

VOICE OF THE PATIENT

Chronic Inflammatory
Demyelinating Polyneuropathy



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Demyelinating Polyneuropathy

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AUTHORS AND COLLABORATORS

Chelsey K. Fix, MPH, GBS|CIDP Foundation International
Lisa Butler, GBS|CIDP Foundation International, Executive Director
Ellie Herman, GBS|CIDP Foundation International
Alex Stenman, MBA, GBS|CIDP Foundation International
Laura Blair, GBS|CIDP Foundation International
Peter Donofrio, MD, Chair of GBS|CIDP Foundation International Global Medical Advisory Board

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Please contact Chelsey K. Fix at Chelsey.Fix@gbs-cidp.org with any questions about this report.

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contents

1	Introduction to CIDP and the Need for Innovation	4
2	PANEL 1 Diagnosis and Treatment Options of CIDP	6
3	PANEL 2 The Everyday Burden of CIDP	11
4	PANEL 3 Patient Centric Trial Design for CIDP	15
5	PANEL 4 Potential Future of CIDP Treatment	19
6	Conclusions	24
7	About the Foundation	25
8	APPENDIX 1 References	26
9	APPENDIX 2 Finalized EL PFDD Agenda	27
10	APPENDIX 3 Testimony from Each Panelist	28
11	APPENDIX 4 Scientific Overview by Dr. Peter Donofrio and Dr. Jeffrey Allen	47

1

Introduction to CIDP and the Need for Innovation

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) is a condition of the peripheral nerves⁽¹⁾. Symptoms of CIDP typically begin as debilitating weakness, intense numbness, and life-altering fatigue.

Treatment options for CIDP are typically invasive infusions that include an array of side effects. Additionally, the treatment for CIDP is expensive, dependent on the finite resource of human plasma, and can only be administered intravenously or subcutaneously. The GBS|CIDP Foundation International hopes innovation in the CIDP treatment space will increase patients' overall quality of life.

CIDP is a rare disorder, with prevalence between 0.8 and 10.3 per 100,000 individuals⁽¹⁾. The prevalence in the US is approximately 60,000 individuals. Onset of CIDP typically occurs in middle-age, between 48-60 years of age, but people of all ages may be diagnosed with CIDP⁽¹⁾. Some studies have found that the prevalence of CIDP is higher in males than in females⁽¹⁾. There is no known trigger or cause, but the current research suggests immune system involvement which impacts the myelin sheath⁽¹⁾. In fact, the prevalence of common autoimmune disorders is significantly higher in CIDP patients than in the general population⁽¹⁾.

Typically, a person living with CIDP receives immunoglobulin (IG) treatment upon diagnosis, and sometimes this treatment is initially administered in the hospital — called a loading dose — before transitioning to an outpatient, chronic treatment schedule⁽²⁾. Because of the complexity of IG therapy, a person's options for getting the treatment are somewhat limited and often complicated by restrictive insurance policies⁽¹⁾. Typical options for receiving IG therapy include: 1) receiving an intravenous infusion at a specialty infusion center approximately monthly, 2) receiving an intravenous infusion through a specialty pharmacy that provides the service in-home approximately monthly, or 3) self-administering subcutaneous IG formula approximately weekly with multiple injection points per administration^(2,3). Corticosteroids are also frequently prescribed, both in place of IG treatment or in addition to it^(2,4). Other immunosuppressing medications, such as azathioprine (Imuran), mycophenylate mofetil (CellCept), methotrexate, or cyclophosphamide (Cytoxan) are sometimes used in an off-label capacity. If these therapies are unavailable or unhelpful in treating CIDP, then plasma exchange, a process by which blood is removed and returned with a plasma substitute, may be utilized⁽²⁾.



IG therapy is created from human plasma, thus the creation of the therapy is dependent equally on the manufacturers of the product and the benevolence of people who donate plasma.

Side effects of IG treatment may include migraines, nausea, fatigue, brain fog, and general malaise⁽¹⁾. The logistics of treatment also present many challenges to patients living with CIDP^(1,5). Because of the disabling nature of the condition, patients may be fully or partially dependent on a caregiver — either paid or unpaid — to assist with many aspects of their day to day life. Some of the tasks that caregivers may assist with include transportation to treatment, assisting with the basic setup of the treatment, assisting with mobility, and other daily tasks. Anecdotally, CIDP patients report having their drivers license medically revoked because of the disability brought on by CIDP, increasing their dependence on others.

Relying on IG therapy presents other risks. IG therapy is created from human plasma, thus the creation of the therapy is dependent equally on the manufacturers of the product and the benevolence of people who donate plasma⁽⁶⁾. If a disruption occurs to the supply chain, or a disruption in plasma collection, patients may experience a disruption in the product they receive or the frequency of their treatment⁽⁶⁾. Additionally, plasma products are non-interchangeable, meaning that most patients can not easily switch between different brands of IG therapy⁽⁶⁾. CIDP patients, for example, may try several brands of IG because of allergic reactions or unfavorable side effects. This creates more pressure on the manufacturing process to keep up with the increased global demand of various plasma products⁽⁶⁾.

Partly because of the complex process involved in creating IG therapy, treating CIDP is a delicate ecosystem. Moreover, the mechanism behind IG treatment is not fully understood but focuses solely on treating the neurological symptoms of CIDP and not necessarily aiding the cascade of other physical, emotional, and mental changes associated with CIDP. Innovation is essential to ensure patients living with CIDP are being treated holistically.

To catalyze the innovation currently happening for CIDP, the GBS|CIDP Foundation International held an externally-led patient focused drug development meeting virtually on March 25, 2022. The aim of this meeting was to solidify the patient perspective on innovation for CIDP and elevate the patient voice in the research process. The virtual meeting included four live panels featuring patients, a live chat for patients to submit their own comments, live polling questions, and a follow up survey for CIDP patients. Platform Q hosted the meeting virtually.

Each patient panel focused on a specific aspect of living with CIDP and fostering innovation: Diagnosis and Treatment Options; The Everyday Burden of CIDP, Patient-Centric Clinical Trial Design for CIDP, and the Potential Future of CIDP Treatment. Each panelist gave their own introduction, and then participated in a deeper discussion on different aspects of each panel topic. A live chat feature within the virtual meeting platform allowed patients in the audience to ask questions of the panelists or weigh-in with their own experiences, and live-polling was utilized to engage patients in the audience on specific questions. A follow up survey for CIDP patients also provided an opportunity for more patients to add their own perspectives and experiences on these topics.

2

PANEL 1

Diagnosis and Treatment Options of CIDP

”

I relapsed earlier this year when my feet started feeling numb and then continued up my legs. My balance was off as well so I called my doctor. I received a couple double doses of IVIG and then he increased my monthly dosage. I'm feeling better but when the first signs of CIDP returned I felt dread and despair all over thinking I was going to be paralyzed again.

PATIENT QUOTE

The first panel of the EL PFDD Meeting featured a discussion on the diagnosis process and treatment options for CIDP. The discussion was moderated by Dr. Mamatha Pasnoor, a professor in the Department of Neurology at the University of Kansas Medical Center and member of the Foundation's Global Medical Advisory Board. The panelists included Julie B. from Texas, Jim C. from Illinois, Barry F. from Texas, and Diana C. from North Carolina. Each of the panelists started with an introduction of themselves and provided in-depth testimony of the experience of being diagnosed and treated for CIDP. Though each panelist described a distinct pathway (full testimony in Appendix 2), there were several striking similarities between each panelists' testimony.

Specifically, each panelist recalled early symptoms that indicated issues with coordination, describing “awkward gait” (Julie), “extra clumsy” (Diana), “foot drop” (Barry). Another common theme that was described was the remitting and relapsing nature of CIDP after the initial diagnosis. Jim recalled “hospital stays on and off” as the most severe symptoms waxed and waned, and Barry described his journey with CIDP as one that “ebbs and flows.”

These themes were echoed not only among the panelists but among the broader CIDP community as measured by the post-meeting survey. Patients wrote in to describe their relapses:

I relapsed earlier this year when my feet started feeling numb and then continued up my legs. My balance was off as well so I called my doctor. I received a couple double doses of IVIG and then he increased my monthly dosage. I'm feeling better but when the first signs of CIDP returned I felt dread and despair all over thinking I was going to be paralyzed again.

The panelists then reminisced about their diagnosis process. This was described as “a long road” by Jim while Diana noted that she saw five neurologists before being properly diagnosed. The panelists explained that the diagnosis process often takes so long because CIDP becomes a diagnosis through the process of elimination of other diagnoses. For CIDP, improvement with IVIG is often what helps to solidify the diagnosis and rule out other neurological conditions, such as ALS.



The discussion then turned to treatment options that have been used for CIDP by the panelists. All four panelists described being treated with IVIG, with varying results. For Barry, IVIG is what he believed “stopped the progression” of his symptoms. Julie described herself as “IG dependent” and described a process of getting a port to make her IVIG infusions easier on her. While a port may allow for easier access for blood draws and IV medications, it also comes with its own set of risks, including a surgical procedure and increased risk of infection, thus becoming another medical burden for some CIDP patients to care for.

The benefits of IVIG are well-documented in the CIDP community. One person has described IVIG as such: “IVIG was liquid gold for me! I went from being stuck in a wheelchair to walking again!!” However, the downside to IVIG, according to the community, include the side effects, cost, and frequency of the treatment.

I received my CIDP diagnosis in September 2021. Since then, I’ve received monthly IVIG treatment. The side effects are difficult to manage, but the treatment has helped so much.

Required every 17-21 days for health ambulatory, motor control.

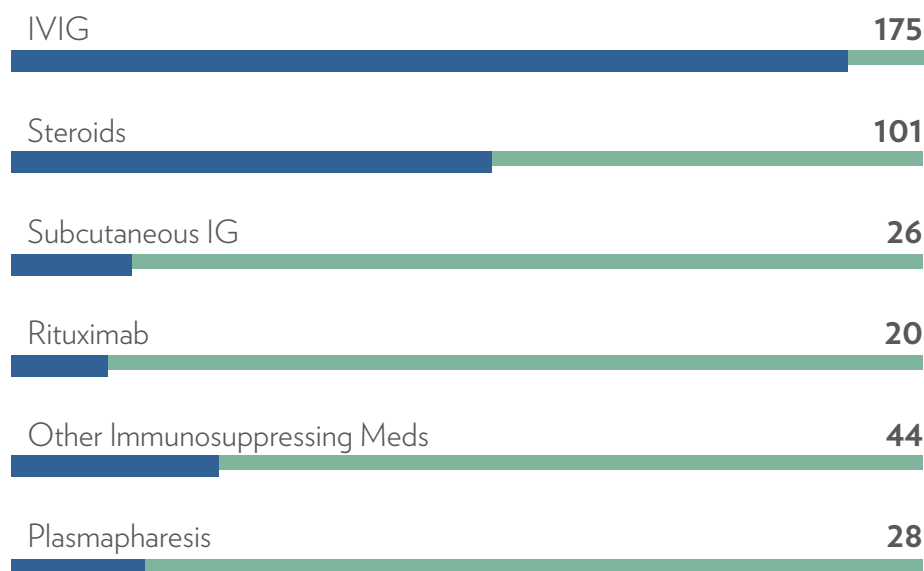
I do well on my treatment, but I would prefer not to have to have infusions every three weeks and I would like to reduce my dependence on infused steroids.

In looking at the broader community in the post-meeting survey, IVIG was definitely the most common medication used with 95% of respondents having taken IVIG at some point in their CIDP journey. Though IVIG was the most

(cont’d on next page)

WHAT TREATMENTS HAVE YOU EVER TAKEN TO TREAT CIDP?

Select all that apply. (n=184)



”

There seem to be very few effective treatments available that don't cause long, damaging side effects. With no cures or funding for research, it seems I will need [treatment] for the rest of my life. All I can do is keep up with IVIG, floor exercise (yoga), and PT and hope it helps to delay this progressive, disabling, terrible disease.

PATIENT QUOTE

popular treatment option, subcutaneous IG is gaining popularity in the CIDP community. From the post-meeting survey, 14% of respondents report having ever used Subcutaneous IG. Subcutaneous IG is a relatively new treatment option for CIDP patients with only 1 brand being FDA approved, but is slowly gaining interest as an option for patients who are burdened by the IVIG treatment schedule but still looking to benefit from the IG therapy.

The next most common medication used to treat CIDP after IVIG was steroids (prednisone), with 55% of respondents reporting that they have taken steroids. The four panelists also all had experience with steroids. Julie described the “horrible side effects” that came with steroids, and that was echoed by the other panelists. Jim mentioned having to rearrange his whole life to accommodate both an IVIG infusion and a weekly steroid infusion, both of which gave serious side effects. These side effects of steroids, including weight gain, insomnia, anxiety, and many others, are well documented beyond the CIDP community, but patients described their experience as such:

I was on prednisone at the beginning of my treatment which elevated my sugar and I have never recovered.

I was started on steroids which only worsened my weakness.

Other common medications used to treat CIDP, according to the CIDP community via the post-meeting survey, include Rituximab, other immunosuppressing medications (such as Cellcept or similar drugs), and plasma exchange.

Somewhat surprisingly, it was discovered that the majority of patients have been on multiple medications for their CIDP. Of the patients who completed the post-meeting survey, 65% have been on multiple medications to treat their CIDP. This has resulted in the overall feeling in the community that their conditions are “managed” but never “cured.”

There seem to be very few effective treatments available that don't cause long, damaging side effects. With no cures or funding for research, it seems I will need [treatment] for the rest of my life. All I can do is keep up with IVIG, floor exercise (yoga), and PT and hope it helps to delay this progressive, disabling, terrible disease.

