

FPN member NEWS

FALL 2023



From the Executive Director

Dear FPN Friend:

Every day, a new patient is diagnosed with peripheral neuropathy (PN). With an estimated 30 million Americans alone that have PN, and countless more around the world, it's a disease that is ever expanding. Patients are left asking more questions than are answered. What is PN? What can I do to make myself feel better? Can I ever get rid of it? Will my children get it too?

As most of you know, PN is a complicated disease

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Our Success on the Hill

Since 2019, we have been actively engaged in a campaign to increase federal funding for peripheral neuropathy research. We had an extraordinary breakthrough in fiscal year 2021, when Congress included "peripheral neuropathy" for the first time as an eligible condition for research funding from the Department of Defense's Peer Reviewed Medical Research Program (PRMRP). Congress has renewed this designation for fiscal years 2022, 2023, and once again in 2024. For the upcoming year, the Congressional Committee appropriated \$370 million in total for the entire PRMRP, and PN is one of 47 conditions that are included as eligible for research from the PRMRP.

Building on this success, FPN has turned its attention to deepening its relationship with the National

Institutes of Health (NIH), the world's largest funding agency for biomedical research. We are pleased to report that, thanks to our advocacy efforts, the Senate Committee on Appropriations took an important step in recognizing the importance of peripheral neuropathy research and the need for the NIH to take a more coordinated and focused approach on the condition. On July 27, 2023, the Committee approved its version of the fiscal year 2024 Labor-Health and Human Services-Education Appropriations Act and included the language about PN in the report that accompanies the bill.

We are confident that both accomplishments will help FPN strengthen its relationships with these key funding agencies and ultimately lead to more groundbreaking research on this debilitating condition.

– no one person experiences neuropathy the same as another. Similarly, no one therapy works across the patient population. Some symptoms are easily manageable, and others are not; and in the times when worsening symptoms are upon you, you are left to feel hopeless and alone.

At the Foundation for Peripheral Neuropathy (FPN), we empower patients to take control of their lives by sharing valuable resources with you and supplying a network to lean on. We want you to know that someone is out there fighting for you. We are advocating for you. We are funding the research that will provide these much-needed answers. We are ensuring that PN gets the attention it deserves.

The hopeful truth is that we're getting closer and closer to finding more and improved therapies. And as more generous people like you join our PN community and support our cause, we'll be that much closer to advancing new discoveries and finding those elusive cures.

I believe this newsletter will offer you the hope you rely on us for. We're making big waves, and I'm excited for the future. Happy reading and thank you again for your involvement with FPN!

Be well,

Lindsay Colbert



PN HOLIDAY GIFTS

By Rochelle Friedman, MD

When I think about holiday gifts for my youngest daughter who has autoimmune small fiber polyneuropathy, my mind goes in two directions: gifts that provide bodily comfort or that nurture the spirit.

In the first category are foot massagers, foot soaking basins, peppermint, or chamomile oil to put in the soaking water, shea butter intensive foot cream or balm, sleep socks that are soft and cozy, cabin sock slippers and sleep gloves for moisturizing and warmth.

To soothe the spirit, I would choose "The Pain Management Workbook" by Rachel Zoffness, MS, or "Mindfulness and Meditation for Pain Relief" by Jon Kabat-Zinn.

Some gifts feed both body and spirit by providing a service with a touch of whimsy. In this category are Swedish designed "Happy Socks," which are colorful and wonderfully designed. I also recommend animal hot or cold-water bottles, which are furry, charming, and irresistible.

Lastly, a donation to the Foundation for Peripheral Neuropathy in honor of someone you care about provides those who suffer from peripheral neuropathy what they want most, which is HOPE.

I hope that this inspires many of you to do some fun and helpful shopping this holiday season!

Faces of PN:

Will Schwalbe

AN AUTHOR, FPN SUPPORTER AND PN PATIENT SHARES HIS STORY



A lot of our friendship now is based on gratitude.

In May, Will Schwalbe and his friend, Chris Maxey, were guests for our webinar on the importance of your support network. Will's latest book, "We Should Not Be Friends," chronicles his longtime friendship with Maxey, and how they have helped each other weather life's challenges. After the program, the Foundation asked Will about the experience.

FPN: What did it mean to you to do the webinar?

Will: I was thrilled to do the webinar for FPN because I hugely admire the organization. Especially the splendid work it does getting the serious public attention and research peripheral neuropathy needs and deserves—but also providing hope and inspiration by highlighting the lives of people living with neuropathy and enjoying their lives. That's very much what I wanted to do in the section of the book where I chronicle my journey with small fiber neuropathy (SFN). I also mention in the book a support group that was immensely helpful to me — I found that thanks to FPN. I've been to FPN live conferences and rely on the website for up-to-date information and helpful strategies. So, I was excited to do a webinar for the Foundation about the book with my dear friend Maxey. Maxey and I were completely different from each other in college, but we were thrown together into a secret society and wound up becoming friends and staying friends for the last 40 years and counting. It sounds odd, but in a way PN is like its own not-so-little not-so-secret society. Whenever I meet someone with PN, we have one thing in common — and while we may be vastly different, we often discover that we can and should be friends.

