Neuropathy Action Foundation

Awareness ▪ Education ▪ Empowerment

Patient Voice - September 2023 Issue **Visit our Website**

In this Issue

NAF October 27 Event Announcement

Join the NAF for a MMN Virtual Patient Education Event

The NAF is dedicated to ensuring patients have access to individualized treatment. The NAF increases awareness that neuropathy is a serious and disabling condition, which may be treatable when appropriate medical care is provided. Our goal is to deliver programs that create awareness of MMN, other neuropathies, and the use of IVIG and other treatments.

To this end, please join the NAF on **Friday, October 27 at 12:00 PM (Pacific Time)** for a virtual webinar titled: **Multifocal Motor Neuropathy (MMN) 101 – From Diagnosis to Treatment.** Nationally acclaimed neurologists Drs. Richard Lewis (Cedars-Sinai in Los Angeles) and Jonathan Katz (California Pacific Medical Center in San Francisco) will give an overview of MMN. During this one-hour virtual webinar participants will learn about MMN signs and symptoms, incidences and causes, the importance of accurate and timely diagnosis and treatment. Participants will also learn about treatment access challenges and other barriers to treatment.

On October 27, please click the following link to join the webinar: **https://us06web.zoom.us/j/81506696317?pwd=vcfGzFtsoPUHFq-zC3R82GLsA8g4NA.mSHMQ6x4vliuJ9I-** The passcode for the event is wFX7B7. For more information, please visit the NAF website at **www.neuropathyaction.org**, email **info@neuropathyaction.org** or call (877) 512-7262.

Please Join Us for A
Virtual Patient Education Event

Multifocal Motor Neuropathy (MMN) 101: From Diagnosis to Treatment

Friday, October 27, 2023
12:00 PM Pacific Time

Richard Lewis, MD, FAAN
Director EMG Laboratory and
Co-Director Neuromuscular Clinic
Cedars-Sinai

Jonathan Katz, MD
Chief of Neuromuscular Services
California Pacific Medical Center

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Passcode: wFX7B7

This webinar is generously sponsored by: Takeda

IG Infusion Method Affects Treatment – Related Burden and Outcome

In a study that sought to evaluate treatment satisfaction among patients receiving intravenous immune globulin (IVIG) compared with subcutaneous IG (SCIG), the researchers found that the method selected can potentially affect the patient's treatment-related burden and outcome.

In the study, researcher Paul Bassett, MSc, and colleagues used Canadian Immunodeficiencies Patient Organization and Association des Patients Immunodéfcients du Québec survey data to

identify patients with immunodeficiencies. They reached out via email to request their participation in an incentivized online survey containing 101 questions on IG replacement therapy (IGRT) and their experience with its administration. The survey also included questions on demographic characteristics, choice of infusion method, characteristics of chosen infusion method, history of IGRT, switching between IVIG and SCIG, SCIG training issues and structured patient-reported outcomes.

Of the 327 participants selected for the study, 16.5 percent indicated IVIG was their current method of infusion and 74 percent indicated SCIG was their current method of infusion. In the IVIG cohort, the most common reason (43.5 percent) for patient choice was “maintaining steady levels for treatment (IgG) in the person’s blood.” The most common reason in the SCIG cohort (34.5 percent) was “ability to self-infuse.” An unfavorable impact of the treatment regimen on work/school attendance was reported more frequently in the IVIG cohort compared with the SCIG cohort (33.3 percent vs. 8.3 percent). The median duration of the actual infusion was reported to be significantly shorter in the SCIG cohort compared with the IVIG cohort (60 minutes vs. 165 minutes, respectively).

On survey questions drawn from the Treatment Satisfaction Questionnaire for Medication (TSQM–9) on effectiveness, participants in the SCIG cohort scored significantly higher than the IVIG cohort. Specifically, the SCIG cohort reported greater satisfaction with the amount of time it takes the medication to start working compared with the IVIG cohort.

In both the IVIG cohort and the SCIG cohort, the prescriber was most often identified as the person impacting the patient’s chosen method of infusion; this was more prominent in the SCIG cohort (67.8 percent vs. 52.1 percent). Participants who switched from IVIG to SCIG reported in most cases that the stakeholder responsible for the switch was also the prescriber (70 percent).

Of the participants who switched from IVIG to SCIG, 81.5 percent described an improved or substantially improved quality of life, and 33.1 percent reported substantial improvement. Following the switch, 58.1 percent of participants reported better physical health, and 44.4 percent reported improved or substantially improved mental health. “In general, patient preference assessments have highlighted the importance of continually offering patients information and a choice of IGRT infusion options,” Bassett and colleagues wrote. “In addition to training, clinical support and shared decision-making are critical, even if patients have been satisfied [with] one type of IGRT infusion method for many years.”