During the panel, the audience and panelists were asked to choose from a list of symptoms which they would like an innovative treatment to eliminate. The question was posed as “what is your priority for remission” and respondents were able to select two answers from a list: better balance, better use of hands, reduction of fatigue, elimination of the need for steroids, reduction of pain, or none of the above. The panelists gave the following responses:

Julie: *It would be the reduction of fatigue.*

Barry: *I definitely wanted [to select] all [of the choices from the list] but my darkest moments have been with pain and the idea I would have to live for another*

More than 60% of respondents to the survey indicated that the number one priority is a reduction of fatigue.

40 years with this unrelenting thing. The pain takes away my ability to think about having quality.

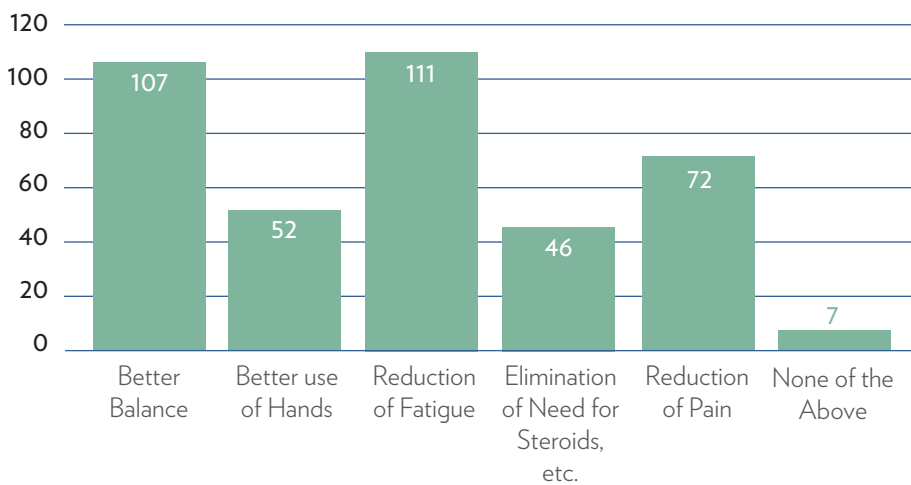
Diana: I would choose the elimination of needing medications because they all have side effects and are difficult to tolerate. But my number one would also be reduction of fatigue. While the physical limitations pose challenges, the fatigue makes it difficult to even just sit and visit with people. Your body just kinds of break down over time. My husband says I have a “cheap battery” and that’s why I would choose that one.

Jim: When I was in the midst of the CIDP, pain was certainly an issue and that would be something that I would want to reduce. After GBS and through CIDP and even still to this date while being in remission, fatigue is a factor, so that would be something I would wish to exclude from the equation.

This question was also posed to the audience during the meeting, and of the people who responded to the live poll, almost 40% also wished for a reduction of fatigue. Better balance, elimination of need for steroids or other medications, and reduction of pain were all also popular answers.

In the post-meeting survey to the broader community, the reduction of fatigue was overwhelmingly the most popular answer choice selected by respondents. More than 60% of respondents to the survey indicated that the number one priority is a reduction of fatigue. A close second priority according to the survey was better balance, with 58% of people choosing that response (respondents were able to choose more than one answer choice). People wrote in to explain their answer choice:

IF YOU WERE TO REACH REMISSION, WHICH OF THESE WOULD BE YOUR TOP PRIORITY?
Can select multiple. (n=184)



”

I degrade throughout the day with fatigue, pain exists but it's the Bone Crushing Fatigue- sleep issues due to random pain and such, I'm semi functional, but only for about 5 hrs a day.

PATIENT QUOTE

It takes a lot of energy to walk and I tire. I push to do 6,000 steps daily. Prior to CIDP I did 10,000 steps daily easily.

Fatigue is my greatest limitation.

I degrade throughout the day with fatigue, pain exists but it's the Bone Crushing Fatigue- sleep issues due to random pain and such, I'm semi functional, but only for about 5 hrs a day.

Prior to my CIDP diagnosis, I was a mostly healthy female in my 20s. The fatigue is like nothing I've ever experienced before. A reduction in that specific symptom would be life changing. I have adjusted to this "new normal," but it would be so wonderful to have more energy again and fewer falls.

Bad balance and fatigue badly affect my work and personal life and no matter what I do nothing helps with these two things.

The intriguing first panel of this meeting brought some key takeaways, including the arduous diagnosis process, the community's reliance on IVIG, and the still untreated symptoms of CIDP that cause major problems for patients living with the condition. It is clear the fatigue that is brought on by CIDP is a priority for the community, evident by it being the most popular answer among the meeting panelists, attendees, and patients who responded to the post-meeting survey. Additionally, it was confirmed that IVIG was still the most popular medication to treat CIDP, but patients with CIDP are rarely ever using only one medication to treat their symptoms.

key takeaways

1. Patients living with CIDP typically experience a long diagnosis process, sometimes with a gradual progression of symptoms.
2. The symptom of fatigue is one that CIDP patients struggle to treat through their treatment plan and is many people's priority symptom to alleviate.
3. IG therapy and steroids are used broadly in the CIDP community with a great deal of success, but also a great deal of burdensome side effects.

3

PANEL 2 The Everyday Burden of CIDP

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I was terrified of waking up every morning to discover another ability that I had lost.

PATIENT QUOTE

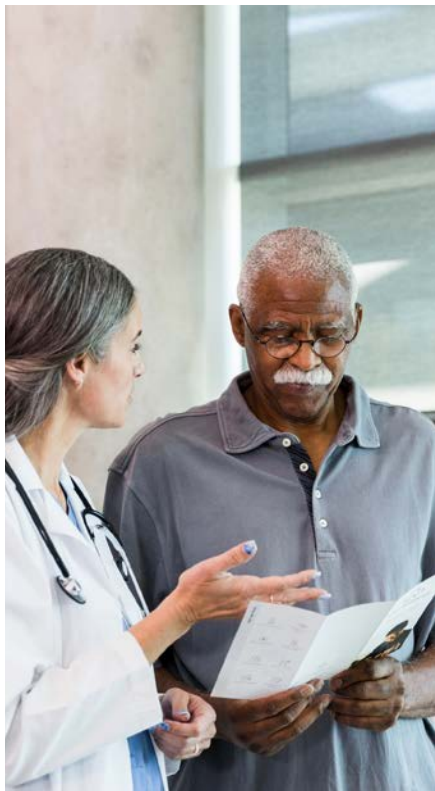
The second panel of the meeting introduced four new panelists: Kelly M. from Pennsylvania, Michele D. from Maryland, Victor S. from Pennsylvania, and Jon S. from New York. This panel was moderated by Lisa Butler, the Executive Director of the GBS|CIDP Foundation International, who connects personally with the foundation as the parent of a person who experienced GBS at a young age. Lisa opened the panel by allowing each panelist to introduce themselves. Again, the panelists recalled their journeys to diagnosis which included themes such as progressing symptoms that resulted in a catastrophic event that started the long search for a diagnosis. During the tumultuous time of the presentation of symptoms and search for diagnosis, Kelly described being “terrified of waking up every morning to discover another ability that I had lost.” Jon, the CEO of a hospital network, described subtle changes that he tried to ignore until his colleagues decided to investigate what was wrong with him. Overall, each panelist recalled the life-altering diagnosis that was eventually reached and then moved into a deeper conversation about adjusting to a new life living with CIDP.

Victor described the everyday tasks that he can no longer do independently because of CIDP:

I can no longer throw a baseball or football. I can't walk more than 15-20 feet without some sort of assistive device. I used to love 3-4 mile power walks. Being wheeled to the TSA is a plus. I can't shovel snow from a driveway. If I stand, my aid is always between us. Feels like a barrier for a fence or a wall. I can't stand to put up the Christmas lights on the trees inside or outside. I can no longer carry anything in my hands without assistance, thus I can't help people move things or put the window AC units in during the summer. I also cannot move outside furniture between the shed and the decks or easily collapse and raise the pool umbrellas.

Jon described similar sentiments about how he has made adjustments to his life while attempting to hold on to his independence:

We do have a place in Dallas, Texas where my grandchildren are, so we try to get down there once a month, but traveling is challenging. I'm a little stubborn. I don't like to use wheelchairs or the assistive devices, so I just kind of hold on for dear life, and try to go any way I can.



Kelly shared her own anxieties about the future:

*In my pockets of time, I'll find my mind wondering if I'll be fortunate to carry my daughter, if when she's old enough, I'll be able to play with her or be the parent she deserves. I choose to embrace what CIDP has brought into my life. **What it has stolen cannot be discounted.***

Michelle also shared this insight into her new normal:

With the demand of work and the flareups, I was reduced to a reduced work schedule. Not only did I have to deal with the physical and emotional challenges associated with this condition. At this point I now had to face the financial burdens of this disorder.

In moderating the conversation, Lisa astutely pointed out that many panelists were describing ways in which their definition of “functional” has changed throughout their CIDP diagnosis. While everyone with CIDP has adapted to living, their functional level now is certainly not what it previously was. This was reflected in audience response to the polling question about what the audience considered the biggest burden to be, which was “fatigue.” The second most popular response was “not being able to do a favorite activity.” These two popular answers complimented the panelists description of their burden by highlighting that life has been changed for people living with CIDP.

These results were echoed in the post-meeting survey where respondents could select multiple answers. The most popularly selected answer choice was “fatigue” (78% of respondents selected) followed by “not being able to do a favorite activity” (71% of respondents selected). “Anxiety for the future” was also a popular choice, with 57% of respondents selecting that choice. Patients wrote in about this anxiety and about their frustrations in changing their lives to accommodate CIDP (graph on next page):

I know that CIDP can be progressive. I responded very well to IVIG but to go from normal to being unable to stand without support, with the pain and weakness, within a couple of days/weeks is terrifying. I've had a few attacks and I'm terrified that the IVIG will stop working at some point and it will progress. I am a caregiver of an adult child with autism and it makes me worried about the future. I worry about losing my insurance and being able to afford it, I worry about the availability of IVIG if people stop donating blood, etc...

The uncertainty is distressing. This issue affects one's ability to work with a clear head.

Coordination of infusions with vacation and other family affairs. I fear progression to wheelchair, even amputation.

I love to travel, and regret that I cannot get to travel to so many places I would like to visit.

The future worries me the most because my CIDP symptoms are so unpredictable.

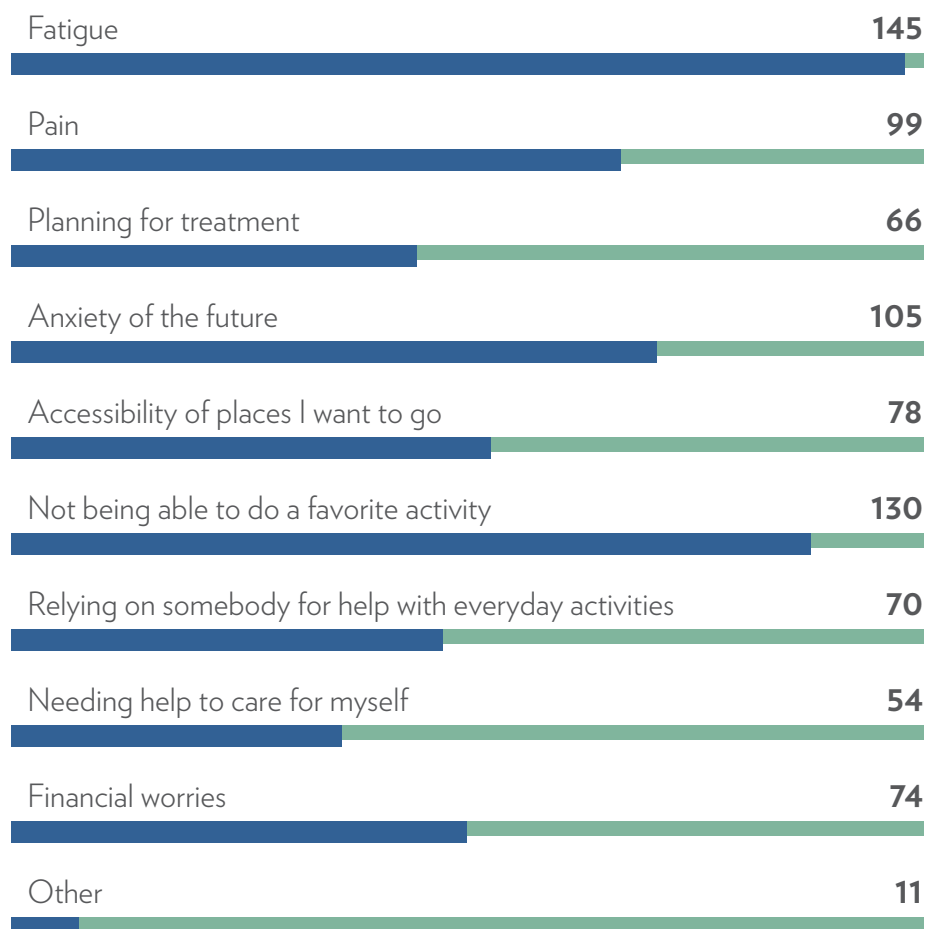
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PATIENT QUOTE

PLEASE SELECT FROM THE LIST WHICH OF THESE BURDENS IMPACTS YOUR LIFE?

Select all that apply. (n=184)



There is definitely a definite level of anxiety due to the long-term unknown of what it is like living with this disease.

My life has changed drastically, as have I - I wish I could be my old self again, with none of the burdens mentioned above.

The panelists reflected on this anxiety about the future as well, prompted by Lisa to think about how they plan for the future with CIDP. Kelly shared her thoughts:

CIDP is a part of my life constantly. I would consider myself in remission right now, but however it's hard to enjoy. I'm constantly waiting for the other shoe to drop. I'm scared whenever I carry my baby down the stairs. CIDP is ever-present. Planning around the future is always planning with my companion, which is CIDP.