FPN: How did writing the book help you in terms of mental health and dealing with your condition?

Will: I don't think that writing helps me in terms of mental health, but it does help me clarify my thoughts. But I do feel I can achieve a kind of honesty when I write, that is harder when I talk about my SFN. When friends ask how I'm feeling, it comes out of a place of kindness and concern, so I sometimes find myself saying I'm feeling better than I am because I don't want to disappoint them. Or, conversely, when I'm having a particularly bad stretch, it's easy to forget that I recently had better

days. Writing is something you do over time, and you can sit with the words you wrote and revisit them to make sure they reflect an overall truth (if that's what you want) and not a flash of feeling or the desire to tell others what you think they want to hear. When I wrote "We Should Not Be Friends," it gave me the opportunity to reflect on my SFN and describe it as accurately and truthfully as I could, borrowing passages from an article I had written about SFN for *The Washington Post*.

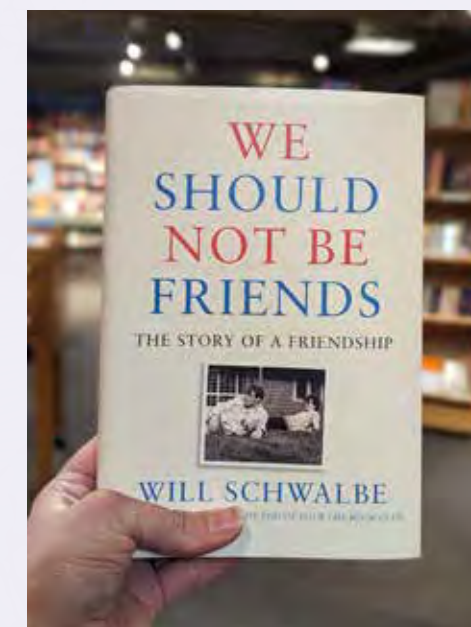
FPN: Why was it important to you to share your journey with other PN patients?

Will: I think that there can be a lot of loneliness and isolation with PN because it's a chronic, invisible condition. I think many of us get discouraged because we are constantly being told, "Well, you don't look sick," or are given incredibly unhelpful advice. For the longest time I wanted to get a t-shirt that said, "Yes, I have tried yoga!" For those of us who have had to make significant changes to our lives (having to leave a job; not being able to take part in activities we love; needing to cancel events at the last minute; or all of the above) it can be especially frustrating when others don't understand. So, I think it's important that we share our stories with each other and give each other

writings and accounts we can share with people in our lives to give them a sense of what PN (in my case SFN) can be like. That said, I also wanted to share my journey in the context of my life as a whole—which is active, happy, and hopeful! My PN is one part of me, but just one part. I'm a writer, a book editor, a foodie, a husband, a sibling, a godparent, a reader, a friend, and all sorts of other things. We all get something, and we don't get to choose what we get. Part

of the journey I wanted to share was helping my friend Maxey through his challenges and allowing him to help me. I wanted to share a journey where PN was part of the story but far, far from the whole of it. That's part of the point of the whole book:

We all contain multitudes.



Looking for something new to read? Try out one of these books:



No Time Like the Future
Michael J. Fox

Actor and philanthropist, Michael J. Fox, shares personal stories and observations about illness and health, aging, the strength of

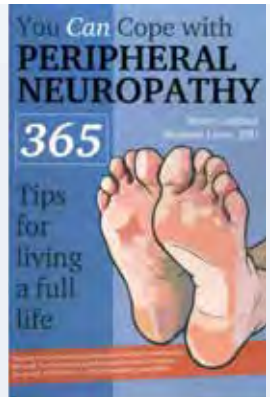
family and friends and how our perceptions about time affect the way we approach mortality.



The Invisible Kingdom: Reimagining Chronic Illness
Meghan O'Rourke

“[O'Rourke] gives shape and color to the invisible life of patients whom society has failed. She offers

hope for patient-driven change. Most important, she provides an account that many will be able to relate to—a ray of light into those isolated cocoons of darkness that, at one time or another, may afflict us all.” – *The Wall Street Journal*



You Can Cope with Peripheral Neuropathy: 365 Tips for Living a Full Life
Norman Latov, MD, PhD

“You Can Cope with Peripheral Neuropathy” is a collection of tips,

techniques and life-task shortcuts that can help those living with this painful condition. It's also a good resource for families, caregivers and health-care providers.

PNS Annual Meeting & CMT Trials

By Simone Thomas, MS, Head Research Coordinator at Johns Hopkins University

FPN's annual attendance at the 2023 Peripheral Nerve Society (PNS) meeting in Copenhagen, Denmark, was an opportunity to network with leading neurologists and PN researchers, and learn about important updates in the field.

One common theme from this year's meeting was highlighting some new research on Charcot-Marie-Tooth (CMT) – a progressive nerve disease that affects 1 in 2,500 people – and the forthcoming research trials.

