GBS|CIDP Foundation International

Speaker Series Summary Episode 24:Review of Treatment Landscape

Overview

Join us for an in-depth review of the current treatment landscape for GBS, CIDP, and MMN with Dr. Jeffrey Allen, Neurologist and Chairman of the GBS|CIDP Foundation International Global Medical Advisory Board. This session will explore established therapies, emerging treatment options, and the latest advances in clinical research. Attendees will gain a clearer understanding of how treatment approaches are evolving to improve outcomes and quality of life for individuals living with these rare neuromuscular conditions.

Summary

Introduction	**Jeffrey Allen, MD is an Associate Professor in the Department of Neurology at the University of Minnesota, Minneapolis, MN and adjunct faculty at Northwestern University, Chicago, IL.** **Dr. Allen's research has focused on defining the clinical and diagnostic boundaries of the inflammatory neuropathies, as well as investigating treatments for those disorders.** **He serves on the GBS|CIDP Foundation International Global Medical Advisory Board as well as the Inflammatory Neuropathy Consortium (INC) board. Dr. Allen also serves as the primary investigator for the Foundation's patient registry.**

With new therapies now available for CIDP, how do clinicians approach first-line treatment decisions?	• **Historically, treatment for CIDP has been dominated by immunoglobulins, primarily IVIG, because they have the strongest evidence.** • **Subcutaneous IG also works well for maintenance. Corticosteroids and plasma exchange are effective options as well.** • **Now that we also have FcRn antagonists available, clinicians can individualize treatment decisions based on disease characteristics, comorbidities, patient preferences, and logistical considerations.** • **The most important thing is choosing an evidence-based therapy and tailoring it to the individual patient.**
What does shared decision-making look like in CIDP treatment?	• **Shared decision-making involves weighing effectiveness, tolerability, side effects, and ease of administration.** • **For example, if starting IVIG, the first goal is determining whether it works by objectively measuring changes in strength, sensation, and function.** • **If it's effective and well tolerated, clinicians then work with patients to customize dosing or consider alternative options that may reduce treatment burden while maintaining efficacy.**
How does treatment differ for patients with multifocal motor neuropathy (MMN)?	• **MMN is more limited in treatment options. IVIG remains the primary and proven effective therapy. The challenge is finding the right dose and frequency, which can vary significantly between patients and even over time in the same patient.** • **Clinicians aim to maximize benefit while minimizing unnecessary exposure and treatment burden.**

What do we know about the long-term safety of IVIG and subcutaneous IG?	• **Overall, immunoglobulin therapies are very safe, with extensive long-term data from both clinical trials and real-world use.** • **Common side effects include headache, nausea, and fatigue.** • **Serious events like thromboembolic complications are rare but can occur, particularly in patients with cardiovascular risk factors.** • **Risk is assessed individually, and strategies such as dose adjustments or switching to subcutaneous IG may help reduce risk.**
How do clinicians decide between IVIG and subcutaneous IG?	• **From an efficacy standpoint, both work similarly.** • **The main considerations are dosing accuracy, patient autonomy, IV access issues, and comfort with self-administration.** • **Subcutaneous IG can provide greater independence and eliminate the need for IV access, but it's not ideal for everyone.** • **If subcutaneous IG is ineffective, it's often due to incorrect dosing rather than lack of efficacy.**
Are there biomarkers that can guide diagnosis or treatment decisions in CIDP or MMN?	• **There are two main areas of biomarker research: improving diagnostic certainty and monitoring disease activity.** • **CIDP remains a clinical diagnosis, and there is no single definitive test.** • **Biomarkers such as neurofilament light chain are being studied, but they are nonspecific and not yet reliable for tracking disease activity in real time.** • **The future likely involves using a combination of biomarkers rather than a single test.**
What is neurofilament light chain, and how might it be used?	• **Neurofilament light chain is a protein released when nerves are damaged.** • **Levels can be elevated in many neurological conditions, so it is not specific to CIDP or MMN.** • **It may be helpful at diagnosis to indicate active nerve damage, but it is less useful for monitoring short-term disease fluctuations or treatment response.**

How has the standard of care for GBS changed, and what therapies are being developed?	• **GBS is an acute immune attack on nerves that requires rapid intervention.** • **Standard treatments remain IVIG and plasma exchange. Emerging therapies focus on blocking the complement system, which plays a key role in nerve damage during acute GBS.** • **Complement inhibitors aim to protect nerves during the immune attack and potentially improve recovery outcomes.**
What is efgartigimod, and who may benefit most from it?	• **Efgartigimod is an FcRn antagonist approved for adults with CIDP.** • **It works by reducing IgG antibodies, including pathogenic antibodies that may attack nerves. In clinical trials, it was effective in preventing relapse for many patients.** • **It may be especially beneficial for patients who respond to IVIG but struggle with side effects or treatment burden.** • **It is administered as a once-weekly subcutaneous injection.**
Has plasmapheresis fallen out of favor?	**No, plasmapheresis is still effective for some patients. However, it can be burdensome to administer long-term, requiring frequent treatments, which limits its practicality for many people.**
What are the most promising therapies currently in clinical trials?	**In addition to FcRn antagonists, complement inhibitors are a major area of active research in CIDP, MMN, and GBS. Early-phase trials have shown encouraging results, including improvement in patients who were refractory to standard therapies. Several phase**

Final takeaways!	**More treatment options are now available for CIDP.** • **The addition of FcRn inhibitors expands an already evolving treatment landscape beyond IVIG, subcutaneous IG, steroids, and plasma exchange.** **Care must be personalized and patient-centered.** • **Treatment decisions across CIDP, MMN, and GBS depend on individual disease features, comorbidities, treatment burden, and patient preferences.** **IVIG remains foundational, especially for MMN.** • **IVIG continues to be the primary proven therapy for MMN and a cornerstone for CIDP, with strong long-term safety data.** **Research is driving the next generation of targeted therapies.** • **Complement inhibitors and other targeted approaches show promising early results, particularly for patients who do not respond to standard treatments.** **Early intervention, access, and participation matter.** • **Timely treatment, overcoming insurance barriers, and patient participation in clinical trials are essential to improving outcomes and advancing care.**

Relevant Resources

Centers of Excellence: https://www.gbs-cidp.org/support/centers-of-excellence/

Doctor to Doctor Consult: https://www.gbs-cidp.org/doctor-to-doctor/

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