Despite most CIDP patients, including the panelists, having a stable treatment regimen, the panelists reflected on how accessing treatment for CIDP is a burden

key takeaways

1. The symptoms of CIDP are a constant companion and reminder of the things patients can no longer do because of their physical limitations.
2. Living with CIDP requires a careful planning for the future, which can be stressful and anxiety-inducing.
3. Interpersonal relationships are also strained because of CIDP, including the burden that is placed on loved ones while a patient adapts to their new life with CIDP.
4. Access to treatment is a burden and could be a barrier to consistent management of CIDP, thus exacerbating physical, social, and emotional burdens.

in itself. Jon shared that his infusions “take between seven and eight hours” because of his need for a slower infusion rate to prevent side effects. Michele shared that the side effects of treatment, specifically “weight gain” from steroids, really became a burden to her. She also lamented the long infusion times, which impacted her career because of her constant need for time to get treated. The weight of treatment burden was echoed by comments from the post-meeting survey:

Working around treatments affects everything, appointments, vacations, getting together with friends...

Accessibility includes finding treatment places when I visit family or others in other states and arranging for delivery of medical records so a local doctor can prescribe my required meds.

Finally, the panelists shared a surprising insight in that they often felt the caregiver had a heavier emotional/mental burden than they did. Victor shared the insight that his wife is often more protective or more nervous for his safety than he is, saying “My wife is a lot more worried about me than I am. She doesn’t seem to understand it. I will come close to the line. I know where the line is. I’ll come close to it but not cross it. She’s definitely afraid of me crossing a line that I’m not going to cross.” Jon agreed that his wife was also typically more worried about his safety than he was. Michelle and Kelly found a similar issue in that their interpersonal relationships became quite strained because their friends and family didn’t quite know how to talk about CIDP. Kelly said “It was either they treated me like I was an egg and super overly cautious with me, or they really didn’t know how to approach me or speak to me, so they just stopped”. The post-meeting survey also mentioned a few of the interpersonal relationship worries:

I rely on my husband for doing quite a bit around the house and some shopping. I’m worried about our future...

...they all cause an extreme amount of worry for my wife and I. We are heading to our retirement years and we simply aren’t sure what the future holds for us both.

I’m lucky I have a girlfriend that’s willing to help me with some of my shopping but without her I’d be lost.

After discussing the financial burden of CIDP (including the cost of treatment vs. lost wages because changing work schedules due to disability), the panelists ended the discussion with a more hopeful tone, highlighting that finding a community of other CIDP patients has helped to reduce the burden. However, innovative new treatments that move towards curing the most burdensome symptoms of CIDP would be monumental.

4

PANEL 3

Patient Centric Trial Design for CIDP



The third panel of the meeting brought three new panelists and welcomed Dr. Pasnoor back as the moderator. Dr. Pasnoor highlighted her own experience on pivotal trials for CIDP, including trials that have helped advance the treatment of CIDP. The panelists then introduced themselves; Betsy B. from South Carolina, Dave R., from New York, and Dave T. from California. The panelists all briefly described their CIDP journey, and a common theme among these panelists was the gradual progression of symptoms over months that eventually led to a “low point” that inspired a new course of action. As Dave R. described, “had tingling in my feet, then into my legs, and then the weakness began, and it was very scary because they didn’t know what was going on. That was over a course of maybe six months, seven months, eight months, and then it became very scary because the tingling started in my fingers, my arms, and then the weakness came in...”. Betsy mentioned being in and out of the hospital for months during the process of getting stabilized, and Dave mentioned the 2 year gap from his first symptoms until he was diagnosed with CIDP. These challenges echoed the stories of the previous panelists of a gradual but dramatic change in life once CIDP symptoms begin.

Dr. Pasnoor opened the discussion on clinical trials by reminding the panelists and audience about the definition of washout period, which is when patients participating in a clinical trial spend a period of time not taking any medications before being randomized into an investigational or a placebo group within the trial. The panelists all agreed that the idea of a washout period is “scary.” As Dave T. said, “it’s scary or fearful because it is — we’ve — not just myself — worked so hard with our neurologists to get a plan to help us function. It may not be perfect or work all the time, but it works, and when we have setbacks where we go off the meds for some reason, it can be really difficult to get back to a certain level.” Betsy agreed, saying “There’s so much fear that we have now for those of us who had relapses and had to work back. We know every time we go backwards and relapse, there’s damage to the nerves that may or may not recover and cause new difficulties for us, so the idea of an additional regression may take us back and cause more damage or even new damage that we didn’t have before, and a new disability, it’s a lot to overcome.” Despite this fear, both Dave T. and Betsy mentioned that whether a trial is in Phase 1, 2, or 3 might also influence their

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Most of us do understand that we can relapse. We can get worse at any given point, so stopping the progression is a huge, huge, huge deal.

PATIENT QUOTE

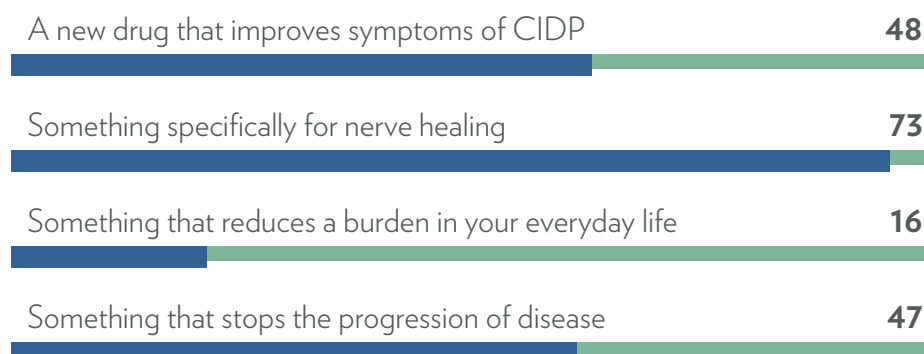
feelings on a washout period, as well as the likelihood of receiving placebo and a well-communicated plan for rescue. Dr. Pasnoor mentioned that this fear of washout periods might be deterring people who want to participate in trials.

Moving the discussion along, Dr. Pasnoor posed another question to the panelists and to the audience; What is the highest priority for something undergoing a clinical trial? The response options for the question included: 1) Something specifically for nerve healing, 2) Something that stops the progression of disease, 3) Something that reduces a burden in your everyday life, 4) A new drug that improves symptoms of CIDP.

Dave R. was the first panelist to weigh in on this question, noting that his top choice would be something that heals nerves, but that stopping the progression of disease was also a very high priority. “Most of us do understand that we can relapse. We can get worse at any given point, so stopping the progression is a huge, huge, huge deal.” Betsy agreed that stopping the progression of disease would also be a top priority.

In the post-meeting survey to CIDP patients, the most common answer was “something specifically for nerve healing” (39%), but a drug that improves symptoms of CIDP and something that stops the progression of disease tied for the next most common answer, each receiving 26% of responses. That there was not a majority reached in any of the answer choices shows the need for innovation across many aspects of life with CIDP and affirms that any progress will be generally welcomed by the community. It also highlights the interconnectedness of all aspects of CIDP in a person’s life. A respondent from the post-meeting survey wrote to explain their response: “My nerves and numbness create the biggest barrier; numbness creates unbalance issues, the constant balance corrections adds greater fatigue, extreme fatigue is also a result of anxiety and depression.” Because of this interconnectedness, the community is anxious for any incremental improvement. Other patients who took the post-meeting survey shared their thoughts on this as well:

WHAT IS YOUR HIGHEST PRIORITY FOR SOMETHING UNDERGOING A CLINICAL TRIAL? (n=184)





Yes, I would like someone to look at more than drugs to help CIDP & how about looking inside the body as to causes and what we may be able to do inside the body to create a remission instead of creating new drugs that are unaffordable, have tremendous side effects and only work on some patients.

What I'd most like is to heal the nerves in my feet so I don't have the pain, numbness, and fasciculations and I can comfortably resume hiking and walking barefoot.

A drug that would be easier to administer and that would heal.

If we had something to help heal our nerves, hopefully our pain, fatigue and other symptoms would go away.

I would like a treatment that cures CIDP, but I know better.

They are all priorities for different times in your journey.

Within the panel discussion, the conversation moved towards better understanding the burdens placed on patients who are actively participating in a clinical trial. Dave R. weighed in, saying “One, I guess, you know, is how do you measure the progress? Is there actual improvement? I do this test that’s simple, like ‘can you pick up this chair’ and things like that, and that’s something that can be measured at home. Transportation, that’s a big one. What if you can’t get there? Is the company going to be offering transportation?” Dave R. also went on to describe that he will need all of the most important information before deciding to participate, including the goal of the trial (“Is the goal to be heading towards a cure or to stop the progression?”), the amount of visits, the type of doctor connected to the trial and whether they are a CIDP expert, and whether or not there is a 24-hour hotline to monitor issues (“I want to know if all the sudden my face turns beet red, I’d like to pick up a phone and find out, is this part of the drug, or is that, you know, something else?”). Dave T. pointed out another burden for patients, saying “let’s say you’re in the placebo arm, and they see that the other arm of the study is showing great results, will they stop the placebo line and move you into that group versus swapping you out to see what happens? I think those are big burdens in the sense of stressful weight on patients looking at trial design and what it’s going to do and what are the end points?” This punctuates the need for in-depth patient education on each unique trial design.

The anxiety of participating in trials was a major discussion point for the panelists. The panelists all expressed some fear of declining during a clinical trial and not regaining the functionality lost during the decline. Because CIDP patients work so hard to adapt to their new normal, it seems they are reluctant to put that new normal at risk without a promise of major benefit for themselves and the community. Dr. Pasnoor reminded everybody of rescue protocols often utilized by clinical trials, but the panelists still agreed that declining while living with CIDP, whether because of a placebo arm or because the interventional treatment was not effective, invoked extreme anxiety and might prevent patients from enrolling in a clinical trial. Betsy reiterated this point that succinctly summarized the overall attitude:

”

So looking at that from a scientific point, you almost need placebo, but from a patient perspective, then it absolutely terrifies me that the fear of not having that medication to keep my nerves completely fine so that I can go about daily living so I have my mobility and ability to interact with my family, children, and colleagues, that part could potentially dissuade me from joining a clinical trial, especially after the washout period so there's a lot of nuances to this clinical trial design that need to be addressed and investigated to ensure that we really are protecting patients.

PATIENT QUOTE

“So looking at that from a scientific point, you almost need placebo, but from a patient perspective, then it absolutely terrifies me that the fear of not having that medication to keep my nerves completely fine so that I can go about daily living so I have my mobility and ability to interact with my family, children, and colleagues, that part could potentially dissuade me from joining a clinical trial, especially after the washout period so there's a lot of nuances to this clinical trial design that need to be addressed and investigated to ensure that we really are protecting patients.”

The panelists progressed the discussion by ruminating about how patients learn about clinical trials, noting that access to an expert who is “plugged into” the most cutting-edge research was essential. Theoretically, any physician can learn about the clinical trials going on, but the panelists expressed much more confidence in a physician who is an expert on CIDP, the mechanism of the trial, and the science behind it. Additionally, the panelists agreed that patients often have to be their own advocates and do some of their own research if they are interested in clinical trials, mentioning resources from the GBS|CIDP Foundation International and clinicaltrials.gov. Most importantly, all panelists agreed that their doctor's input on whether to participate in the trial would be instrumental for them, but also pointed out that patients without access to specialists, especially in rural communities that may not have a trial site near time, have a different experience in learning about clinical trials and deciding to participate.

The panel concluded with some questions that were posed by the audience, sparking brief conversations about the potential benefit of home infusion during trials (potentially useful, but some expressed the desire to be in a hospital if being given an experimental drug), the level of care given by trial staff, and when a person might advocate to their doctor to participate in a trial. Dr. Pasnoor closed the panel with a hopeful summary that reminded panelists and audience members that researchers also want to see improvements for CIDP symptoms and quality of life, with optimism that a healthy partnership to design patient-centric trials could bring more participation from patients.

key takeaways

1. Washout periods in clinical trials are challenging for CIDP patients who fear a return of their CIDP symptoms can lead to irreversible damage and disability.
2. CIDP patients want to know all of the details of a trial before signing up.
3. The role of a doctor in a CIDP trial is important, but patients aren't afraid to be their own advocates.

5

PANEL 4

Potential Future of CIDP Treatment

Each panelist emphasized how IG therapy had improved their overall symptoms, so the discussion focused more on whether their treatment plan allowed the panelists to live their best lives with IG therapy, often focusing on the administration and how that impacts their lives.

The final panel of the meeting was moderated again by Lisa Butler. Lisa allowed the four panelists, Nancy D. from Palermo, IT, Angela P. from Colorado, Linda P. from Ottawa, and Corbin W. from North Carolina, to introduce themselves and share a quick overview of their CIDP story. Again, all panelists mentioned a long road to actually receiving diagnosis and experiencing symptoms such as weakness and numbness that they, and their doctors, tried to attribute to other things before exploring a neurological diagnosis. Each panelist also highlighted ways in which their life has now changed; Nancy, Angela, and Corbin mentioned the flexibility given by Subcutaneous IG therapy and Linda remarked that the IVIG is what “keeps her going.”

Each panelist emphasized how IG therapy had improved their overall symptoms, so the discussion focused more on whether their treatment plan allowed the panelists to live their best lives with IG therapy, often focusing on the administration and how that impacts their lives.