Your Health Records:
Why and How to Access, Organize and Use Them

By Leslie Levine

As a neuropathy patient, you probably have seen multiple physicians and other care providers. Whenever you have an appointment, you are asked to provide information, and the provider's findings and conclusions, as well as new test results, are added to yo ur growing medical records. You may well have multiple sets of records, one for each hospital system or provider group. While the task may seem daunting, you may want to consider obtaining your records and putting the

information into a format that will be very helpful in getting you the best medical care as efficiently and cost effectively as possible.

Why should you do this? If you know what is in your medical record, you will be able to understand your health history and be your own best advocate. You'll be able to ask better questions, and work better with your providers to make decisions. You can track your lab results and medications. Each healthcare provider you see wants to know your medical history, but does not have time to read through hu ndreds of pages of medical notes and lab test results. They rely on you to summarize what you know about your medical condition(s), what testing has been done, test results and the conclusions of other providers you have seen. Few people can accurately r ecall the details of their medical visits and testing, and frequently the provider only shares part of his or her thoughts and concerns with each patient. In the few minutes allotted for the initial patient - doctor conversation, some material may be misun derstood or entered in your record inaccurately. The only way for you to be sure that your providers know your past medical history, so they can partner with you to give you the best care, is for you to get your records, check them for accuracy, organize the contents into a usable format , and share this summary with your medical care team. Your efforts will be welcomed by your team of providers, especially new ones trying to get up to speed.

What does this involve? First, you need to get the complete record from each of your providers and hospital systems. You have the right, under the Health Insurance Portability and Accountability Act, or "HIPAA", to see and get copies of all your health records. While some providers have website portals where yo u can access some of your test results and certain other materials, these almost never include all the information that is in your full medical record. Typically you need to request your records in writing. Most providers will give you a records release form either by request or available on their websites. You will need to complete the release forms, designating yourself as the one to whom the records should go , and mail or fax it back . The release form will ask the purpose for which you want your reco rds. Since you are using the information to provide to your care team, you should mark th e reason as being for medical care; this often will in cur no charge for the records. Be sure to request your entire medical record, including providers' notes about visits, discharge summaries, reports on labs, operations, pathology, radiology, genetic screening and mental health care reports. You can also request information on your account and billing history. The law requires that you be provided with your medica l records even if you may still owe medical bills. You will usually be able to choose the form at in which you get your records. If you request your records in paper form, there may be a per page fee. T he fee is typically less, or even free, if you requ est that your records be provided electronically by secure download. There may be an additional fee if you want copies of photographs or x - rays along with your records. The provider has 30 days to send you your records, but often will provide them in less time.

Now that you have your records, what next? You might want to get sets of file folders, and separate the records into sections, such as for visit notes, hospital inpatient stays and discharge reports, lab tests, radiology/imaging reports, cardio logy, pathology, microbiology and other tests (like neurophysiology testing such as nerve conduction velocity or autonomic function testing). The records will typically be in chronological order. As you go through the records, be sure to note if you th ink there is an error. You would then ask your provider to correct the record. Even if the provider disagrees with you, you have a right to have the facts of the problematic material put into your medical record.

Click here to continue reading.

Understanding the Immune System

By Michelle Greer, RN, IgCN
April/May Edition of IG Living

THE IMMUNE system is a complex network of cells, tissues, organs and proteins that work together to keep the body healthy. The immune system's primary roles are to recognize something foreign and destroy it, differentiate self from nonself, keep self healthy and destroy malignancies. Because of the many intricacies within the immune system, if something is deficient or defective, the result can be an immunedeficiency or autoimmune condition.

The Immune System's Lines of Defense

The immune system has two main lines of defense: innate immunity and adaptive immunity. Innate immunity is what people are born with. The innate immune system is the first to respond to an antigen (foreign substance), and its response is immediate — within minutes or hours. It is made up of several parts that form a physical barrier to protect the body. Components of innate immunity include the skin and any other mucus membranes that protect organs such as the cornea of the eye and the lining of the lungs and intestine….

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Importance of Participating in Clinical Trials

Participating in a clinical trial is an invaluable way to get involved in the pursuit of a cure for Peripheral Neuropathy. For patients, new medicines offer fewer side effects, fewer hospitalizations, improved quality of life, increased productivity, and importantly, extended lives. We have partnered with Antidote to help you find and take part in a clinical trial that is right for you. Simply answer a few questions and Antidote's smart search engine will match you to a trial, quickly and easily. The rapid pace of scientific advances is enabling a greater understanding of diseases at the molecular level. The drug development process begins when **researchers** perform laboratory and animal tests to discover how the drug works and whether it's likely to be safe and work well in humans.

