Summer 2017

GBS|CIDP Foundation International

The Communicator

Providing Strength Through Support

Working for a future when no one with Guillain-Barré syndrome (GBS,) chronic inflammatory demyelinating polyneuropathy (CIDP,) and related syndromes such as MMN suffers alone and that everyone has access to the right diagnosis and the right treatment, right away.

In This Issue

Patient Story

94 Days

by Tom Bartlett, Maryland

I had gone 36 years since I last spent a night in the hospital. That all changed in 2015.

I had been infused about 100 times since 2013 at the Mayo Clinic. But I just wasn't right. While shooting a basketball on Christmas Day 2014, I lost my balance and fell to the ground. From that day on—2015 is a year to forget.

Selling my business, my mom passing away, our only child Megan getting married, my dad dying—all I thought was stress, making me worse. The IVIg was just not working anymore.

By summer I was getting weaker. Now I couldn't walk. I received five days of Plasmapheresis the week before the wedding. On Friday, some dear family friends picked me up in Jacksonville and drove me to Asheville for our daughter's wedding the next day. There I met my wife, daughter, and family for the Friday evening rehearsal dinner. I had no appetite. It all starts to get foggy from here.

On Saturday morning, my brother-in-law drove me to get a haircut and shave at an old-time barber shop in Asheville.

At the Catholic church in Asheville, I attached my leg braces, got out of my wheelchair, and grabbed the walker. As the doors opened...my daughter looked like a shining light. She grabbed my arm.

"No tears!" She nodded.

I said, "If I go down, let go or you are going down with me."

"I'm good dad!!"

Off we went. It was a beautiful wedding.

One week later, I checked in to Johns Hopkins Hospital. IVIg didn't work. Plasma didn't work. I just kept getting worse. My doctor, Dr. David Cornblath, M.D., gave me Rituximab.

Pneumonia. Pneumonia again. Chest tube right side. Chest tube left side. One feed tube. Two feed tubes. Ventilator. Bed sore.

Horrible ICU delirium. Day after day...

By mid November, I had turned the corner. On the 94th day in Johns Hopkins Hospital, I was released to rehab weighing 108 pounds. At 6'3", I was skin and bones.

Over six months I received six treatments of Rituximab. By the fall of 2016, I no longer used a wheelchair, walker, braces, or a cane. I can't run, but I can bike. I walk two to five miles a day.

I can't thank God enough.

Or my families and friends for all I put them through. Or the folks that looked after me.

I pray for anyone going through this terrifying experience.

We take this opportunity to thank ***CSL Behring*** *for their support in making this newsletter possible through an unrestricted educational grant.*

in the mailbox

My name is Ashley Currey and I am in eighth grade attending Piney Grove Middle School. I have been assigned a project called the IMPACT Project, which stands for Influencing My Peers And Changing Tomorrow. For this project I will give an oral presentation about Guillain Barré Syndrome, how it has affected many lives, and how my peers can get involved and help the cause. For the IMPACT Project I was allowed to pick any topic, so I chose one that personally affected my family.

About thirty-seven years ago, my grandmother who is currently living with my family, was paralyzed for six months with Guillain Barré. My mom, who was in seventh grade at the time, and her two older brothers had to take care of themselves and my grandmother while my grandfather went to work. Today she has constant foot pain which she says Guillain Barré is the cause of. Not being able to move for half of a year had an impact on how my mom and my two uncles were raised. When someone is affected by GBS, it can put their life on hold as well as the lives of their family. So that is why I find GBS an important topic to raise awareness about.

Influencing and teaching my peers about GBS could do more than just education them about the topic. Thank you for the information you sent to me to share. By working together I hope to help accomplish your mission of improving the quality of life for those affected by GBS.

Sincerely,
Ashley Currey
Cumming, GA

Meet a new member of our Global Medical Advisory Board: Diana Castro, MD

Dr. Castro is an Assistant Professor of Pediatric Neurology and Neurotherapeutics at Children's Health University of Texas, Southwestern Medical Center, Dallas, Texas.

We are so pleased to have Dr. Castro as a resource for our young patients. Congratulations, Dr. Castro!

From the Executive Director

Dear Friends,

I am so proud of our organization—what we do and where we are, especially at the new "One-Day Conferences" in March in Fort Lauderdale, FL and May in Baltimore, MD.

What made me so proud?

- The presenters who give of their time to help our patients and guide them through treatment and recovery.
- The respect and appreciation the attendees had for our efforts and for the help we strive to bring to them.
- The work our staff did and attention paid to each detail to make the meetings run so smoothly.
- The corporate supporters whose confidence in what we do inspires them to help make events such as this happen.
- The fact that we will be holding another "one day conference" this year in Chicago!