Corbin discussed the positives of switching from IVIG to Subcutaneous IG and the flexibility that switch gave him, saying “There was some convenience associated with all of that. Therefore, allowing me to plan other things in my life without being so focused on that. It also gave me some ability for an increased mental clarity. Before I had some pretty strong medications that I had to take with the IG and the infusion itself often caused me to have some significant flu-like symptoms and recovery issues at the end.” Nancy echoed how the switch positively impacted her life, saying, “I am able to work again, which is important for me, which makes me feel independent and makes me feel that I’m still a little bit healthy and capable of living a normal life. When I was on IVIG, it did help me, but I felt like I had to put my life on hold. It was something that I would always say, and putting it on pause, because I would have to take sick days off, and I have to drive to the hospital which was in Palermo, and it was pretty nauseating.” Angela also agreed with the positives of switching to subcutaneous IG, mentioning that she is able to work and her schedule is more flexible. On the other hand, Linda explained why IVIG was her preferred method, saying “I had considered subcutaneous IG, but I like the idea of going to the hospital once a month, getting it over with, and that’s all I have to do. I also like the safety of getting it done in a hospital.”



To focus the conversation around how life might be better for CIDP patients, Lisa posed a question to the panelists and the audience, asking them to choose from a list of options which would be the most appealing improvements to their treatment. The options included: shorter time of administration, fewer needles, a better device/mechanism of injection, a way to independently give myself treatment, less frequent administration of treatment, less invasive administration of treatment, such as an oral medicine (i.e. a pill), a cream, or something with an auto-injector (microneedles). The most popular choice from that choice, according to the audience, was a less invasive administration of treatment. The second most popular choice was less frequent administration, and third most popular was a way to independently give treatment.

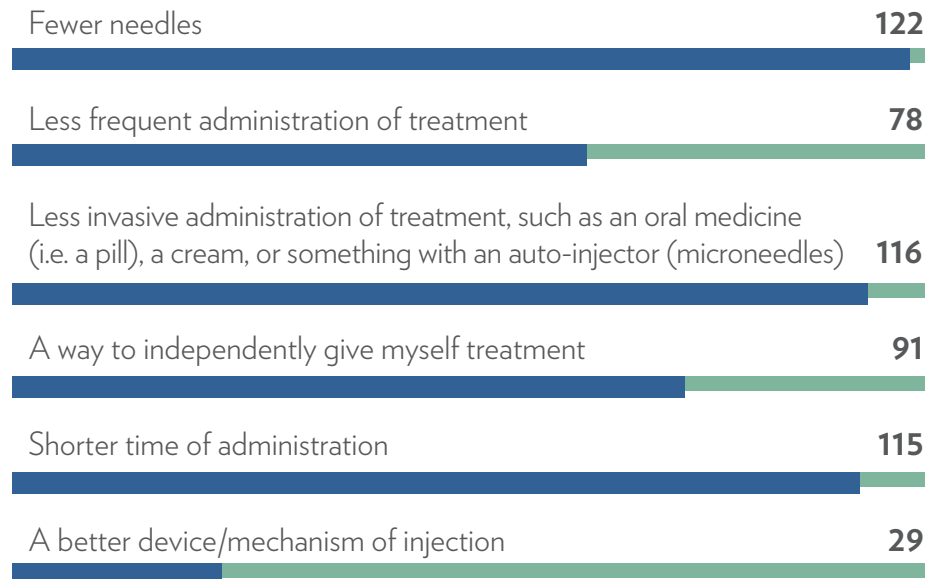
This question was also included in the post-meeting survey to the wider CIDP community, and results were similar to the live-survey during the meeting. Results were: (people could select multiple choices)

People wrote in to explain their choices:

(cont'd on next page)

WHICH OF THE FOLLOWING WOULD BE THE MOST APPEALING IMPROVEMENT TO YOUR TREATMENT?

Select as many as you like. (n=184)



”

Not having to plan my infusions to my providers' hours of work would be great. I could administer it after work at my convenience allowing me to not have to make up time missed on the weekends.

PATIENT QUOTE

I could take a vacation or make an appointment and still get treatment without having to be at a specific location.

Not having to plan my infusions to my providers hours of work would be great. I could administer it after work at my convenience allowing me to not have to make up time missed on the weekends.

Would like something that last longer than a few weeks like treatments in MS.

Now if there was a treatment that met these qualifications, I definitely would be open to it because my life pretty much revolves around my treatment and I hate needles but I'm afraid to try something new because it's worked so well for me.

It's just not fun having to jab needles into your legs every week.

The panelists echoed some of these sentiments in describing the improvements that they would like to see. Corbin described that while the Subcutaneous IG has facilitated more vacations, flexibility in schedule, and independence, that there are still limitations to IG therapy overall. Specifically, Corbin noted that anything that interacts with the immune system is nerve-racking during the pandemic. Also, Corbin described that IG therapy has not brought back all of the functionality he lost because of CIDP. He noted, "I am much more functional today as compared to crawling up the steps. I am much more functional today in that I can maybe walk from my car into a grocery store, but don't ask me to walk more than a couple blocks. Then I am looking for walking assistance or other things. It's a relative term to say I am much more functional, but that's still not where we really want to be."

Angela weighed in on an area that wasn't touched on in the survey, which is wanting a medicine with fewer side effects. She said "The biggest thing for me would be less side effects. I am still on corticosteroids, which causes brain fog. It causes weight gain. It can affect blood sugar levels, calcium coming from your bones. There are so many different side effects to steroids, but they are effective. If we could have something like steroids without those side effects. Subcu, I get swelling at my infusion sites, and I have five of them on my stomach. It can be painful and itchy. It makes it really uncomfortable...The independence is great, but there are still some things that could definitely use improvement." Nancy echoed this sentiment about the infusion sites from subcutaneous IG, saying "Of course, I am grateful because there is something that at least doesn't give me a relapse at the moment. It's also hard to deal with the side of the injection needing to stay clean for you, sometimes it gets puffy and red...That is kind of hard, especially in the summertime. I love to go to the beach but I can't because there is the puffiness and it's red. You don't want to get saltwater on that. Yeah, there are a lot of positives of the treatment, but, of course, there are a lot of down notes on it."

As the moderator, Lisa honed in and asked Angela for more detail on the mental toll that subcutaneous IG might take on a person. Angela described her situation:

I have skipped treatment because I just mentally did not feel like sticking myself five times and then sitting there while fluid goes into my body, and I know it's going



to make me better, but for those three hours I am stuck in my house. I can't really do anything. It's hard to even get up and get a glass of water. It is mentally draining to have to do that. I don't like needles, so I don't know anybody that does like them, but I still have been doing subcu myself for almost a year and a half. I still get the sweaty palms and sometimes I get shaky, even more so than normal. It is just very mentally taxing to have to do this every week, on top of normal pills that we all have to take.

Corbin agreed, saying “...I won't say I skipped treatment. I've deferred treatment because of schedule convenience, inconvenience, coming back on vacation, getting back in the middle of the night on a Sunday and I've put it off a day or two, but for me putting it off a day or two, I begin to feel the effects of that. I have deferred it for a week one time.”

Linda, referencing her IVIG regimen, expressed a limitation on where she lives because of a strict adherence to her treatment schedule. Linda described that deviating too far from her schedule can result in setbacks to her symptoms, so she is cautious to not disrupt her schedule or her system. She said “I am scared to move. I have considered moving to be closer to family, but I'm scared to have to try to set up new appointments, find a new neurologist, get the treatments set up, and what the gap would be like.”

Overall the attitude about what could be improved focused on finding ways for CIDP patients to live a life that was not dictated by treatment schedules, side effects, and the rollercoaster of fatigue/mobility that comes between treatments. That reflection of what could be improved segued the conversation into finding out what gives the panelists hope for the future. Each panelist weighed in.

Nancy: *The new studies, the research, those who dedicate their time trying to find a better treatment, a key here that one day will free us of this...I wish that we could have a positive outcome for let's say a life that doesn't, like I said before, chain us to our bodies and our homes and to a town that may be want to change. Those privileges that a healthy person has. You only notice them that you don't have them when you feel that you can't make changes.*

Corbin: *To look back on that and see what has unfolded. To see things to begin to unfold now. It gives me hope to know that with all of this technology, we are learning more. Our physicians are becoming more knowledgeable. They are gaining more analytical capabilities...Our community here, the active collaborations and partnerships that occur with our organizations, governmental organizations, and within our community all the support and education and research that takes place with the medical researchers and medical team. I mean, all the stuff that's happening here gives you that sense that you are not alone in this thing.*

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PATIENT QUOTE

Linda: *I am really thrilled that through the foundation that there is research going on all over the world and that doctors are working together. I have hope that they will come up with another treatment. If they don't, I still have hope because I am alive. I have a life. I live independently, and IVIG does that for me.*

Angela: *...there's options now but I know there are more options to come. That's what gives me hope... looking forward at where I was, where I am now, and my potential. That potential is what I really cling to.*

IG therapy has been life-changing for many CIDP patients and effective for its intended purposes. Because continued usage and availability is critical, it seems that the administration of this therapy is ripe for innovation that could enhance the quality of life for people living with CIDP. Patients also seem more cognizant of the mental, emotional, and social aspects of CIDP that are not fully treated by IG therapy. Addressing these issues may call for an additional therapy, additive to IG. The future for CIDP patients is bright because of thoughtful partnerships that prioritize the community's needs.

key takeaways:

1. Improvements have already been made in the way that CIDP is treated, but patients are still burdened by their treatment regimen.
2. The side effects, regimented schedule, and untreated symptoms remain areas that CIDP patients wish to see more improvement.
3. Though an ideal treatment for CIDP, according to the community, is a once-daily pill, the community remains inspired by the research currently happening and encourages innovation to continue.

6

Conclusions

The use of IVIG, steroids, and other accepted treatment options has allowed patients with CIDP to find a sense of normalcy in their lives as they adapt to new levels of disability, new treatment regimens, and new mental, emotional, social challenges.

CIDP is a rare, debilitating neurological disorder. The use of IVIG, steroids, and other accepted treatment options has allowed patients with CIDP to find a sense of normalcy in their lives as they adapt to new levels of disability, new treatment regimens, and new mental, emotional, social challenges. However, CIDP patients are still struggling despite the existence of treatment options. The intense treatment regimen, looming threat of product shortages, and financial burden posed by IG therapy create new challenges for CIDP patients beyond the neurological disability caused by the disease. Further, not all symptoms of CIDP are effectively treated by currently available medications, with fatigue and pain being significant invisible burdens in the CIDP community.

Fortunately, innovation in the CIDP space is currently hopeful. The GBS|CIDP Foundation International brought the CIDP community together with key opinion leaders through this EL PFDD meeting to ensure that innovation happening in the CIDP space considers the needs of people living with CIDP. It is also essential that the innovation process remains patient-centric, as it is clear that traditional trial designs are burdensome for people living with CIDP. Innovation in the space requires creative strategy from all stakeholders.

Trial design may improve with patient input and help dispel fears and misunderstandings. The current activity in innovation for CIDP is growing. Yet, as a rare disease, the availability of trial participants may face limitations. Meaningful partnerships can help inspire the patient and research community to work together to help clinical trial recruitment succeed, thus fostering innovation.

The Foundation hopes that the stories shared by panelists and survey-responders during this meeting push innovation towards a future where potential future treatment options of CIDP treat all symptoms — physical, mental, emotional — and allow CIDP patients to improve their quality of life before their diagnosis. With cooperation, innovation can improve, and save, lives of CIDP patients.

7

About the Foundation

Our vision is that every person affected by GBS, CIDP, or related syndromes will have access to early and accurate diagnosis, appropriate treatment, and knowledgeable support services.

The GBS | CIDP Foundation International is a global nonprofit organization supporting individuals and their families affected by Guillain-Barre' syndrome (GBS), chronic inflammatory demyelinating polyneuropathy (CIDP), and related conditions through a commitment to support, education, research and advocacy.

We improve the quality of life for individuals and families affected by GBS, CIDP, and related conditions. Our unwavering commitment to the patients we serve is built on four pillars: support, education, research and advocacy. We support patients by nurturing a global network of volunteers, healthcare professionals, researchers and industry partners to provide them with critical, timely, and accurate information. We educate doctors, clinicians, patients and caregivers to increase awareness and understanding. We fund research through grants, establishing fellowships and other appropriate avenues to identify the causes of and discover treatments. We advocate at the federal, state, and grassroots levels to educate policymakers and help them make informed decisions that benefit our patient community.

Our vision is that every person affected by GBS, CIDP, or related syndromes will have access to early and accurate diagnosis, appropriate treatment, and knowledgeable support services.

The Foundation is an international organization of 30,000 members with 200 volunteers in 49 countries, all of them dedicated to providing support and assistance to GBS/CIDP patients and their families. The Foundation continues to be funded by contributions from individuals who have been personally touched by GBS, CIDP, and other variants, as well as by corporate donors.



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8

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9

APPENDIX 2 Finalized EL PFDD Agenda



Voice OF THE PATIENT
SUMMIT



GBS|CIDP
Foundation International

FRIDAY, MARCH 25, 2022

Externally-Led Patient Focused Drug Development Meeting on CIDP

The GBS|CIDP Foundation International is leading the effort to characterize the patient journey and bring the patient voice into the drug development process. We will continue to utilize a patient-reported information registry and robust support and education programming to accurately describe the patient experience that also encourages self-advocacy in both clinical and research settings. The Foundation hopes that an externally-led Patient Focused Drug Development meeting will elevate the CIDP patient voice and increase understanding of patients' preferences when it comes to tolerable side effects, most hindering symptoms of CIDP, and a better understanding of the progressive disability associated with poorly managed CIDP. Ultimately, the Foundation aims to bring the patient voice into the drug development process. Learn more now at www.gbs-cidp.org/voice-of-the-patient-summit/.

To the right is our Patient Centric Program Agenda for the EL-PFDD on March 25, 2022. All are welcome to attend this virtual event. Times listed reflect Eastern Standard Time Zone (EST).

