

GBS|CIDP Foundation International

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# Listening Session with the FDA on Guillain-Barre Syndrome – Patient-led



## **Objective:**

The objective for this listening session was to impart a greater appreciation and understanding of the lifelong battle that the current diagnosis and treatment landscape imparts upon patients that are stricken with Guillain-Barre Syndrome (GBS). It is the Foundation's hope that by highlighting the economic and emotional burden of GBS to the FDA, more complete analyses that consider the patients' perspective may be performed on innovative treatments that may be in development for GBS.

## **Summary of topics discussed:**

*Background of GBS and The GBS|CIDP Foundation International, presented by Lisa B.*

- Guillain-Barre Syndrome (GBS) affects about 2 in 100,000 people and is characterized by weakness, numbness, and ascending paralysis.
- Fortunately, GBS is typically treatable, with intravenous immunoglobulin and plasmapheresis usually reversing the acute symptoms. However, post treatment, many patients still face a long recovery due to the severe nature of the condition and complicated diagnosis process.
- The GBS|CIDP Foundation International was started 40 years ago as a support group in our Founder's (Estelle Benson's) living room. Since then, the Foundation has grown to offer Support, Education, Research, and Advocacy for GBS, CIDP, and variants patients. The Foundation also oversees a Centers of Excellence program of 56 worldwide centers and has invested more than \$4 million in research.
- The Foundation is very grateful to share patient experiences of GBS with the FDA with the hope that future treatments may address the whole patient experience.

## Topic 1: Diagnosing and Treating Guillain-Barre Syndrome When The System Works, presented by GBS patient, Lee Ann G.

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### Diagnosis Process

- Lee Ann recalled her beginning symptoms of Guillain-Barre Syndrome (GBS), highlighting numbness in her feet, tingling, and hip pain. After a day of rest, she noted that she did not feel relief but sought out a chiropractor and tried to return to work.
- With no available chiropractor appointment and worsening symptoms, now including pain, an excruciating headache, and shortness of breath, she finally visited a local emergency room for fear of a serious asthma attack (had previously suffered from asthma). Her husband also noticed left side of her face drooping.
  - Her hope was a quick fix that would allow her enough relief to travel on a family vacation to Mexico the next day. The ER doctor prescribed muscle relaxers with a breathing treatment and wished her well on vacation, urging her to see her primary care physician (PCP) upon return.
- Still feeling unwell, now reporting balance issues and more pain, Lee Ann described a journey to Mexico characterized by increasing debilitation to the point of immobility and described a family vacation where she relied on her husband to help her move, even requiring assistance to get in and out of her bed.
  - Seeing a doctor at the resort they were staying offered little insight, but did give muscle relaxers which only mildly tempered the extreme pain Lee Ann was feeling. At this point, Lee Ann described severe emotional distress at the realization that her body was not functioning and the extreme fear of not being able to get home to a trusted doctor.
- After a difficult journey home, which was almost derailed because of how ill she looked to the flight staff, Lee Ann describes a journey directly from the airport to the emergency room, where a spinal tap was performed to immediately diagnose GBS.

## Treatment

- After the diagnosis, she was admitted to the Intensive Care Unit (ICU) and given pain medication, which finally allowed her to get some rest (the pain had been so excruciating up to this point that she had barely slept).
  - Lee Ann described another wave of emotional trauma as GBS was described to her, with the risk of death being heavy on her mind. It was also found that Lee Ann had Miller-Fisher Syndrome variant of GBS, which affected the muscles in her face, in addition to the traditional GBS.
- Lee Ann underwent a course of intravenous Immunoglobulin (IVIg) for five days, which eventually relieved the GBS symptoms enough for her to move to an inpatient rehab facility, where she underwent rehab with various specialists – speech, physical, occupational, to name a few – 6 days a week for 2 and a half weeks. Lee Ann recounts the physical, mental, and emotional toll that therapy took because it meant relearning how to do everything from walking to chewing.
- After inpatient rehab, Lee Ann completed 10 months of outpatient rehab, with pain and fatigue lingering though most functionality has returned and allowed her to live a fairly normal life.
- Lee Ann mentioned that her diagnosis, treatment, and recovery amount to a medical bill total of \$1 million, which required her and her husband to drain their life-savings, retirement, etc.
  - The stress of paying bills often fell to her husband because Lee Ann was too disabled to work, but eventually was able to return to her job, which was held for her.
- Lee Ann described ongoing symptoms of pain, fatigue, post-traumatic stress, and some residual muscle weakness around her face. Despite this, Lee Ann is grateful for the care she was given and the support she had throughout her GBS journey.