So join me in feeling proud of the organization whose mission is to care for its patients—we have never lost sight of why we are here.

Enjoy the summer!

Sincerely,
Lisa Butler
Executive Director

Save the Date!
Saturday, September 23
One-Day Regional Conference in Chicago, IL
Check website for details.

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Please update your contact information to make sure we have your current email address. Your information will not be shared with anyone outside the *Foundation*.

Contact us online at gbs-cidp.org
or by emailing info@gbs-cidp.org

Visit us on Facebook: facebook.com/gbscidp

Research Update: Antibodies in GBS|CIDP

by Eric Lancaster, M.D., Ph.D.
Assistant Professor of Neurology, University of Pennsylvania, Philadelphia, PA

GBS and CIDP are autoimmune diseases that target peripheral nerves, but patients may be affected to different degrees and in different ways. Some patients have relatively mild attacks, while others may be paralyzed. Symptoms of weakness or numbness may occur in any proportion, from a purely "motor" condition causing only weakness to a purely "sensory" problem causing numbness and difficulty controlling movements due to lack of position sense. Patients with GBS usually have only a single attack due to an immune response that stops within a few weeks, but CIDP patients can have an ongoing autoimmune neuropathy for many years. Some patients respond readily to the usual treatments, but others do not. This diversity of outcomes may contain clues to finding better ways to diagnose and treat GBS and CIDP, if we could understand what causes it.

One window into this question is to study the antibodies of patients with GBS and CIDP. Antibodies are manufactured by the immune system to fight off infections. A normal immune system can generate billions of potential antibodies in a random process and then select the ones that useful. Many types of antibodies are generated in this way every day. In this process, there will be an occasional mistake - an antibody that can affect a normal part of the body rather than something foreign. Fortunately, a quality control system eliminates most of these, preventing them from being made. When this quality control system fails, there can be an autoimmune disease. If the antibody in question targets the nerves, the result might be GBS or CIDP.

There are good reasons to believe that GBS and CIDP are often caused by autoantibodies. For instance, treatments designed to wash out antibodies from the blood (plasmapheresis) or to neutralize antibody effects (IVIg) work for many patients. In the case of GBS, antibodies targeting structures on nerves called "gangliosides" are found in subsets of patients. In CIDP, the targets of the antibodies have been more difficult to find.

In the last few years, specific antibody targets have been identified in small subgroups of patients CIDP. These proteins are mostly structural elements on the nerves and their myelin (insulating cells) that hold the nerve and myelin together. Some of these proteins include Neurofascin, Contactin, and Caspr. Interestingly, antibodies to these proteins associate with CIDP that is resistant to the most common treatments, particularly IVIg. But patients with these antibodies may respond well to other types of immune therapy, such as rituximab. These rare cases are the first examples of antibody tests predicting how CIDP patients will respond to specific treatments.

These experiments raise other important questions that have not been answered. When do these antibodies emerge in the disease course? Is it possible to identify certain patients with GBS who are more likely to progress to have CIDP based on antibody tests? Do the antibody responses go away after successful treatment? Are there other patients who have these antibodies and then spontaneously stop making them - resulting a limited disease? Could we someday develop treatments to get the immune system to stop making that one particular type of antibody without causing the side effects of current treatments? (This concept of the immune system learning to stop responding to something is called "tolerance" and may be important for explaining why most patients with GBS have a self-limited immune response).

So far only a small group of CIDP patients can be classified with one of these antibodies. The antibody tests we have now can be used to understand why certain small subgroups of CIDP patients do not respond well to usual therapy and to suggest alternatives for them. Additional work is required to understand the full nature of the immune response for most patients. It is possible that in the future, we will be able to use antibody tests to diagnosis GBS and CIDP more quickly and reliably. A more precise understanding of the antibodies involved may also allow individualized "precision medicine" approaches to treating each patient.

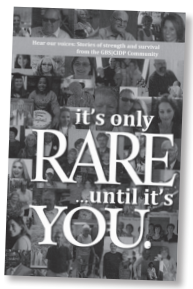
Looking for some interesting reading?

You will find a list of the most popular books on Guillain-Barré syndrome, CIDP, and variants on our website "library," including an exclusive book, *It's Only Rare, until it's You.*

It's only RARE, until it's YOU, is a fascinating collection of real patient survivor stories guaranteed to lift you up in your journey as you discover how others survived their trauma and lived to tell about it.

It's Only Rare until it's You is only available for purchase through the *Foundation*.

Call 610-667-0131 to order your copy today!