OUR SPONSORS



AGENDA

10:00 - 10:05 AM

Welcome & Expectations

Jim Crone, *President of Board of Directors*

10:05 - 10:15 AM

Opening Remarks from FDA

Dr. Michelle Campbell, *Division of Neurology*

10:15 - 10:25 AM

Brief overview of CIDP

Mechanisms & Diagnosis

Dr. Peter Donofrio, *Global Medical Advisory Board Chair*

10:25 - 10:35 AM

Current Landscape of CIDP

Treatment Options

Dr. Jeff Allen, *Global Medical Advisory Board Member*

10:35 - 11:30 AM

Patient Panel 1: Diagnosis & Current Treatment Options

MODERATOR: Dr. Mamatha Pasnoor, *Global Medical Advisory Board Member*

PANELISTS: Crystal Sada, Julie Bell, Barry Fortner, Diana Christian

11:35 AM - 12:30 PM

Patient Panel 2:

Everyday Burden of CIDP

MODERATOR: Lisa Butler, *Executive Director*

PANELISTS: Michele Dearing, Kelly McCoy, Jon Schandler, Victor Sheronas

12:30 - 12:45 PM - BREAK

12:45 - 1:40 PM

Patient Panel 3:

CIDP Trial Design

MODERATOR: Dr. Mamatha Pasnoor

PANELISTS: Betsy Blake, Lynn Rogers, Dave Rosner, Dave Tuck

1:45 - 2:40 PM

Patient Panel 4: Potential Future

of CIDP Treatments

MODERATOR: Lisa Butler

PANELISTS: Nancy DiSalvo, Angela Patton, Linda Paul, Corbin Whittington

2:40 - 2:45 PM - BREAK

2:45 - 3:00 PM

Summary of the Meeting

Closing Remarks

Lisa Butler

10

APPENDIX 3

Testimony from Each Panelist

(In order given during EL PFDD meeting)

JULIE B. *from Texas*

I would like to thank you all for taking time out of your day to listen to not only my story, but all of our stories about our daily living with CIDP.

I am Julie Bell and I live in San Antonio, Texas. I have had CIDP for 27 years. I was diagnosed at the age of 27. I began showing symptoms of aching hips and an awkward gait. It was Thanksgiving of 1995. I flew to my parents house with my seven month old son. My mother a nurse and my father a doctor noticed my awkward gait. My dad suggested I go see my primary care physician and get a referral to see an orthopedic doctor. I went to my primary care physician who had me stand on my toes and heels. I thought this doctor is crazy. Who goes around on their toes and heels? The doctor ran some blood tests and sent me on my way with my script to see an orthopedic doctor.

The next day I was at work and got a phone call from my doctor saying that my CPK level was elevated. I was going to see a neurologist at 1:00PM that day. At that moment I knew this was a serious situation. I had EMGs with nerve conduction, muscle biopsies, spinal tap, and lots of blood work to determine my diagnosis of Chronic Guillain Barre Syndrome. This occurred in a 1-month time period.

Back then the treatment was plasma pheresis. I had treatment for 28 days in a row. The doctors would say how do you feel and I would say, "How am I supposed to feel?" "You are the doctor." I discovered at that point in time I really had to learn to get in touch with my body and understand how my body was feeling. The 28 days in a row drained my blood cells resulting in the need for blood transfusions. I then started high doses of steroids with horrible side effects. I had weight gain and chronic diarrhea. The only way I got out of jury duty was to tell the judge I will serve but if I say I must go, you must let me go. I was referred to by my late husband as a Russian weightlifter because I gained so much weight.

I went to UCLA and Dallas and got 2nd and 3rd opinions within the next couple of years. I was on various immunosuppressant's. I've taken different products throughout the 27 years. You name it, I probably tried it along the way. 27 years is a long time. I have a Medi-port. I had one that lasted for 15 years. I used to call it Old Faithful. I'm on my 2nd port. I currently receive treatments every 2 weeks. I have infused in various settings. I have been infused at the hospital, oncology office, neurology infusion suites, and at home. I currently get my IVIG treatment at home.

It's been determined I am IV IG dependent. Getting it for over 20 years. I tried a trial medication where I had to stop taking IVIG. Within 5 days of not receiving IVIG I was declining physically. I immediately started trial medicine. After 3 weeks my Dr. and I made the decision to discontinue the trial. I could not walk well. I had tremors and was unable to get my food up to my mouth. I was very weak. I went back in IVIG and it took about 6 months for me to recover and get back functioning I had lost.

Life has come with a lot of changes. In 2017 I made a tough but necessary decision to retire at the age of 50. I could no longer work full-time as a special education teacher of 23 years. I wear ankle orthotics because of drop foot in both feet. I'm losing functioning in my hands. I can no longer straighten my hands. My balance is affected. I live in constant pain. I have a hard time buttoning, zipping, any fine motor skills, and opening certain containers. Life is just difficult. The disease has made taking care of my grandson. I cannot safely carry him. I'm slowly losing my independence and that is very scary. Overall, the disease has given me hope and allowed me to be involved. The disease has inspired me to advocate not just for myself but for other patients dealing with GBS and CIDP. Thank you.

DIANA C. *from North Carolina*

Hi, my name is Diana Christian and I live in Asheville, NC. My CIDP story began in 1979 when I was 4 1/2 years old. Over a few months my parents noticed I was falling down a lot, I was extra-clumsy, and fatigued easily. GBS was diagnosed by a lumbar puncture at the hospital, which I remember as a terrifying test at that age. My limbs became nearly paralyzed so I was admitted for a week, my breathing was monitored, and I was treated with oral prednisone. I later had PT and attended the first part of kindergarten in a wheelchair and later in leg braces.

I declined and relapsed at least twice after the initial diagnosis, which was updated to: chronic recurrent GBS, what we now think of as CIDP. My parents remember a time filled with fear and anxiety over my health, and taking turns staying awake overnight to listen to me breathe. But we also felt joy as I improved. Overall, after age 6, I lived a relatively normal life until the age of 27.

I was a newlywed, had recently graduated from my master's program, and was starting my career as a speech-language pathologist when fatigue and weakness once again began to show up and grow to a crushing level over a few months.

One morning at work I fell a couple of times in the hallway when my legs gave out, and I felt tingling and numbness in my limbs. CIDP relapse was diagnosed at the hospital and soon after I was started on IVIg and steroids which began to give me some stability. To confirm the diagnosis and get a reliable treatment plan, I had 4 other consultations with distant experts and repeated tests. It was a scary, uncertain, and expensive time for us as newlyweds.

In 2011, after 4 years on my subcutaneous Ig, my preferred brand was discontinued and I had an allergic reaction to its replacement with aseptic meningitis. I took Prednisone for 14 months to treat the meningitis. It was extremely difficult to tolerate, and the meningitis kicked off a chronic migraine disorder that I have been living with for the last decade.

Unfortunately I haven't been able to return to work and we made the difficult decision not to have children because of my disability. My CIDP is now accompanied and exacerbated by my chronic migraine and another autoimmune disorder: psoriatic arthritis. I continue to have fatigue, lack of physical endurance, and persistent weakness and nerve pain. Over the past 2 decades I have used a wheelchair and walker to assist me to varying degrees. My CIDP symptoms also include autonomic nervous system dysfunction.

I miss being athletic and as active as I used to be, and wish I could have experienced more of the career I had just begun. But my involvement with the foundation as a volunteer and helping fellow patients keeps me hopeful and inspired by the strength and resiliency of others living with CIDP and GBS. I volunteer with other groups at times, and I try to engage with my incredibly supportive friends and family as much as I am able. I'm so grateful that my husband has been such a loving and supportive partner through this journey.

I sincerely thank you for listening.

BARRY F. *from Texas*

Thank you for letting me be a part of this and I'm very excited to tell my story. I have been married for 29 years, 3 kids. I'm embarrassed to say how much I love my 2 dogs. I like to play golf and I'm a clinical psychologist focusing in health psychology. Currently the head of medical affairs for -- in the US oncology business unit and my comments will be about my personal story and not representative of -- my story starts 7 years ago. I was doing a presentation on a weekend and between flights in Charlotte I heard loud slapping sounds and thought someone was following and it was foot drop. I was crushing my feet against the ground trying to get to the airport. That was Saturday night and by Monday I could not roll over and move my arms or legs and was in sheer panic that I was going to be fully paralyzed. I was lucky that that morning there was a neurologist rounding who recognized the ascending paralysis as GBS — received

IV IG that day which I believe stopped the symptoms and I was very grateful not to have been intubated. That began 7 years of multiple medications and treatments. My story has been one of ebb and flow. Currently functioning fairly well but live with pain and the severe burning with my feet. I remain as functional and active as I possibly can and that is certainly my goal in my I'm so grateful that you are here today listening to our story so we can try to find a way forward with this disease.

Thank you.

JIM C. *from Illinois*

Thank you and thanking everyone for the opportunity to participate in this panel and this event today. I want to mention that I am not Crystal Sada. She was scheduled to be on this panel and unfortunately she is under the weather today which can happen with chronic conditions and immunosuppressant's and whatnot. She was unable to join us in the fine folks at the Foundation asked Me to fill in. I wish crystal the best and I will do my best to fill in on her behalf.

My story begins when I was 25 years old. At that time I was a perfectly healthy individual. My last physician prior to this journey as a pediatrician and my wife said to me we should probably become adults and get real doctors and have internists or family medicine doctors. We were scheduled to do that. My story began before that was able to happen. As I like to say I have been able to live the best of both worlds in terms of GBS and CIDP. I had a cold a couple weeks prior to my symptoms coming on and my symptoms progressed rather rapidly. I sort of dismissed it as being very tired or having fatigue and things like that. Just thought I was ill and needed to rest more. My wife is a physical therapist, so she just evaluated me one day and decided I needed to go to the ER immediately. I am lucky that we do have a Regional Medical Center that was within 5 to 10 minutes of our home that has a specialty in neurology so they were able to fairly quickly diagnose my GBS. I presented a little awkwardly to them, but I quickly progressed to a typical GBS and was immediately transferred into ICU at that point. I was fully paralyzed and on a ventilator, and stayed in that state for about 6 weeks where my brain was fully functional and I knew I was there but had no means of moving, breathing other than dependent on a ventilator. My only ability to communicate was blinking my eyes once for yes, twice for no. I felt trapped within my own body. After that 6 weeks, I began to progress and recuperate and was able to move into outpatient therapy and eventually return to regular life. I returned to work and at that point I thought my adventure with what I thought was an acute rare illness was over and I was going to go on living life. About 6 months later, I started having symptoms again and immediately reached out to my neuromuscular specialist who got me immediately in and did an EMG in his office very quickly. Afterwards, he said "you have CIDP, get your belongings at home, you are getting admitted to the hospital ASAP and we will start a round of IVIG and treat it from

there.” Moving on from there I did have hospital stays on and off as exacerbations happened, but I had a variety of medications which we can talk about in a little bit that included IV IG immunosuppressant’s and steroids and things of that nature. I really did that for about 10 years. I was doing IVIG about every 4 weeks and then progressed to 3 weeks, 2 weeks and ultimately 10 days as I noticed the need to take it otherwise symptoms would progress. At that point, being in my mid-30s with a wife and 2 young children and a regular dependence on IG, I knew that I needed to perhaps do something a little different and looked into some clinical trial options and did select one option which was a stem cell transplant that was successful in my particular case. It certainly is not a typical or front-line treatment by any means. That treatment is still in investigation at this point, but it put my CIDP into remission and I’m grateful for that. That’s why I want to pay it back or forward and support all the patients that are involved or still have active CIDP or GBS at this point, and I’m joined in with the Foundation in terms of volunteering and chairing the board. It’s been an interesting, rewarding journey as well, and I think we will also hear people mention that while there is the pain-and-suffering, there’s also some good experiences that come out of it, or some interesting stories and things like that that are unique and would only have come from going through rare conditions such as this.

MICHELE D. *from Maryland*

In 2001, at the age of 26, my life changed forever. I was released from a specialty hospital in May 2001 after five months in the hospital due to complications from a surgery. I had survived an acute respiratory issue that required me to be placed on a vent and in a drug-induced coma. I was on the road to recovery, though forced to leave my home in West Virginia to move in with my mom and step-father in Alabama who served as my caregivers until I recovered. While visiting family in July 2021, I was laying on the couch when my mom placed a blanket over me. I immediately noticed that the blanket hurt my skin. Fast forward to the next week, I began to trip and fall. I chipped my front tooth during one fall. I could barely stand. I immediately saw a neurologist. Within days, I was unable to walk or use my hands. I had temporary paralysis. I was diagnosed with Guillain Barre Syndrome—the cousin to CIPD. Never-ending pain, tingling, numbness, weakness, and electric shocks would rule my day-to-day life. At times it seemed like too much to bare. I imagined myself permanently unable to walk or care for myself. And in moments of weakness I imagined death was the only possible solution to this pain. I was angry...why was this happening to me? After a few months at an in-patient rehab facility, I spent the next few months at an out-patient facility undergoing physical and occupational therapy. I spent months in a wheelchair and then slowly progressed to using a four prong cane. The next 2 years were filled with uncertainty, pain, tears, and regular doctor visits. By 2004, despite the odds, I was able to return to practicing law and move out from my parent’s home. Aside from a routine visit to the neurologist every 6 months, my life was back to some level of normalcy.

And then there was 2010. While getting out of bed, I noticed a feeling in my legs and feet that felt hauntingly familiar. I could barely stand. Was I about to fall? Was I about to be paralyzed again? Now living in Maryland, I found a new neurologist that conveniently was located within walking distance to my work. After several tests, I was diagnosed with CIDP. He initially prescribed steroids. After little improvement, he ordered IVIG infusions. IVIG is a plasma product. Who would imagine that plasma that I donated in college for shopping and drinking money would be the very substance that treats my condition today. For the next 10 years, I would have infusions. At first, I had infusions with the help of a home health nurse, that consisted of a 5 day loading dose and thereafter for a period of 3 days at my home (each one lasting about 5-6 hours) every 3 weeks. In-home infusions allowed me to telework with less interruption to my career. With the demands of work and the roller coaster of flareups, I was forced to work a reduced work schedule for reduced pay for a few years. Not only did I have to deal with physical and emotional challenges associated with this condition, at this point, I now had to face the financial burdens of CIDP. It was during that time that I switched to subQ infusions of Hizentra in order to return to a full work schedule. Terrified at first to inject myself with about 6 needles, I managed to appreciate the freedom of administering the infusion myself. Despite the infusions, the benefits began to plateau. In January 2020, I was referred to Mayo Clinic in Minnesota where I underwent a week of testing. All physicians have landed at the same conclusion.... this is a condition that I will have to manage for an undetermined amount of time. Excessive fatigue, pain, tingling, numbness, weakness and electric shocks continue to be a part of my everyday life. Despite the ups and downs of this terrible disorder, I'm still standing and fighting against the everyday burdens of CIDP.