## Topic 2: Diagnosing and Treating Guillain-Barre Syndrome When The System Doesn't Work, presented by GBS patient, Tonya C.

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### Diagnosis Process

- Tonya recalled that before feeling her first symptoms attributed (later) to GBS, she was active, healthy, and an avid runner. She was first prompted to see her PCP because of worsening fatigue, which was attributed to “getting older”.
- She eventually described worsening symptoms, which now included tingling feet, struggling with some movements, pain, and numbness. She again visited her PCP who ordered an ultrasound to check on her thyroid function.
- By the time the ultrasound and other tests were completed with no real significant findings, Tonya was almost fully reliant on her husband to assist her with day to day tasks, often leaning on him to walk. Also, she described excruciating pain, every day, and significant weight loss. Tonya noted that she trusted her PCP, who did not yet recommend a visit to the emergency room, and followed his orders for an echocardiogram to check on her heart health.
- Eventually, Tonya’s husband advocated for a second opinion and they utilized the insurance help line to speak with a nurse and describe symptoms. That nurse advised that they see a neurologist and endocrinologist, and Tonya made the appointment to see the specialists locally.
- Finally, one of the specialists ordered an EMG test, and Tonya described an extremely painful process of needles being placed in random places of soft muscle tissue and electric shocks being delivered to measure nerve functions. The doctor completed the test and immediately took Tonya to be admitted through the emergency room for a full assessment, where she remained for 9 days. At this point it is confirmed that Tonya has GBS and she is admitted to the hospital and started on IVIG, prednisone, and given medicine for her pain (which only dulled the pain but allowed her some relief).

## Treatment

- Tonya recalled being seen by another neurologist when her GBS symptoms were not improving; she was still experiencing extreme pain, and also weakness, numbness, and tingling. She went through a second EMG test, which was again excruciating.
  - The neurologist was not convinced that GBS was causing all of her ongoing symptoms, and Tonya was seeing a number of different specialists.
  - Eventually, a neurologist told her this was going to be “as good as it gets” for Tonya’s recovery. She recalled the extreme emotional trauma as she processed that, and then a new determination to find a way to improve despite this doctor’s assessment.
- Tonya eventually secured a referral to Johns Hopkins and was accepted to be seen there, which is a rigorous process. She was seen at Hopkins after 1 year of experiencing the various symptoms.
  - There, she underwent a 3rd excruciating EMG, and was eventually prescribed plasmapheresis.
- Plasmapheresis brought about a new set of challenges, such as a chest port to maintain and the physical toll brought on by the treatment, but it eventually relieved many of her symptoms and she began to improve after 9 days of inpatient treatment and then a course of outpatient treatment.
- Tonya recalled the financial hardships brought on by managing this complex condition with many specialists while being reduced to a one income household. She also described the lingering symptoms, including pain, chronic fatigue, intermittent numbness and tingling, post-traumatic stress, but maintains a healthy lifestyle as much as possible to give herself the best chance.