Announcing the GBS|CIDP 2017 Grant Awardees!

1. Enhance Peripheral Nerve Repair by Modulating Macrophage Subsets:

Gang Zhang, PhD, Assistant Professor of Neurology, University of Texas, Health

Synopsis: Autoimmune neuropathies consist of a group of peripheral nerve disorders associated with dysregulation of adaptive and innate immune responses. Among these disorders, Guillain Barré Syndrome (GBS) is one of the most common causes of acute flaccid paralysis worldwide. Two immune modulating therapies, namely, Intravenous immunoglobulin (IVIg) and plasma exchange, have been developed to treat GBS. However, there are many disadvantages including high cost, supply shortages, and multiple side effects that are usually associated with high dose and long infusion time of IVIg. Therefore, new therapeutic strategies that can limit the nerve injury during the acute phase of the disease and enhance repair during recovery period are highly desirable. It is in this context we propose a novel therapeutic strategy of modulating macrophage polarization (promote M2 polarization) for treating GBS. Macrophages are central regulators of inflammation.

2. Probing The Role of Skin Biopsy in CIDP Nodo-paranodopathies:

Raffaella Lombardi, MD, IRCCS Carlo Besta Neurological Institute, Pavia, Italy

Synopsis: CIDP is a heterogeneous pathology that critically lacks of diagnostic and prognostic biomarkers. The recent identification of IgG4 anti-neurofascin 155 (Nfasc155) or anti-contactin 1 (CNTN1) antibodies in a subset of patients has widened the spectrum of CIDP phenotypes. These patients how disabling tremor, poor response to intravenous immunoglobulin, and distal and sensory disturbances. Cell-adhesion molecule Nfasc155 and CNTNl are expressed on the paranode, and play key roles on sodium channel clustering and glia-axon interactions. Our preliminary results on skin biopsies from three IgG4 Nfasc155-positive CIDP patients revealed complete loss of Nfasc155 staining at the paranodes, asymmetrical paranodes and widening of the nodes of dermal myelinated nerve fibers. One IgG4 CNTN1-positive CIDP patient showed abnormal nodal/paranodal immunostaining with different features as compared with IgG4 Nfasc155-positive patients suggesting specific changes.

Ernest Hayden Award

3. Flavivirus and Arbovirus Associated GBS in South East Asia:

Umapathi Thirugnanam, MD, National Neuroscience, Singapore

Synopsis: A number of antecedent infections are associated with Guillain-Barré Syndrome (GBS). Recently, symptomatic and asymptomatic Zika virus (ZIKV) infection has been shown to trigger GBS. In the Southern hemisphere the burden of flavi and other mosquito-borne arbovirus infections such as dengue, chikungunya and Japanese encephalitis is considerable. Even in a small, developed country like Singapore, dengue infections can exceed 20,000 a year. Symptomatic dengue has been documented to antecede GBS. Recently we reported that asymptomatic dengue could also trigger GBS. In parts of South East Asia, GBS cases increase after rainy season when mosquitos breed and dengue infections are common. The anecdotal experience of doctors working in these region suggests that diarrhea associated GBS is infrequent. The actual burden of dengue and other arbovirus, including ZikV, associated GBS is uncertain. The ZikV strain responsible for the current epidemic is Asian, described in 1960-70s in South East Asia. It is conceivable that ZikV isendemic in this region.

We have moved!

NEW ADDRESS:

375 E. Elm Street, Suite 101
Conshohocken, PA 19428

Phone (the same) 610.667.0131

After 25+ years in Narberth, PA, we have moved! As we expanded our services, activities, events, and support system, we outgrew Narberth.

We cordially invite you to stop by and visit us in our new home!

Patient Notification System (PNS): Be the first to know!

Confidential notification of plasma protein therapy withdrawals and recalls

The name says it all! The Patient Notification System notifies the patient directly of a voluntary and/or mandated recall of plasma protein therapies. GBS|CIDP Foundation International endorses the Patient Notification System. Immune Globulin therapies used by individuals with GBS|CIDP are one of many therapies on the PNS. This system makes all the difference—while other notification systems are designed to inform physicians and pharmacists, the PNS empowers the patient to receive this important information directly via email , telephone, or fax. There is no charge to register

Register today at: www.patientnotificationsystem.org.

About the PNS

Nearly two decades ago, the Plasma Protein Therapeutics Association (PPTA) and its members worked in collaboration with consumer organizations to develop the first-of-its-kind Patient Notification System (PNS).