KELLY M. *from Pennsylvania*

Good morning, everyone.

My name is Kelly McCoy, and I'm from Philadelphia, Pennsylvania. My personal journey began at 28 years, working as a financial marketing manager. I had been sick for a few months prior with what my doctor called the June flu. While on vacation, I had a bout of food poisoning I assumed was the cause of my general malaise. One day on my walk down the stairs of my apartment, I took a fall down the entire flight. I sat at bottom shaken. My feet did not listen to my brain, and my legs just buckled underneath me. I made an appointment with my physician that day. Eight months rolled by. I was terrified of waking up every morning to discover another ability that I had lost. One day I couldn't open a jar, then I couldn't lift my arms to dress myself. Eventually, I was army crawling to and from the bathroom dragging myself back into bed where I'd spend my day unable to pull a comforter over my own failing body. After being dumped by two neurologists who lumped me into the ALS bucket since they weren't sure what was going on, I found one that changed my life. She decided to try — and after five days of a loading dose,

I squeezed her hand tighter than I had before. The first two years were some of the — times of my life. I would spend four days a month hooked up to a machine. I would spend the next few weeks unable to do anything. Didn't feel I was getting anywhere. The eight years that have brought me to today have been stepbacks, dosing changes, more needles than I ever came to think about, painful PT, at the end, I am gaining my independence by working again. My journey has been long, but it's given me back more than it's taken in some ways. Seven months ago, despite everything, it gave me my daughter who inspires me to look toward the future with hope. My treatment is currently a cocktail that works and is sufficient for this stage in my life but it's far from perfect. Living through a pandemic has been at the very least nerve-racking. The side effects of steroids have been hell. In my pockets of time, I'll find my mind wandering if I'll be fortunate to carry my daughter, if when she's old enough, I'll be able to play with her or be the parent she deserves. I choose to embrace what's CIDP has brought into my life. What it has stolen can not be discounted. Thank you for allowing me be part of this story and be a part of this inspiring group.

JON S. *from New York*

My name is Jon Schandler. I live in the suburbs in New York City. Diagnosed with CIDP June 2015. Probably in the case of many patients, subtle changes -- feelings and well-being. One day, feeling okay, maybe tired. Soon after, sensations of something had taken over my body. I experienced weakness in my lower extremities, significant pain, cramping, muscle pain. This progressed over four to five weeks. I discounted it because I felt that I hadn't exercised over the winter, I was turning 65, and this is what happens when you get older. Slowly, it became a normal event for me to not move my legs. I needed the use of one or two canes to walk, depending on how I was doing. My balance was an increasing problem. I was a CEO of a local hospital for 40 years. I recently retired from that position. A lot of my friends and colleagues decided they were going to figure out what is going on with me. I had EMGs, spinal taps, complete screenings, gallons and gallons of blood taken.

I was fortunately diagnosed rapidly and treatment began. My diagnosis included no reflex from my waist down. Failed neurological exam, trying to stand up and close my eyes and extend my arms. I would just topple right over. Massive protein in my spinal fluid. Both sides of my body, the EMGs showed significant demyelination. As many know, my immune system had begun attacking the myelin surrounding my nerves and short circuited the signals to my arms and legs. Manifested itself by terrible weakness, spasms, hundreds a day, exhaustion. I was first treated with — and then transitioned to IVIG. At the time we were concerned with the IVIG, if it would work. My neurologist wanted to give it 90 days. After 90 days, I was getting worse and worse and worse. He said although the prevailing thought was that it would take 90 days to see a response, he said let's try 120 days.

Still no response. At 150 days, I started getting positive results. I appreciate his willingness and interest in continuing to stay with me. After about 18 months of treatment, I was stabilized, about 80% back in terms of my strength and what I could do, as long as I constantly had the IVIG infusions.

I've had a number of relapses over time, which is very frustrating because you feel like you've got it under control, and then all the sudden, I start falling, slipping, I can't do things. Two interesting factors that occurred for me. When I was being analyzed and screened, they found a lung cancer tumor on my right lung, which probably never would have happened if it wasn't for CIDP. I had that removed in 2015, so to some extent, I think CIDP is one of the reasons I'm actually alive today, because nobody would have ever seen that. Fortunately, it was a stage one lung cancer. The other thing that happened recently is that I started falling fairly frequently. The neurologist felt the falling was not due to CIDP but due to spinal is a noesis. Six weeks ago I had surgery to repair that. I appear hopeful that they're right and that a lot of what I was feeling was spinal cynosis. Although I'm still numb in my feet. I appreciate the opportunity to tell my story, and I'm inspired by everybody else on this call.

VICTOR S. *from Pennsylvania*

Good morning, I am Victor Sheronas. I am 81, retired and live in Glenmoore, PA...a far-western suburb of Philadelphia. I have symmetrical CIDP; I was diagnosed at least 10 years after the onset of symptoms.

I was first diagnosed about 13 years ago. My diagnosis was prompted by a neurosurgeon who was, fortunately, not so quick to operate. I had always been a tad clumsy but had increasingly been tripping over blades of grass and breaths of air. After checking with some doctors, we have a diagnosis...spinal stenosis. After various treatments for the stenosis, I consulted with two neurosurgeons to find out "what, if anything, would and could surgery do for my stenosis?" The first one showed me MRI results showing calcium build-ups at the L3-L5 discs which were irritating the nerves to my feet. That could explain my drop foot; this prompted my spinal laminectomy.

The second neurosurgeon said he could do something but refused to operate until after I had seen a neurologist. He was quite certain that something else was going on. I deferred seeing a neurologist for a while — in hindsight, a regrettable and costly decision — because I felt so good after the laminectomy. One day, I'm returning home from a lovely power walk. Feeling chipper, I decided to jog the last few hundred yards. My left leg wouldn't come forward fast enough on the second step — toe drag — so I dropped like a sack of potatoes. Then I got serious about seeing that neurologist.

The first neuro was OK and somewhat familiar with CIDP. He referred me to a specialist who knew well CIDP & GBS. He diagnosed & confirmed my CIDP with an EMG test. He believed that a spinal tap would be useless because of my spinal stenosis.

My first treatment was with steroids. They had no effect and was thus stopped after 6 months. I have been on IvIG ever since. I've tried 3 different brands and have found that the brand I began with has provided the most beneficial and noticeable results.

My treatment history has also been a physical journey combined with various protocols:

- I've been infused in 4 different locations before settling on a center close to my home.
- I've been infused in both an out-patient and in-patient situations.
- I've been infused in a hospital, hospital-affiliated infusion center and a third-party location.
- Frequency: I began with monthly visits and have now settled on bi-weekly visits.
- Dosage amount: I began with 200 grams spread across three consecutive days at monthly intervals. I am now at, and have been for years, 50 grams every other week.
- Dosage rate: I began with 5 hours for a 50-gram bottle, a rate even more conservative than the manufacturer's fine print on the instructions. I now receive 50 grams in 2-1/2 hours
- My age and history of DVTs have been complicating factors. My primary neuro was worried about strokes and thus wanted either to wean me off IvIG or at least find the minimum dose I needed. We tried mycophenolate mofetil for the weaning experiment...no success. We also went as low as 40 grams biweekly to find my minimum dose. Yup, it's 50 grams biweekly.

The years-long interval between symptom onset and diagnosis caused me to suffer significant nerve damage, which resulted in loss of strength and muscle function. Since diagnosis, I have also suffered a reduction in the transmission speed of my nerve impulses as indicated by a recent EMG. I have learned that:

- I am now a "prisoner" of IvIG or SubQIG.
- I will never get any better.
- I am also a prisoner of Physical Therapy...use it or lose it! Targeted PT, guided by a person knowledgeable with CIDP or neuropathies, is essential...just like breathing is.

Having been active all my life (swimming, crew, wood splitting, power walks, weight lifting, catches, chores at home, etc.), I haven't gleefully accepted — nor with grace — the new normal of my physical limitations centered on balance and

strength. My limitations are now the source of my lows...details in a few minutes. My highs and inspirations come from deepened relationships and friendships along with a willingness to be more open and vulnerable. Most of these highs have been inspired and instigated by the GBS/CIDP Foundation's family of patients, staff and caregivers. Their collective can-do attitude is astounding. Ironic, isn't it, that the foundation created to serve the community afflicted by my source of lows is itself the source of my highs??!!

That said, I've also found many ways to adapt, to remain active and to remain of value, such as gardening, pool exercises, targeted PT, assistive devices, travel, run for local office, etc. So, while I'm a bit down, I'm damn sure not out!

BETSY B. *from South Carolina*

I am Betsy Blake from Lexington, South Carolina. I was initially diagnosed with Guillain Barre Syndrome in January of 2015. I am a pharmacist and work with other health professionals in a primary care practice. Their recognition of the recent onset of changes in my physical abilities sped up my diagnosis. I was able to get an immediate appointment with a neurologist. Within a few weeks, I underwent several diagnostic tests and started a 5-day treatment course with IVIG. I had an initial great response, but three weeks later, I regressed. Over the next two months, I experienced some mild improvements in symptoms but had more serious regressions to the point that I made frequent trips to my neurologist with some brief hospitalizations. By mid-March, I was completely paralyzed. My diagnosis was changed to CIDP at that time. I spent most of this two-month period at home. My husband was brushing my hair and teeth, feeding me, and getting me dressed. He spent a great deal of time scheduling appointments and advocating for my care to ensure I would not continue to regress. Three months after my initial diagnosis, I was finally able to get an appointment with a neuromuscular expert in Augusta, Georgia. What started as an outpatient consultation appointment turned into an admission to the NeurolCU. Within hours, I had a central IV was placed, and plasmapheresis was started. I went in limp as a rag doll. After five days of treatment, I was able to move my arms and kick my legs. On the 7th day, I walked a few feet for the first time in over 3 weeks. I walked out of the hospital (rather slowly) 8 days after being admitted. I had a few complications from the procedures, but I was incredibly grateful to be upright and moving again. It took months of recovery. I was started on steroids and an immunosuppressant. I continued to receive IVIG every three weeks and responded well, but I had a slight relapse a year later. This induced quite a bit of fear as I was terrified of regressing back to my previous paralysis. With some changes in my treatment and continued IVIG infusions, I was able to regain full mobility. After seven years, I am no longer on immunosuppressants, but I still receive IVIG infusions at a local infusion center every 4 to 5 weeks. I tell people that I am getting my superpowers renewed! I spend 6 or more hours at the infusion center for each appointment. They have

many private rooms, and I am able to work for the majority of the time that I am there. I continue to express my gratitude to all of the health professionals there who have helped me find the best care for my CIDP. I am quite appreciative of what I have overcome and that I can continue at almost full speed with my personal and professional life.

DAVE R. *from New York*

Hi, first of all, good afternoon, thank you for joining us on our journeys. I'm Dave, and I live in New York with my wife and my three daughters, so I have a good support system. CIDP is a very frustrating disease because it presents itself in so many different ways. That's why you'll hear different journeys from each one of us.

My journey began about ten years ago when I began to feel tingling in my feet, it then moved into my legs. Soon after my legs became weak. It was very scary because the doctors didn't know what it was. Over the course of maybe six or seven months, it became even scarier when the tingling started in my fingers and my arms followed by weakness. I started to realize, my god, I'm not only losing my legs, but I'm losing my arms and that was devastating.

My lowest point was one particular day when I was in a parking lot and I just went down, my legs went out from under me and I hit the ground. When I tried to get up, there was nothing there, no strength in my arms or legs. I don't know how long I laid there but eventually enough strength came back and I crawled to the car. I cried. This is my life.

Soon after that, the doctor put a name to my disease, CIDP. She was a local neurologist and diagnosed me by default, because I had nothing else. She had only seen a few CIDP cases before but started treating me with IVIG and I did respond. However the treatments were only going to be as I needed them. My wife soon after found a specialist in CIDP, and I've been with him now for ten years. He immediately put me on a regimen of infusions every three weeks. He said, "Let's get you stabilized, and then we'll start to see if we can lower the dosage". We've been through the highs and the lows together. Today, I'm doing pretty well. I'm on subQ IG and have use of my arms and my legs. I work part-time, I'm on the Board of directors of the CIDP Foundation, I spend time with my family and do woodworking on the side. However I still have the effects of the disease: fatigue, weakness and balance issues. Somebody asked me a while ago, what do you really miss? I really had to think about it. What I miss the most is energy. That's what I miss. It affects every day of my life. If I unload a car of groceries, I have to sit down. Whatever I do that expends energy causes me to have to sit down and recharge.

So, clinical trials, that's what's going to get my energy back someday. I truly believe that someday somebody is going to come up with something that is going to help this community, hopefully cure the disease. So, whatever can be done to help in that effort would be an amazing blessing to this group of people.