## Topic 3: Rehab, Recovery, and Residuals, presented by GBS patient, Jeremy C.

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### Diagnosis Process

- Jeremy opened his topic with the observation that though GBS has similar characteristics, it affects people differently. He also shared that he is a physical therapist.
- Jeremy then emphasized the important role that rehabilitation played in his overall recovery from GBS, noting that the moment when he got the inpatient rehab facility was the first time where he felt that “death wasn’t imminent”.
  - Jeremy recalled that the journey to get to the rehab facility was marked with time spent on a ventilator, being completely paralyzed in bed, with his only means of communication being a bell around his head that was just barely able to move to get attention of the nurses if he had a need.
  - He also recalled that his IVIG treatment was initially unsuccessful, and he sought treatment at Hopkins, which at the time was 1 of 13 Centers of Excellence under the GBS|CIDP Foundation International’s criteria, where he was treated with plasmapheresis. That successfully relieved the GBS symptoms and prompted Jeremy to begin inpatient rehabilitation
- Jeremy described his first impression of rehab as a way to “claw himself back” to the person he used to be. He described an intense schedule of rehab that included 4-5 hours in the gym daily as well as speech, occupational therapy. He recalled the therapy needed to dress himself again.
  - Also included in the typical therapy was pain management and that the progress was monitored by his neurologist.
- Jeremy reiterated that GBS causes total devastation on the body and there is a lot of work required to put the body “back together”. As an example, Jeremy described one of the biggest milestones in the GBS recovery phase was being able to stand on his own for 8 seconds.
- After inpatient rehab, Jeremy recalled that he began an intensive course of outpatient therapy that focused on re-building strength in his body to get as close to normal as possible. After 14 months – roughly 180 visits – of outpatient rehab, Jeremy felt comfortably recovered, but mentioned that he still has some residual symptoms.
- In terms of residuals, Jeremy mentioned that he still feels his speech is “mumbled” that he still suffers from neuropathy, and still experiences post-traumatic stress, which is heightened with every cold/flu season and cough that someone around him experiences. He also noted the social isolation that has come with the pandemic because of the fear of another immune system event that may trigger GBS.

## **Topic 4: Emotional & Economic Burden on the Family, presented by Caregiver, Elsa O.**

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- Elsa told the group about her husband's diagnosis with GBS in May of 2019. Because of his active lifestyle as a bodybuilder and large muscular size, she personally took him very seriously when he remarked that he was not feeling well. However, Elsa recalled the frustration she felt when doctors initially dismissed his symptoms as side effects of possible drug use.
- At this point, Elsa described how her role shifted to that of an advocate for her husband through many different doctors' appointments. As his symptoms progressed, a neurologist eventually recognized the GBS and began IVIG treatment. However, a bad reaction sent her husband into the ICU for monitoring and emergency intubation.
  - He spent 40 days in the ICU and 120 days as a hospital inpatient recovering from GBS and the reaction from IVIG. Elsa mentioned that her advocacy for her husband had to continue throughout the hospital stay as many doctors again looked to blame his symptoms on something attributed to his lifestyle as a bodybuilder.
- Elsa then described the personal transformation she had to undergo, often hiding her fear in front of her husband to cheerlead and bring positivity to his hospital room.
  - Elsa recalled doing this during long days where she would visit her husband in the morning, head to work for a day, and then stop by to check on him later that night.
- Throughout her husband's illness, Elsa recalled the financial strain that was placed on her family that was only mildly relieved with her husband receiving short-term disability assistance. She mentioned that their health insurance co-pays were adding up to \$300 per week.
  - Elsa mentioned that her husband went back to work almost 1 month after coming home from the hospital.
  - Elsa also described that her husband is still recovering, seeing specialists, and might be having surgery to help expedite nerve recovery from damage due to GBS.
- She reiterated that she felt her primary job throughout his illness was that of a caregiver and she was glad to be there to advocate for him as a person and be his voice when he could not.



## **Topic 5: Variants of GBS, presented by Miller Fisher Syndrome patient, Jonathan G.**

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- Jonathan began by describing that the variant of GBS that he experienced – Miller-Fisher Syndrome (MFS) – is very rare, and he experienced the condition twice. He also described that MFS is similar to GBS in that it is monophasic, but differs because it primarily affects the muscles in the eyes and face.
- Jonathan experienced a gradual onset of symptoms over two days, with his most prominent symptom being related to vision problems. He noted that when diagnosed with MFS, the neurologist is often the last specialist someone may see, and they are then sent to a hospital for monitoring,(which is similar to the GBS diagnosis process). Jonathan also described the loss of tendon reflex that comes with MFS, but reiterated that the vision change was the most debilitating symptom.
- Jonathan noted that even after leaving the hospital, his vision problems persisted, and were worsened by his second bout with MFS.
  - After his second MFS diagnosis, Jonathan recalled that his driving ability was greatly affected.
  - After a consult with Hopkins, Jonathan feels that his vision is improving to the point where the discrepancy is only noticeable at an eye doctor visit and is otherwise quite healthy.
- Jonathan remarked that he also lives with the stress and lingering concern of experiencing this condition again. He noted that he feels very concerned for his vision if he were to contract MFS again.

## Topic 6- Pediatric GBS + Unmet Needs of the Pediatric and Adult GBS Community, presented by GBS-CIDP Foundation International Executive Director, Lisa B.

