for CMT/HNPP patients. He also discussed the HNF-supported clinical trial of the Alter G anti-gravity treadmill. (Note: this is a research study that was developed as a result of HNF's 2016 Patient-Centered Summit)
- Mitch Warner, CPO, founder of Ortho Rehab Designs, discussed how ankle-foot-orthosis can treat pain by providing better alignment and a more natural gate.
- A panel including Bob Twillman, PhD (executive director of the Academy of Integrative Pain Management-AIPM), Pamela Katz Ressler (keynote speaker) and Kristin Gelzinis (HNF patient advocate) discussed the current national focus on the "Opioid Crisis," its effect on chronic pain patients, and the possible misunderstanding and misrepresentation of the cause of the "crisis." While new prescribing guidelines have been issued, some physicians are opting out of prescribing pain medications and some insurers are limiting access to safer forms of opioids because they are more expensive. Meanwhile, there is little movement for insurers to cover non-pharmacological treatments for pain. This led to a conversation about the practice of Mindfulness-Based Stress Reduction (MBSR) and how powerful it has been in helping some CMT patients reduce the amount of pain medication they require. Dr. Twillman then continued the topic over lunch with an advocacy update from his perspective as head of the AIPM professional society.
- Chris Dito, a CMT patient and a physical trainer specializing in human biomechanics with a focus on posture and breathing, also spoke during lunch, sharing his story of getting himself out of a wheelchair and into the gym. The impact of a CMT patient demonstrating a remarkable physical transformation was extremely powerful for the audience.
- National Organization for Rare Diseases (NORD) representative Allison Seebald presented an overview of the robust natural history study to support the second most common inherited neuropathy, HNPP, as well as the rarer types for which there are major gaps of information.
- Dr. Mamatha Pasnoor, from HNF's COE at the University of Kansas Medical Center, then presented her PCORI-funded study of four common drugs used to treat pain in idiopathic neuropathy patients and how the study incorporated patient-reported outcome measures. Dr. Pasnoor suggested that this research data/conclusion/outcomes could translate to support future studies in CMT/HNPP to help manage chronic pain.

Summit participants divided into two highly interactive workshops where they learned from each other as well as from the facilitators:

- **"The CMT/HNPP Patient Experience Workshop"**--patients developed case studies for medical school students about CMT/HNPP with the help of facilitator Libby Bradshaw, DO, MS-academic director of the Pain Research, Education, and Policy Program at Tufts Medical School
- **"Communicating Your Pain Workshop"**-- Gwenn Herman, LCSW-C, DCSW, clinical director and founder of Pain Connection (a U.S. Pain Foundation program), taught how to communicate with their healthcare providers by using a "Treatment Tree Plan" with seven branches (representing aspects of daily living): physical, cognitive/emotional, spiritual, social, career, leisure/hobbies and opportunities.

The Summit then wrapped up with Allison Moore reported on current HNF initiatives, while Rene Goedkoop, MD (Pharnext), Kenneth Attie, MD (Acceleron), Thomas Wessel, MD, PhD (Flex Pharma),

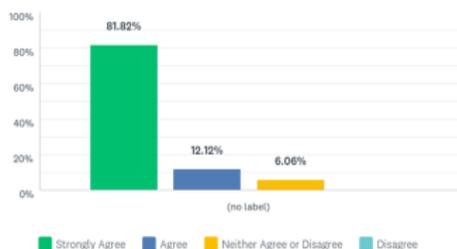
and Sean Ekins, MSc, PhD, DSc (Collaborations Pharma) presented their respective clinical trials for CMT and research for CMT/HNPP.

Key Findings

This Summit represents a starting point for documenting and addressing the chronic pain needs of the CMT/HNPP community. Heretofore, there has been little to no acknowledgement of the chronic pain experienced within this community: it is not being researched, there are no outcomes measures included in current clinical trials, there are no standards of care. Not only did the Summit document such findings as 64% of attendees experiencing chronic pain that is a combination of different types of pain, candid Workshop conversations and Q&A exchanges revealed that patients felt minimized, marginalized, and even bullied by their health care professionals in trying to find resources to manage their pain, talked about challenges with misdiagnosis, and expressed dealing with physical and emotional feelings that left them “crushed” by “horrendous” pain. By using polling before, during, and after the event, and encouraging the patients to engage with the meeting’s activities as much as possible, the input and data we were able to collect was critically insightful to begin to quantify the significant scope of this problem so we can begin to encourage community stakeholders to champion and address this problem. Already, those pharma with clinical trials in process have re-evaluated how to incorporate pain into their quality of life/outcomes measures. It is our hope that our post-Summit reporting will galvanize the community of researchers, clinicians, pharma and payors to take a new and dedicated look at the dynamics of pain this patient community is dealing with. The Summit enabled the documentation of the gaps and disparities in standards of care, available treatments, and the challenges in access to care for pain management. Attendees expressed in post-Summit evaluations that they felt the Summit’s information was invaluable and that they felt encourage to participate in future research to help themselves and others:

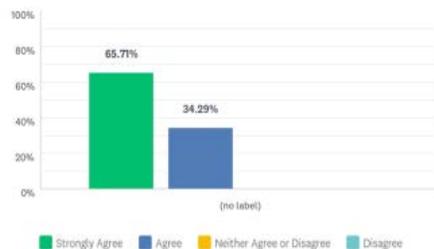
Q10: Would you participate in Patient-Centered Research to advance therapies for CMT/HNPP?

Answered: 33 Skipped: 2



Q8: Do you feel the information presented was relevant and valuable to you?

Answered: 35 Skipped: 0



Dissemination Plans

As noted, Summit findings are already informing clinical trials currently in development. HNF plans to approach the leading professional societies representing the healthcare providers in this space (AAN, AANEM, Child Neurology Foundation, pain societies) to share the outcomes from this meeting and--at a minimum--encourage that CMT/HNPP chronic pain be considered for their upcoming meeting agendas in 2018. In the Spring of 2018, Neurology Reviews will again be featuring a Summit summary article in its Rare Disease Report special edition, which was one of the highest viewed articles from its 2017 edition. HNF will continue to seek other opportunities to publish a similar summary article in other patient-facing and professional-facing media outlets, in professional journals, as well as in poster abstracts at professionally led meetings in 2018. The Peripheral Nerve Society has already invited HNF to submit an overview article for possible publication. In addition, the Summit findings will be a significant point of discussion during our September 28, 2018 externally-led Patient Focused Drug Development meeting, which HNF has received the go-ahead from the FDA to host next year. Including chronic pain treatments at the PFDD meeting and patient testimonies as an example of the treatment gaps experienced by this community will greatly benefit from the data and insights we were able to gather during this 2017 Summit.

Appendix

- Conference Program PDF
- Results of True Reply Telephone Survey
 - Raw data analysis report
 - PowerPoint presentation of survey data from the Summit
- Case Study Development Workshop Summary Write-Up
- Communicating Your Pain Workshop Summary
 - Treatment Tree provided to attendees
 - Summary Write-up
- PowerPoint of Summit attendee evaluation responses to date