DAVE T. *from California*

Hello, my name is Dave Tuck. I live on the Central Coast of California and agree with what many of the panelists today have expressed. CIDP is a journey that changes constantly and presents itself in different ways so that each day is a new norm. You don't know what's going to hurt or what's not going to function on any given day, however, you take it as it comes. I was diagnosed with CIDP in 2008. In 2006 I began noticing some numbness between my toes and some vibratory loss in my ankles. I spoke to my internist, and he said I should follow up with a neurologist. I began seeing a local neurologist in 2008 who suspected CIDP after performing a few tests. He referred me to King Engel, MD at USC Department of Neurology, and the Jerry Lewis Muscular Dystrophy Center at Good Samaritan Hospital. After going through multiple batteries of testing (muscle biopsy, nerve stimulation test, imaging guided spinal tap, and a huge quantity of lab testing) I was officially diagnosed with CIDP that same year. Everything functionally in my body was normal at the time, however, I noticed a slow decay in my walk and my gait as the years progressed. I also noticed some fatigue, although at this juncture I could still handle it without any direct treatment besides vitamins that are important for nerve health and possible nerve regeneration. In 2017, I began calling into work once or twice a month because my body would not functionally move. I would literally and mentally tell my legs to move or body to stand up and it would not respond very well. It became very apparent it was time to consider and start intravenous Ig treatments. My neurologist wanted to begin the IVIg at a conservative dose and chose 400 mg/kg which is the dose for primary immunodeficiency to see how I responded. He had me on a every 4-week schedule and after 3 months the "spring to my step" and reduction of fatigue was consistently falling off at 18 to 21 days of a 28-day cycle. We chose to reduce the interval to every 21 days (3 weeks) continuing with the same dose and achieved success for the next two plus years. Again, the effect of the Ig infusion began to fall off at 14 to 15 days, so we chose to increase the dose in 2020 to 750 mg/kg maintaining the same 3-week interval. This is my current dose and regimen and still maintains positive effect with some fall off the last 3 to 4 days of the cycle. We still can increase the dose to 1000 mg/kg and/or reduce the interval to every 2 weeks when deemed necessary. Unfortunately, the treatment options available for CIDP at best may slow down the process and help you stay somewhat functional (reduced fatigue and spring to your step) but mostly they just help you maintain in life. My biggest frustration is that the decay process caused by CIDP currently cannot be stopped and nerve loss and muscle deterioration continue to progress slowly over time.

I also began having issues with foot drop and stumbling on cracks and changing terrain with some falls in 2018. I began using trekking poles to stabilize me when out walking and eventually had to add AFO's (ankle foot orthotics) to adjust for my foot drop and stumbling. Additionally, in 2018 my ability to function in my role at work became a safety issue as I was more frequently stumbling and catching myself before falling. The reality that I was a huge fall risk had to be confronted

which eventually led to me being medically retired July 2018 and required me to go on permanent disability. The loss of interaction with people within my profession/work and greatly reduced intellectual stimulation has been a challenge at the least and requires a great amount of energy on my part to marginally maintain. I also began having greater issues with responsiveness to the vehicle accelerator and brake while driving including the inability to safely know where my feet were in relation to the pedals. I have lost almost all of my dorsal flexion (could not lift toes or feet up) which led me to a referral to an occupational therapist that was a certified driver rehab specialist (CDRS). The recommendation was to add hand controls to my vehicle sooner than later before I completely lost the ability to use my feet and legs while driving or cause an accident or injury to myself or someone else. The hand controls were added in 2020 and were clearly necessary by then.

I think the hardest thing living with CIDP is the loss of freedom. What I mean by that is you lose spontaneity because you must plan everything, and you must plan for how to prevent stumbling depending on what the activity is. You need to be proactive in everything you do because you ARE a fall risk! A recent example is my 2-year-old grandson was here this past week and for me trying not to stumble in or outside our house on him or all the toys that were everywhere was very challenging. Likewise, wanting to be on the ground or floor to play with him which you can do with difficulty, however, trying to figure out how to get back up is equally as difficult. Travel is problematic anywhere domestic or internationally. It becomes a challenge because of not knowing what is and is not available to you for help with your disease and disability wherever you are traveling to. Moreover, the travel segment (air, train, boat, or car) itself can be quite traumatic and difficult secondary to what disability help is available or not available. Physical impairment is another difficulty living with CIDP. You eventually can't jump or run, and you are slower in everything you do. Additionally, you are dealing with balance issues constantly. You find that there's nerve disconnection meaning you'll walk, and then a sudden nerve disconnect (power loss) and you collapse for a moment. Hopefully, you're somewhere that you can grab and hold onto something or have an assistive aid/device you are using to help you not fall. However, if not, you do damage to your knees or worse. The reality is, you're disappointed and frustrated at times on days that you can't function, and you just get tired easily. It is also a very humbling disease because suddenly you find things you were able to do before developing symptoms from CIDP that you can't do anymore. You become dependent upon others and need to call on them to help you. That's good in the long run, but it does humble you because you are forced to rely on others instead of doing or performing the task yourself (humanly we tend to want to be self-reliant). One of the greatest disappointments is what you have lost in terms of physical impairment and function. You become separated from normal functions when friends or family visit or gather. There is always something you cannot do anymore so you sit on the "sidelines" and watch instead of directly participating. And there is always the occasional down day where nothing seems to work or help. Fortunately, this is temporary, and tomorrow is almost always better.

I think on a high point, you learn to get over yourself in some ways and ask for help. You meet people who have similar diseases and can compare and discuss each other's journey with CIDP. I would have never met these special individuals and families had I not had the disease and can listen to their stories as you are hearing other patient's stories today. You find it is encouraging and helpful to interact with others who have similar issues and hear how they navigate this journey with CIDP.

Finally, my dad gave me a lot of inspiration albeit in a negative way that I was able to turn into a positive direction for me. He had the same disease and was always angry, bitter, and not proactive in his journey with CIDP. I was determined, after observing him over the years, not to let the disease win. I choose to win by not being bitter, angry, depressed, or apathetic. Instead, I choose to be a positive influence and encourage those around me including those who have similar diseases as well as those who are disabled for whatever reason. When I was diagnosed with CIDP I chose to use whatever tool/aid that would keep me as mobile and independent as possible. I also believe much of my help and outlook comes from a very loving and supportive wife, as well as my personal faith in Jesus Christ that gives me much hope. The disease makes me more reliant on God and that is truly where my joy comes from.

Thank you for listening to our stories and for the time we have with you here today. This is a brief review of my journey thus far and I trust it was helpful.

NANCY D. *from Palermo*

My name is Nancy Di Salvo. As Lisa mentioned, I am from Palermo, Italy. I have been with the Foundation since 1997 as a patient first, then as an international liaison in Italy (2013), and now also as a board member (2019).

I am a CIDP patient, diagnosed in 2006 at age 36. Before CIDP, I was diagnosed with GBS patient at age 20.

Although dealing with CIDP is very difficult at times, it has also become part of who I am as a person because I have learned to live with my difficulties, with my treatments, and all that goes with the disease itself. I have become a better person.

When I was first diagnosed with CIDP in April 2006, I was initially treated with steroids, then I started my journey with IVIG. In the beginning the treatment of 5 days of IVIG was only necessary every three months, but then slowly I became very weak reaching the end of those three months, so my physician had shortened the in between timing of the treatment; at a certain point, after a couple of years, I needed my IVIG every four weeks with 3 days of infusion. It became very difficult to deal with those 3 to 5 days in day hospital every month, because I had started to work again and was trying to live my life on a normal basis. Having to go to the hospital every month had nothing to do with normal. I felt like I had to hit the "pause" button in my daily life, get boosted with IVIG and then return to hit the play button.

At a certain point, in January 2018, I was introduced with subcutaneous immunoglobulins. I must say, it changed my life because from that point on I was able to administer my treatment at home and on my own time while watching TV, working on my computer, or just simply dedicating that moment to myself. Subcutaneous therapy gave me the opportunity to work without having to ask for sick days or leave of absence. In the long run, thanks to subcutaneous therapy I have achieved more, and my physical condition is much better, thanks to the continuity of the treatment.

With CIDP you learn to live with your condition, you accept it and make the best out of it. You never forget where you came from... those moments of weakness and severe disability change your life forever. And if you're lucky enough it can change your vision of life, remind you to focus on the important things of life, it empowers you to a point in which you want to share your story not for your own good but to help others.

ANGELA P. *from Colorado*

Hi, everyone. My name is Angela. I'm originally from St. Petersburg, Florida, but I moved to Denver, Colorado a few years ago. I was diagnosed with CIDP at 21. At the time, I was working full-time, going to college full-time, and I had to stop all of that and to learn a completely new way of living, relearn how to walk, and make various adaptations to every part of my life. Some of those adaptations include limiting how long I exercise for, accommodations at work, and even changing the clothes and shoes I wear. I am now 26. I now can hike a couple miles with my dog and hang out with my friends and family. I've returned to the workforce full-time. I've been able to start a career. I've actually started real estate school as well, so it's been really nice to have my life back. These changes have been assisted by things like flexible scheduling, but most of the improvements are due to the various medical treatments I have been on. Everything from steroids to IV immunoglobulins every three weeks. Now I do subcutaneous immunoglobulin therapy every week. While I do subcutaneous therapy, I can work from home. It's just been amazing to have my life back. That being said, there are still some side effects that I'm dealing with. There is brain fog, weight gain, all different side effects that affect my life. It's not a cure-all. An ideal treatment would be a pill or injection that doesn't cause other health issues like steroids do. It's very time consuming to do subcutaneous treatment every week, so if there was a shorter option, that would also be helpful. I still have residual CIDP symptoms, such as limited feeling in my fingers and toes, loss of balance, and substantial fatigue. If I could lessen the additional problems due to medicine, that would be entirely life-changing.

LINDA P. *from Ottawa*

My name is Linda Paul. I am from Ottawa. I got CIDP when I was 49. I was a healthy, active person. I was working for the federal government as a manager and Senior policy advisor. I walk to work every day. It was about 25 minutes each way and took the stairs 12 flights. We had a challenge going on with all of my workmates on how many times we could climb the 12 flights of stairs in a week. It includes swing dancing two or three times a week, and I went rockclimbing on Saturdays. I was a healthy, active person. My first indication was when I woke up from a nap and my feet and ankles were numb. This happened in May about four weeks after a bad case of the flu, which was probably H1N1. The numbness continued to bother me for a few months, but it got better. I was having trouble putting my feet on because they got -- I'm sorry, having trouble putting my shoes on. Eventually, the problem got somewhat better. I went away to a dance camp with five hours of classes per day and dancing all night, and they started to get worse again. Eventually, got a little better. On August 30, I had my first relapse. Between lunch and supper, I noticed my feet getting numb again, and by suppertime my legs were numb to the top and into my fingertips. My gait was really uneven, and I was having trouble walking. I went to my family doctor the next day, and he told me to go to emergency. He also sent me to aphis I trust -- into a psychiatrist. He diagnosed me with CIDP tell you when I went to emergency, I brought a note saying that he thought I had GBS, but the neurologist in emergencies that I did not have GBS and sent me home. This neurologist was very familiar with GBS, because he worked with the foundation on numerous occasions, but he still did not recognize the fact that I had GBS or CIDP. I was told to keep going back to the ER until I got an appointment with a neurologist, which I did in mid-September. I could barely walk, and I was falling frequently. That eventually resulted in a sprained wrist. My bowels were not working properly. The neurologist diagnosed mild GBS and told me to come back in three months to see how I was doing. I had two more acute relapses, one while traveling for business in Toronto and, again, I woke up and could barely walk. When I got home, I went to the ER and saw a different neurologist who told me that it was not safe for me to live on my own and sent me back to my neurologist at the other hospital. At that time, he decided that he would run some more tests, and I got a lumbar puncture. Before that, I had family members that came to stay with me. Then I had another relapse on November 6 and I woke up later paralyzed to my face. This time they admitted me to the hospital and they worked on diagnosing me. This is when they told me I had CIDP. They gave me the full form. Chronic inflammatory demyelinating — hard to remember that. I was happy to get a diagnosis, even though they diagnosed me with a few other things first, including lymphoma, because all the lymph nodes in my body were swollen. They also diagnosed me with an infectious disease as a result of IVIG tainting the blood results. Once I received my loading dose of IVIG, after the first day my feet could move again. by the second day, I could get up and take a few stumbling steps. I had immediate positive response to IVIG, and it's what's been keeping me going. I really, really am happy about that. by the time I checked out of the hospital five weeks later, I could walk with a walker. I

started off on a very low dose of IVIG maintenance for the first couple years. Only 35 grams every month. At one point, the doctor tried to wean me off by moving it to every five weeks and every six weeks. In the year that he did that, not only did I regress and develop new symptoms, but I lost all the progress that I would have made in that year. That was really hard. Eventually, through getting second and third and fourth opinions I got back on IVIG every four weeks and got the dosage increased. I now get 75 grams every four weeks. I have been improving at a slow and steady rate since then. Once I got the higher dose, I went into a 5K walk with my walker. My goal was to complete it and not come in last. I made both of those goals. When it was increased the next year, I have to my time in doing it. That made me really happy. Without IVIG, I think I would be bedbound.

IVIG has saved my life. I cannot run or jump, but I can dance again, just not very well. I still see improvements even after 12 years. My big improvement this year was I was able to lift my toes off the floor and stand on my heels. It doesn't sound like much, but to me that was another big improvement. After 12 years, I'm still improving. That gives me hope.