- Easily accessible, comprehensive, and up-to-date information about all brands of immune globulins, blood clotting factors , alpha-1 proteinase inhibitors, and other lifesaving plasma protein therapies
- Confidential: all registrants , patients, nurses, physicians, pharmacists, or family member are guaranteed that their information is never shared nor is it accessible by anyone other than the third-party company that houses the computers to run the system and send the notifications.

Ensuring Confidentiality

Maintaining patient confidentiality was a major consideration when developing the system. In 1998, a working group comprised of stakeholders including the U.S. Food and Drug Administration , Alpha-1 Association, Alpha-1 Foundation, Committee of Ten Thousand, Hemophilia Federation of America, Immune Deficiency Foundation and National Hemophilia Foundation helped to design the system to safeguard sensitive registrant information. Subsequent to the founding of the PNS in 1998 the following stakeholders have added their support Canadian Blood Services, GBS|CIDP Foundation International, Hereditary Angioedema Association , Inc., Jeffrey Modell Foundation, and Platelet Disorder Support Association. To ensure privacy, the PNS is operated by Stericycle, Inc. , an independent organization that specializes in informing the public of pharmaceutical withdrawals and recalls (notifications). All registrant information is kept strictly confidential.

How to Register

1. Visit www.patientnotificationsystem.org or call the toll-free number, 1-888-UPDATE-U (1-888-873:"2838). When you sign up, some basic contact information will be required, such as your name, address, email , and phone number. You will set up your own password.
2. Choose your notification preference. During the registration process, you will be asked to select your "primary" method of notification. Registrants currently have the option of being notified by email , telephone, or fax. We think you'll agree that email is a great choice for your primary method of notification because it is instantaneous and it is accessible anywhere, even if a registrant is traveling. It is very important for patients to receive this information about recalls or withdrawals; don't delay in registering and consider email as your primary choice for how to be notified. Your primary notification is always followed by a duplicate notification sent via U.S. mail.
3. Choose the therapies about which you would like to receive notification. Choose "other" if you are unsure of what medicine you are taking or if you wish to receive all notifications.
4. Once you submit the required information , you will receive a confirmation and unique identification number. You will need this number and your password to access the system.

PNS in Action

If a therapy is withdrawn or recalled , the company involved immediately contacts Stericycle, Inc., which then notifies the registrant. Every effort is made to notify registrants within 24 hours Registrants are notified twice. First, you will receive a notification from Stericycle via your designated primary notification method. Second, you will receive a U.S. mail letter containing the same information. The redundancy of two types of notification is intended to ensure that you receive your notification and are aware before you infuse or inject your therapy that there has not been an event. "Event" is the term that is used for a recall or a withdrawal.

Consumers can also go online to www.patientnotificationsystem.org or call a 24-hour, toll-free number 1-888-UPDATE-U (1-888-873-2838) for current information on product recalls or withdrawals. To maximize the usefulness of the system, it is important for consumers to keep accurate infusion logs and record the lot number, therapy and manufacturer for all therapies they use. Infusion logs are available by calling the toll-free number.

PPTA's Role

The system is administered by the Plasma Protein Therapeutics Association (PPTA). The PNS is a comprehensive web-based system that is funded by manufacturers including: Aptevo Therapeutics, Bayer Healthcare LLC, Biotest Pharmaceuticals Corporation , Bioverati U.S. LLC, CSL Behring, Grifols USA, Kedrion Biopharma, NovoNordisk Pharmaceutics, Octapharma, Pfizer and Shire.

Robert Benson

The GBS|CIDP Foundation International acknowledges the five year anniversary of the passing of our founder, Robert Benson, on May 17, 2012.

We take this opportunity to thank all those who contributed to the "Benson Fellow in Neuromuscular Neurology" in his memory. It is a true testament to his legacy.

GBS|CIDP Foundation International WALK AND RUN

2017 Walk-n-Roll Events:

Basking Ridge, NJ ~ June 11
Seattle, WA ~ August 6
Myrtle Beach, NC ~September 9
San Francisco, CA ~ September 10
Washington, DC ~ September 14
Pittsburgh, PA ~ September 16
Staten Island, NY ~ September 16
Portland, Oregon ~ September 17
Indianapolis, IN ~ September 30
Houston, TX ~ October 21
Knoxville, TN ~ October 22
Philadelphia, PA ~ October 29
Ft. Myers, FL ~ November 18
Tampa, FL ~ December 2
Syracuse, NY ~ TBD
Chicago, IL ~ TBD
Boston, MA ~ TBD
Columbus, OH ~ TBD
Phoenix, AZ ~ TBD

Congratulations to Patricia Blomkwist-Markens!