Next, a phase of clinical trials begins, and this is where studies are performed on people to determine whether the drug is safe when used to treat a disease and whether it provides a real health benefit. Along the way, investigators uncover important milestones that may lead to new treatments, but there are also often many dead ends and setbacks, which may lead researchers down a new route, or force them to take a step back. While these stumbling blocks can be disappointing, they are an integral part of a complex research and development process; both the setbacks and successes provide invaluable knowledge that help guide and direct researchers to get one step closer to the next advance.

On average, it takes at least ten to fifteen years for a new medicine to complete the journey from initial discovery to the marketplace, with clinical trials alone taking six to seven years on average. The average cost to research and develop each successful drug is estimated to be $2.6 billion.

Ultimately, though, the process of drug discovery brings hope and relief to millions of **patients**. Clinical trials are research studies that use human volunteers. Clinical trials are

conducted to collect data regarding the safety and efficacy of new drug and device development. There are several steps and stages of approval in the clinical trials process before a drug or device can be sold in the consumer market, if ever. Understanding what they are can help you decide if a clinical trial might be an option for you. Alternatively, maybe you have a friend or family member with **peripheral neuropathy** and are wondering if a clinical trial is right for them.

Why are Clinical Trials so Important to Drug Development?
The U.S. FDA requires that a potential therapy's safety and efficacy be tested extensively in a large group of human volunteers before it can receive approval to be manufactured and made available to patients. Yet clinical testing can — and often does — fail because not enough people volunteer. Without sufficient numbers of trial participants, the drug development process stalls and a trial must be repeated, scaled back or, even worse, the potential new therapy is abandoned. This lengthens the time it takes for new treatments to come to market. No amount of funding or other resources can compensate for the lack of clinical research volunteers. That's why volunteers can play a truly unique role at this pivotal stage of drug development, which is crucial for new **treatments** to reach pharmacy shelves.

Why is it critical that more people volunteer for trials?
Clinical trials play a critical role in the development of new and better therapies. Under-enrollment in trials is one of the greatest challenges clinical researchers face. Under-enrollment in trials slows research progress and deters potential funders from investing in research. We all pay the price in terms of higher costs and longer time horizons to get to therapeutic breakthroughs. Across all diseases, 85 percent of clinical trials finish late due to difficulties enrolling participants and nearly one-third of trials fail to recruit a single subject and cannot ever begin. Clinical trials are conducted in a series of steps, called phases – each phase is designed to answer a separate research question.

Phase 1 Clinical Trials
In Phase 1 trials the candidate drug is tested in people for the first time. These studies are usually conducted with a small number of healthy volunteers, generally 100 or less. The main goal of a Phase 1 clinical trial is to assess the safety of the medicine when used in humans. Doctors/researchers look at the pharmacokinetics of a drug: How is it absorbed? How is it metabolized and eliminated from the body? They also study the drug's pharmacodynamics: Does it cause side effects? These closely monitored trials are designed to help researchers determine what the safe dosing range is and if the candidate medicine should move on to the next stage of development.

Phase 2 Clinical Trials
In Phase 2 clinical trials, doctors/researchers evaluate the candidate drug's effectiveness in 100 to 500 patient volunteers. Many Phase II trials study patients receiving the drug compared with patients receiving a different treatment, either an inactive substance (placebo), or a different drug that is usually considered the standard of care for the disease. Doctors/researchers also analyze optimal dose strength and schedules for using the drug and examine the possible short-term side effects (adverse events) and risks associated with the drug. If the drug continues to show promise, they prepare for the much larger Phase 3 trials.

Phase 3 Clinical Trials
Phase 3 clinical trials generate statistically significant data about the safety, efficacy and the overall benefit-risk relationship of the investigational medicine. Phase 3 trials may enroll 1,000 to 5,000 patients or more across numerous clinical trials sites around the world. This phase of research is essential in determining whether the drug is safe and effective. Each patient enrolled in a Phase 3 clinical trial has a chance of being in one of the following groups:

- Control group – the group that gets the standard treatment
- Study group – the group that gets the new treatment being tested
- Doctors/researchers do not know if the new treatment is better than the standard treatment, but they believe it is as good and may be better.

After the Phase 3 trial, the FDA reviews the clinical trial results to make sure the treatment is safe and effective for people to use. The FDA decides whether to approve the treatment so that it is available for all patients.
There are many frequently asked questions about Phase 3 trials. These include:

How are patients put into groups?

Would my doctor know which group I am in?
Would I be given a placebo?

Phase 4 Clinical Trials
In Phase 4 trials, doctors/researchers study treatments that the FDA has already approved. The goal of Phase 4 clinical trials is to continue studying side effects of a new treatment.

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