CORBIN W. *from North Carolina*

Good afternoon, good morning, good evening. I know we are all over the world here. My name is Corbin Whittington. I live in Chapel Hill, North Carolina. I am a husband of 39 years. I am a father of two grown men with their own families, and I am also a Papa of three very active grandkids that were just here last week. Happy to see them. Happy to see them go. But we had a great time. My CIDP story really is more like a 12-year journey. Like many people here have described, it evolved over time. For me it began back in 2010. That was kind of in hindsight and retrospect, because back in 2010 I was a globetrotting senior technology organizer, and I managed engineers and scientists and technologists, and I traveled the world frequently. I was on an airplane a couple times a week. I navigated airports from New York to Tokyo and managed tens of millions of dollars from month to month. I began to notice in 2010 that I was beginning to have this odd sensation in my feet, particularly at night. They were getting numb and tingling. I also began to notice I was having more and more difficulty moving around through airports and going up and down stairs. More and more I found myself choosing the escalators instead of staircases, choosing the elevators instead of the stairs. I saw my doctor on a regular basis. We talked about it. "You are not a spring chicken anymore." At the time I was 50 years old. "You are not a spring chicken anymore. You need to get better exercise and lose some weight and get some more rest." Okay. Symptoms continued to worsen. I found that I was struggling more and more just doing basic things, walking around the grocery store, going up and down stairs. Lifting a gallon container of milk out of the refrigerator. The pain began to increase and the numbness began to increase. Again, went back to the doctor and said that there is something wrong. He said, you need more exercise, et cetera,

et cetera. He did a pretty thorough analysis. He found out I had a heart condition and. Now we are in 2012. Serious heart condition, life-threatening heart condition. Dilated cardiomyopathy. All the focus became the reason for everything. Well, you are weak because of this heart condition. You lost strength because of this heart condition. You are tired and fatigued because of the heart condition. You have brain fog because of this heart condition. Maybe this numbness and tingling is circulation problems. The heart condition began the problem for everything. I couldn't even navigate my way to the mailbox in the morning, let alone around an airport. I had to leave work and go on disability.

I was being treated pretty aggressively for the heart condition, but those symptoms persisted and continued to get worse and worse until one day around 2014, I really insisted that something else is going on here. Now I am stumbling and falling, tripping, dropping things. I am having difficulty just getting myself up in the morning. I couldn't walk in a grocery store. They determine a neurological issue going on. Unfortunately, I lived in an area where we had a really good neurological medical organization. It took a lot of months. A lot of testing, a lot of analysis. Ultimately, with a surgical biopsy being able to determine that I had CIDP. At that point, I'm really struggling. I could still walk, but barely. Stairs were a major challenge for me. We started on the IVIG like many folks. Many of the medications, et cetera, they weren't available to me because of the implications of my heart. So, the IVIG had to work for me. Fortunately, it did. by 2015, I'm on the road to recovery. Those symptoms began to diminish. I began to feel better. We gained functionality. Ultimately, I was able to get pretty active in my community. I serve on two boards of directors, two global organizations. I also serve as a pro bono consultant working with nonprofit Board of Directors in the North Carolina Triangle region to help them perform their missions better. More recently, I decided to step out of disability and to step back into for-profit work. All of that is still well navigating this debilitating illness. I still have to get subcutaneous now because the IVIG was not working well with my heart condition anymore because of the stress that it put on my circulatory system. Fortunately, for me, around that time, subcutaneous came about and I was able to make the switch. I still depend on it weekly. Every Sunday, I spend almost an entire day of pre-medicating and infusing and post medicating and the next day pretty much dealing with the aftereffects. But it gave me some freedom. It gave me some ability to do some more things. I still deal with the fatigue. That comes and goes, as we all know. I still deal with a lot of pain. I decided to manage my pain in multiple ways. Oftentimes we get a little loopy, a little brain fog. This work that I'm doing, I can't have brain fog. I still struggle with steps from time to time. And I still deal with these relapses. Just recently I had a relapse. It scares the heck out of me, because I thought I was making all the progress, making all these plans, and then I relapse. Now what? Fortunate for me it kind of reverts itself and I literally get back on my feet again. I have gained some function. I've regained some capability. I feel really good about some of the activities I am involved in. I have renewed sense of purpose, but I still have that little bit of fear, that little bit of anxiety that one of these relapses is going

to set me back significantly or that the IG is not going to work anymore, and then what? I have no more options pretty well I am very optimistic about the future, excited about my situation today, I do have a little bit of concern about going into tomorrow as to what kind of options and treatments are available to continue to, hopefully, cure this condition.

11

APPENDIX 4 Scientific Overview by Dr. Peter Donofrio and Dr. Jeffrey Allen

DR. PETER DONOFRIO

My name is Peter Donofrio and I am privileged to be the chair of the Medical Advisory Board of the Guillain-Barre CIDP Foundation. I've been asked to give a brief overview of CIDP and I thank you for the opportunity to do this. So, first of all, how common is CIDP? Well, CIDP varies between one and two per 100,000 or that would be 10 to 20 per million people. The prevalence is much higher because it's a chronic illness and many people have CIDP for years and decades. You'll see that the incidence of CIDP is about the same as Guillain-Barre Syndrome. Now, what's interesting is CIDP is common enough that when people go to a major academic medical center, it will represent about 20% of all the people who have a peripheral neuropathy and it will be approximately 10% of all patients referred to neuromuscular clinics, either at academic medical centers or large medical centers. Notice that the age of onset is 40 to 60, so this is not an illness of older people and even children can develop CIDP. And CIDP is slightly more common in men than women.

So, the characteristics of CIDP and I glean this information from about 16 to 17 articles on the topic, so probably the most important thing is that the time course of CIDP is usually two months or more. So, most of the time, people present to their doctor with symptoms for months to years, but at least two months. Patients are weak both proximally in the arm and legs and distally in the hands and feet. They also have abnormalities of sensation. We call this large and small fiber. Large fiber would be vibration and joint position sense and small fiber would be pain and temperature. When the doctor evaluates them, usually the reflexes are absent or they're reduced. And then there are characteristic nerve conduction study abnormalities that are detected in people with CIDP. There is slowing of nerve conduction velocities. There may be conduction block. There is a phenomenon called temporal dispersion. And if patients undergo a spinal tap, the protein is often elevated and there will be few inflammatory cells. If a nerve biopsy is done, and nerve biopsies are not necessary to make the diagnosis, we would expect there to be evidence for loss of myelin (demyelination), or inflammation with white

blood cells in the nerve. So, the diagnostic criteria for CIDP, and this was created by the European Federation of Neurological Society and the Peripheral Nerve Society in 2010. IT has just recently undergone a revision that I will not discuss the changes, but it requires that patients have disease for at least two months or longer. We already talked about proximal and distal weakness. The evolution can be slowly progression – progressive over time, a stepwise loss of function or relapsing and remittin. There often is sensory loss, usually large fiber..Cranial nerves five and seven may be involved. There are absent or reduced deep tendon reflexes in forelimbs.

So, let's talk about the pathogenesis and pathophysiology. First of all, we recognize that CIDP is an autoimmune disease but we don't know the cause. It's mediated by B and T cells in the immune system. And I'm not going to go into that because a lecture on B and T cells could go on for a long period of time. If we do a nerve biopsy, there's evidence for inflammation which we've talked about, demyelination, remyelination, and a phenomenon called onion bulb formation. But we must keep in mind that these are nonspecific changes. They are seen in AIDP or acute inflammatory demyelinating polyneuropathy, a form of Guillain-Barre. But they're also seen in other autoimmune neuropathies and in inherited neuropathies. In most patients with CIDP, antibodies in the blood or spinal fluid are not found. But in approximately 25% of CIDP patients, they can have antibodies to peripheral nerve myelin proteins. And it's really not clear whether these antibodies are causing the disease or may reflect a response of the body to demyelination and loss of axon. There have been no biomarkers that have helped to discriminate CIDP from Guillain-Barre, AIDP, and other types of neuropathy. This is one of my favorite slides and it's regarding the pathogenesis of Guillain-Barre but we can use it in people with CIDP. In slide A, we have a normal peripheral nerve. The axon is white, the myelin is gray. This is normal. In the beginning of the inflammatory response, we see that there are lymphocytes that begin stripping away myelin from the axon. This is the beginning of the disease process. If we advance from B to C, we now see destruction of the axon and greater infiltration by inflammatory cells. Then in D, we see complete destruction of the axon, in which case the disease is essentially irreversible for those given nerves. So, we can use this excellent model developed in 1969 for CIDP. Here's an example of demyelination. And so, this is a demyelinating lesion. Here's a model of temporal dispersion in again, Guillain-Barre but it also applies to CIDP. This is a normal nerve, large fibers, small fibers. You see segmental myelination. This is normal. In a condition like CIDP and AIDP, we see what's called segmental loss of myelin. And so, conduction is either blocked or slowed from the left to the right. This is shown in nerve conduction studies. This diagram shows an ulnar nerve stimulated at the wrist, elbow and clavicle. Notice that the waveform at the wrist loses its amplitude and is stretched out at the elbow and clavicle. This is called temporal dispersion and reflects usually demyelination. So there have been, as I mentioned before, some antibodies called autoantibodies recognized in CIDP. Again, most people do not have these antibodies. Then finally, recently, antibodies have been recognized against the nodes of Ranvier and areas near that. So, if the node of Ranvier is on the right side of this model,

here is the node, here is the paranode and here is the juxtaparanode. And this is an excellent model showing antibodies that can be directed toward antigens, the CASPR2, CASPR1, neurofascin155, and CNTN1. And when antibodies are directed towards these nodes there can be blocking of the impulse from the left to the right, producing weakness and sensory symptoms and diseases like CIDP.

DR. JEFFREY ALLEN

I'm Dr. Jeffrey Allen from University of Minnesota. And it's really my pleasure and honor to be able to give this discussion on CIDP standard of care to this FDA patient focus group. Here are a few of my disclosures. And I thought I'd start with sort of just a very high level overview of where we got to where we are right now in 2022 with CIDP treatment. Since this diagnosis was first named in the 1970s by Dr. Dyck at the Mayo Clinic, there have been several clinical trials that have explored CIDP treatments. Some of those trials are shown in red, which are found not to be beneficial, and the ones in green was shown to be effective. And on the basis of these trials, we now know that IVIg, subcutaneous Ig, corticosteroids and plasma exchange are effective evidence-based treatment options for CIDP. If you kind of take a look at this high level and say, "How often do they these treatments work?" About 50 to 70% of patients respond to one of these first five treatments. If you try a second treatment after the first one doesn't respond, overall, about 80 to 90% of patients will respond to one or more of these individual treatments.

So, let's look at the data in a little bit more detail. Corticosteroids were studied first and it's in this publication from 1982 that Dr. Dyck and colleagues compared prednisone to no treatment in patients with CIDP. So, although it was a small study of only 40 patients and there was no placebo arm, it nonetheless showed that patients that were treated with prednisone improved and untreated patients worsened. Despite the design flaws of this study, it's nonetheless a really pivotal CIDP trial and established evidence that corticosteroids are one effective CIDP treatment option.[Inaudible] the IVIg has been studied, several randomized clinical trials, the largest of those trials, which was the ICE trial published in 2008. The ICE trial compared IVIg to placebo in 117 patients and found that 54% of patients improved if they were treated with IVIg compared to only 21% on placebo. And on the basis of this trial in others, IVIg has high quality data to show that it's efficacious for the treatment of CIDP.

So, what do we do when we compare IVIg to corticosteroids, which one is better? Without going into the details of this trial, there's been some suggestion that patients treated with IVIg more often benefit from treatment and it's probably better tolerated. Corticosteroids have one advantage in that they may be able to induce a period of long-term remission if the patient is treated in a way that IVIG is [inaudible]. Subcutaneous Ig has also been studied in a randomized clinical trial in CIDP. This study called the PATH study compared subcutaneous to Ig to placebo in 172 patients. This study was published in 2018. The investigators

found that patients treated with subcutaneous Ig relapsed at a rate of 33 or 39% compared to the dose and that's compared to 63% of placebo patients. So, on the basis of this study, subcutaneous Ig was confirmed to be an effective maintenance therapy option for CIDP. On the basis of these trials, we now know that CIDP first line treatment can be summarized as such. IVIg or corticosteroids are usually used first. And although there's no consensus on which one is best, it's wise that if one treatment is started and doesn't work, then the other is tried unless there's a medical contraindication to do that. Plasma exchange, I didn't show that data, but plasma exchange has also been proven effective, but is generally reserved for unique situations in which IVIg and corticosteroids fail.

And finally, subcutaneous Ig is an option for maintenance therapy if immunoglobulin dependence is established. It's always important to keep in mind what to do to non-responders and if one first line therapy is not beneficial then it's important to take a pause and reconsider if the diagnosis is correct. In the event that the diagnosis is correct, there are other off-label interventions that can be tried. These medications are generally discouraged based on negative clinical trials. However, there are others that have been observed to be beneficial in small anecdotal or small case series. The decision to use any of them requires careful consideration of each individual patient's disease characteristics, treatment history, comorbidities, and risk analysis.

Now, there are some published guidelines to help make sense of these decisions. And I don't have time to go into the guidelines today. But these guidelines published in 2021, late last year, are both diagnostic and treatment guidelines and can be very helpful to navigate through these treatment decisions. I strongly encourage clinicians to reference these guidelines especially during therapy escalation. So, in closing, I just like to raise a few questions that clinicians and patients should address and consider when deciding on what's the best treatment for them. Many of these questions don't have any easy answers, but thinking about them is important to really optimize treatment to every unique person. The first question is, is the disease [inaudible] neurologically [inaudible]? How long is treatment needed? Just because it's a chronic disease, does it mean the treatments are needed forever? Thinking about that is important to make long term treatment decisions. Next, have the first line medications been used in a responsible way? Is there a treatment failure because it doesn't work or because it wasn't dosed properly? Third, is there another explanation for the symptoms? Is there a different diagnosis or a second problem? Fourth, what is unique about my disease that might help guide the next drug choice, other medical conditions or other medical problems? And fifth, what are the risks that the drug has and [inaudible] the risks of the drug relative to my disability and to my current treatment? Is the next therapy worth it? Is the risk worth it? All of these are difficult questions to answer, but each of them is important when deciding on what the best course of therapy is for any individual patient. Thank you very much for your attention.



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Foundation International

375 East Elm Street, Suite 101
Conshohocken, PA 19428
www.gbs-cidp.org
610-667-0131