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- Lisa began by reminding the listeners that GBS can affect anyone of any age, race, gender, at any time. The reminder of this for her is that her son experienced GBS at 5 years old, which required two hospitalizations and 4 months before he would take steps again.
- Lisa noted that GBS is so traumatic and tragic that it becomes a formative experience for the patients who have experienced it, as well as the caregivers who helped them through it.
- There are many unmet needs of the community, but Lisa highlighted some of the most significant:
  - Vaccine Reluctance because of the immune system link of GBS and an illness
  - Relatedly, the immune system's role in triggering GBS
  - Pain with GBS – why do some experience excruciating pain (Tonya, Lee Ann) while others (Lisa's son) do not experience any?
  - Fatigue – a common residual for all GBS patients, but not well understood why
  - Access to treatment:
    - Lisa reminded listeners that the community is very fortunate to have “tried and true” treatment options of IVIG and plasmapheresis, but these are very expensive treatments.
    - Additionally, the complex and expensive nature of these treatment mean that patients in low and middle income countries are often left without the option of these treatments
    - Finally, there are accepted treatment guidelines that are not well distributed across the world. An unmet need for the community is physician education of these guidelines.
  - Emerging viruses
    - There was a surge of GBS in relation to the Zika virus that overwhelmed hospitals in developing countries hit hardest by the Zika outbreak.
    - GBS should be considered a complication of all emerging viruses until known otherwise
- Lisa closed the session by thanking speakers and listeners for their time.

# OPEN DISCUSSION WITH FDA

## **Q1: How can the Foundation help connect patients to research opportunities that may answer some of the unmet needs/questions?**

A1 (Lisa B.) – The community is so strong together because GBS is such a formative experience. This means that the Foundation is easily able to engage with a sophisticated network of volunteers that was cultivated over 40 years. As a result, the Foundation maintains a patient registry of those interested in research of more than 1600 patients (GBS, CIDP, and variants) and experiences extremely well-attended educational events. Finally, the Foundation maintains a strong Global Medical Advisory Board that remains at the forefront of GBS research.

## **Q2: In terms of new treatments, what is the greatest unmet need & what symptoms are less treated in GBS patients? In other words, what symptoms would you like to see targeted by new treatments?**

A2 (Lisa B.) Pain and fatigue after GBS recovery remain extremely burdensome to the community. Additionally, the long recovery time from GBS means more muscle deterioration and weakness, so anything to shorten that recovery time would be instrumental. Finally, acknowledging the post-traumatic stress that GBS patients experience.

## Attendee List:

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### FDA Offices Represented (18 offices/divisions):

#### Office of the Commissioner (OC):

- Patient Affairs Staff
- Office of Combination Products
- Office of Orphan Products Development
- Office of Pediatric Therapeutics

#### Center for Biologics Evaluation and Research (CBER)

- Office of the Director
- Office of Tissues and Advanced Therapies, Division of Clinical Evaluation and Pharmacology/Toxicology/General Medicine Branch II
- Office of Vaccines Research and Review
- Office of Vaccines Research and Review/Division of Vaccines & Related Products Applications/Clinical Review Branch 1
- Office of Vaccines Research and Review/Division of Vaccines & Related Products Applications/Clinical Review Branch 2
- Office of Vaccines Research and Review/Division of Vaccines & Related Products Applications/Clinical Review Branch 3
- Office of Communication, Outreach and Development/Division of Communication and Consumer Affairs/Consumer Affairs Branch

#### Center for Drug Evaluation and Research (CDER)

- Office of New Drugs/Office of Neurology
- Office of New Drugs/Office of Neurology/Division of Neurology I
- Office of New Drugs/Office of Neurology/Division of Neurology II
- Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine / Division of Rare Diseases and Medical Genetics (DRDMG)
- Office of New Drugs, Office of Drug Evaluation Sciences, Division of Clinical Outcome Assessment
- Office of the Center Director/Professional Affairs and Stakeholder Engagement

#### Center for Devices and Radiological Health (CDRH)

- Office of Strategic Partnerships and Technology Innovation/ Division of All Hazards Response, Science and Strategic Partnerships

#### Non-FDA Organizations (2):

- Reagan Udall Foundation for the FDA
- European Medicines Agency