The INSPIRE Trial

Several years back, a new form of Charcot-Marie-Tooth disease Type 2 (CMT2) was discovered. This form is caused by sorbitol dehydrogenase (SORD) deficiency. SORD is an enzyme involved in the carbohydrate metabolism, converting sorbitol – the sugar alcohol form of glucose – into fructose. Mutations in the SORD gene result in loss of the enzyme and, consequently, intracellular sorbitol accumulates resulting in distal motor weakness and sensory nerve damage, typical for CMT. It is estimated that about 1 in 100,000 Americans have SORD deficiency, and pre-clinical research suggests that the condition is treatable.

The Inherited Neuropathy Consortia (INC) is composed of a group of academic centers focusing on investigating different types of CMT. Patients regularly seen at one of the INC sites were asked to participate in a natural history study that documents disease progression over 10 years to learn more about the different subtypes of CMT. In addition, they also collected blood from patients who looked like they had CMT, but no pathogenic variant had been found in their genetic testing. That protocol has resulted in the discovery of many additional genes causing CMT, including SORD.

In cooperation with industry partners, a therapeutic agent (AT-007) was identified, and the INC is currently getting ready to launch the INSPIRE trial to evaluate the efficiency of AT-007 in treating CMT caused by SORD deficiency.

CMT1A Trials

The most common genetic mutation in patients with a diagnosis of CMT is a duplication of the Peripheral Myelin Protein 22 (PMP-22) gene. Because they have three instead

of two copies of that gene, they produce too much PMP-22, which impairs the myelin sheath, which functions as a protective wrap around our peripheral nerves resulting in loss of both motor and sensory nerve function. Several therapeutic agents are currently undergoing pre-clinical evaluations, and it is expected that there will be multiple Phase 2/3 trials opening within the next two years. While none of these therapeutic agents will be able to reverse the previous nerve damage, they are expected to slow down future progression.

At the PNS meeting in June 2023, there were multiple presentations regarding getting ready for these upcoming trials by validating the sensitivity of different outcome measures – which are the tests that are done in order to determine the efficacy of a new drug in a trial, such as muscular strength testing using a dynamometer, the time it takes to walk a certain distance, etc.

TRPV4 Natural History Trial

Another subtype of neuromuscular disease with great potential to be treatable is CMT-2C, caused by mutations of transient receptor potential vanilloid 4 (TRPV4). TRPV4 is involved in calcium channel function, and misfunction results in too much intracellular calcium. Treatment with therapeutic agents can reduce the influx of calcium on a cellular level, and they have demonstrated promising results in pre-clinical research. However, TRPV4 neuropathy is a very heterogenic disease, which means that affected patients show a large variation in their symptoms and disease severity. To better understand the different sub-types of disease, several academic centers are preparing to launch a natural history study with the goal to identify potential biomarkers and validate different outcome measures for TRPV4-neuropathies to prepare for a future clinical trial.

More information about CMT can be found on the FPN website and also via a different patient advocacy group, the Charcot-Marie-Tooth Association.

Commitment to Cures

In May, FPN sponsored the American Brain Foundation's (ABF) annual gala, Commitment to Cures. Held in Boston, the gala was part of the annual conference of the American Academy of Neurology (AAN).

As the largest annual fundraiser for ABF, this year's gala raised \$565,000 for brain disease research. ABF funds research across the spectrum of neurologic diseases, including ALS, stroke, Alzheimer's, and epilepsy. Thanks to FPN's grant of \$450,000, peripheral neuropathy is now included in their portfolio.

To celebrate FPN's research partnership with the ABF and AAN, the Foundation hosted a table that included Executive Director Lindsay Colbert, FPN Board of Director Ron Lissak, Boston-area donors, and Erika Williams, MD, PhD, a 2023 recipient of FPN's Clinical Research Training Scholarship (CRTS). Funded by one of FPN's grants, Dr. Williams' research aims to develop a gene expression map of the autonomic nervous system. More details about her research can be found at FoundationForPN.org.

FPN's Commitment to Cures sponsorship also continues our partnership with the ABF and AAN in 2024. The CRTS program supports early-career investigators who are likely to continue in the field of PN research. The 2024 award winner will be announced in January.



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The information contained in this newsletter is not intended to substitute for informed medical advice. You should not use this information to diagnose or treat a health problem or disease without consulting a qualified health care provider. You are strongly encouraged to consult a neurologist with any questions or comments you may have regarding your condition. The best care can only be given by a qualified provider who knows you personally.

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FOR THOSE AGED 70½ AND OLDER

Special giving opportunity

Did you know that you can give any amount (up to a maximum of \$100,000) per year from your IRA directly to a qualified charity (such as FPN!) without having to pay income taxes on the money?

WHY TO CONSIDER AN IRA ROLLOVER:

- « Your gift will be used today, allowing you to see the difference your donation is making.
- « Beginning in the year you turn 73, you can use your gift to satisfy all or part of your annual required minimum distribution (RMD).
- « You pay no income taxes on the gift.
- « Since the gift doesn't count as income, it can reduce your annual income level.

Questions or want to learn more?

Contact Lindsay at lindsay@tffpn.org or 847-883-9951.