On April 26, 2017, Board Member, Patricia Blomkwist-Markens of the Netherlands, was awarded a "Royal Decoration"—a Dutch recognition of extraordinary services to the community. It is awarded once a year, always on the day before the king's birthday. For this occasion, a special royal decree is issued.

Patricia received the "Knight's Badge," a medal which may only be worn on very special occasions. For everyday use there is a miniature ribbon bar.

Patricia was recognized for her work with the GBS|CIDP Foundation not only in the Netherlands, but internationally as well. We are so proud of you!

Patricia with the Mayor of Amsterdam (Eberhard van der Laan)

Patricia Blomkwist Markens & Johan van Winden, the patient who initiated the request for this royal decoration

GBS | CIDP
Foundation International

CHARITY NAVIGATOR
★★★★
Four Star Charity

Ensuring that Everyone has Access to the Right Diagnosis and the Right Treatment Right Away.

SUPPORT EDUCATION RESEARCH ADVOCACY

What If: You had a cold or flu and four weeks later, you were in intensive care fighting for your life?

What If: You woke up one morning and couldn't stand or walk and suddenly you couldn't move your arms?

What If: You were trapped inside you own body, completely paralyzed and were only able to communicate by blinking your eyes.

What If: You landed in ICU and could hear people talking around you but they never knew you could hear them talking about this: Guillian Barré syndrome could keep you in this state from days to weeks to months.

Guillain Barré syndrome (GBS) can affect anyone at any age. It strikes one in one hundred people occurring when the body's immune system attacks itself causing an inflammatory disorder of the nerves outside of the brain and spinal cord.

What If: You had all of the above symptoms but they came on gradually and you were told that you were simply stressed or tired, or worse that you most likely had MS or ALS.

What If: These symptoms kept increasing and worsening and you were repeatedly sent home from the ER, from physicians who could not diagnose you?

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) is similar to GBS yet slower to progress, is chronic and requires regular plasma infusions (IVIG) to maintain a productive life.

What If: You need support, information, connection, assistance in understanding your new-normal?

THE GBS-CIDP Foundation International is the preeminent, global non-profit organization supporting individuals and families affected by GBS, CIDP and variants.

Founded in 1980 • 35,000 members worldwide • 160 chapters in 47 countries • 35 global centers of excellence • 100 US chapter support group meetings annually • 20 member Global Medical Advisory Board Funding research Improving access to care and global awareness

GBS | CIDP Foundation International
WALK AND ROLL

@GBSCIDP

COMING SOON IN 2017 TO HOUSTON & DALLAS

SUPPORT EDUCATION RESEARCH ADVOCACY

WWW.GBS-CIDP.ORG

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GBS | CIDP
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CHANGE SERVICE REQUESTED

INTRODUCING

2017 One-Day Regional Conferences

These, one-day, regional, chapter meeting/mini-symposia will bring local physicians, patients, families and caregivers together in three convenient locations:

CHICAGO, IL
Saturday, September 23, 2017

SAVE THE DATE!

Join us in San Diego, CA for our 15th Biennial Symposium November 1-3, 2018

Directory

Refer to the enclosed chapter directory to contact the chapter nearest you. In addition, our "Subgroups" are listed below.

Diagnosed with MMN?
Contact: Dominick Spatafora
dominck@dvsconsultants.com

Miller Fisher Variant Group
Please call us for contact with others.

Children with GBS
Lisa Butler, 610-667-0131
GBS/CIDP Foundation International
Email: lisa.butler@gbs-cidp.org
Son, Stuart, had GBS at 5 1/2 years old

Children with CIDP
For children diagnosed with CIDP contact Holly Cannon whose daughter, Hailey, has CIDP.
holly.cannon@gbs-cidp.org

Looking for a 20-Something Contact?
Contact: Kyle Van Mouwerik
kyle.vanmouwerik@gbs-cidp.org

Teenagers with GBS and CIDP
For teens ages 12 to 18 with GBS or CIDP to connect with one another, share stories, and support each other. This group is also open to teenage children of patients. Contact us to find out how to join!

International Office:
610-667-0131

Pregnant Women with GBS
Robin Busch, 203-972-2744
264 Oenoke Ridge
New Canaan, CT 06840
Robin has offered to share her experience with GBS which came about during her pregnancy.

Advocacy
If you are interested in advocacy activities on a federal, state, or local level, contact us to sign up!

Be sure to inform us if you have been diagnosed with one of the following. This will add your name to condition-specific communications.

- ***AMAN***
- ***AMSAN***
- ***Anti-MAG***
- ***Campylobacter***
- ***GBS X2***
- ***Miller Fisher***
- ***MMN***