



NORD®
National Organization
for Rare Disorders

How to Connect with Scientists and Industry Stakeholders

Eric Marsh, MD, PhD, Clinical Director Orphan Disease
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International



ENGAGING ACADEMICS AND INDUSTRY

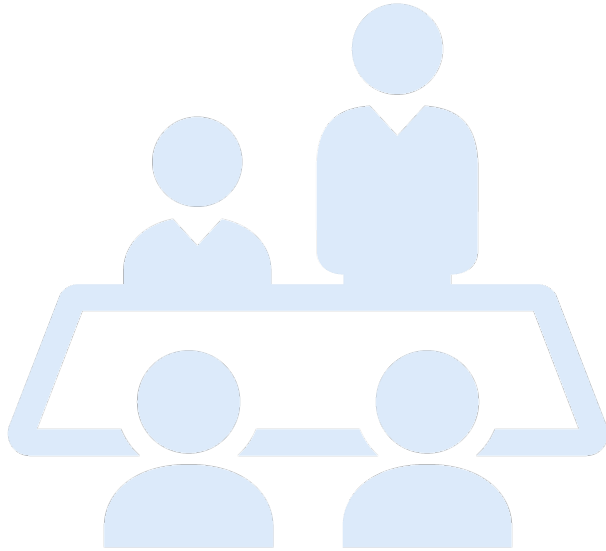
Eric Marsh MD, PhD

- Clinical Director: Orphan Disease Center
- Associate Professor of Neurology & Pediatrics at Perelman School of Medicine at the University of Pennsylvania
- Attending Neurologist, Children's Hospital of Philadelphia



My Background

- Clinical
 - Child Neurologist
 - Peds Epilepsy Fellowship trained
 - Director of Neurogenetics clinic
 - Rett/CDKL5/Dravet centers
- Basic Research
 - Mechanisms of seizures and cognitive dysfunction in the developing brain
 - Focus on mouse models with genetic changes causing epilepsy and cognitive changes
- Translational/Clinical Research
 - Clinical trials for rare epilepsies
 - Natural history studies-Rett and Related disorders
 - Biomarker studies using EEG and electrophysiological measures



MEDICAL ADVISORY BOARDS

- Lennox Gastaut Foundation
- Citizens United for Research on Epilepsy (CURE EPILEPSY)
- International Rett Syndrome Foundation

COMMITTEE ROLES

- American Epilepsy Foundation
- Epilepsy Foundation
- Grant Reviewer- many foundations

COMPANY ADVISING

- A number of companies



Approaching Clinician Scientists

- Intent
- Commitment
- Responsibilities
- Funding



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GBS|CIDP Foundation International

Lisa Butler, Executive Director

National Organization for Rare Disorders | rarediseases.org



ENGAGING WITH THE MEDICAL AND SCIENTIFIC COMMUNITY

- How to form a Medical/Scientific Advisory Board
- How to start a research fund
- Patient Reported registries

Funding Sources

As part of the GBS|CIDP Foundation International's mission to provide education and support research, we offer research grants to qualified applicants.

This is made possible through contributions from individuals who have been personally touched by GBS or CIDP and by several commercial sources. Through the generosity of these contributors, to whom the Foundation is most grateful, we are able to offer a Research Grants Program. The GBS|CIDP Foundation International has worldwide representation, sharing resources with local support groups, chapters nationally and internationally. The Foundation's Global Medical Advisory Board includes many of the most respected clinicians and researchers in the field.

Areas of Interest

Foundation interests include GBS, both demyelinating and axonal variants, Miller Fisher syndrome, CIDP, multifocal motor neuropathy, and related conditions.

Research interests cover a broad range of subjects including development of methods to more rapidly diagnose these disorders, review of educational and communication programs to inform patients and families of the Foundation's availability, identification of mechanisms involved in the pathogenesis of these disorders, development of more effective treatments to limit the disease process, prevent complications and reduce long-term adverse sequelae.

MISSION STATEMENT

To improve the quality of life for individuals and families worldwide affected by GBS, CIDP and variants by:

- Providing research for all patients, their caregivers and families so that GBS or CIDP patients can benefit in the Foundation for support and reliable, up-to-date information.
- Providing public and professional educational programs worldwide designed to heighten awareness and improve the understanding and treatment of GBS, CIDP and variants.
- Expanding the Foundation's role in sponsoring research and engaging in patient advocacy.

MORE INFORMATION

GBS|CIDP Foundation International
375 E. Elm Street Suite 301
Cincinnati, OH 45202
616.607.0333 ext. 101
616.624.8201 ext. 101
grants@gb-cidp.org
www.gb-cidp.org

RESEARCH GRANTS



RESEARCH

Home / Research

RESEARCH IS CRITICAL TO SERVING THE CAUSE AND THE COMMUNITY

The GBS|CIDP Foundation International believes in supporting research on GBS, CIDP and variants by funding grants for projects that contribute to the knowledge of, and towards an eventual cure for, GBS, CIDP and variants.

To date, we have committed \$4 million towards basic and translational research.

[GBS|CIDP RESEARCH REPORT](#)[APPLY FOR A RESEARCH GRANT](#)

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GLOBAL MEDICAL ADVISORY BOARD

Home / About / Global Medical Advisory Board



SOME OF THE BEST EXPERTS IN THE WORLD

The Foundation's Global Medical Advisory Board includes experts in the diagnosis, treatment and research of GBS and CIDP, many of whom have authored textbooks on the disorder, and is considered to be the "think-tank" of GBS and CIDP disorders.

GBS|CIDP PATIENT REGISTRY

Home / Research / GBS|CIDP Patient Registry

How has a GBS|CIDP diagnosis affected you? Tell us about it!

Join the GBS|CIDP Patient Registry, share your experience, and play a critical role in a better tomorrow for patients everywhere.

The GBS|CIDP Foundation invites you to join the online Patient Registry for patients with GBS|CIDP or variants of the condition. The GBS|CIDP Patient Registry, consisting of patient reported standardized questionnaires, will allow healthcare professionals access to critical, de-identified patient data and a more in-depth understanding of the patient experience. This unique collection of data can be critical for the pursuit of medical advancements and development of patient resources. The registry will be hosted in partnership with the National Organization for Rare Disorders (NORD), an independent non-profit patient advocacy organization dedicated to individuals with rare diseases and the organizations who serve them. Studies of these conditions may take years to finish, but patient reported registries provide power in numbers! The Foundation will provide periodic updates and results with the discoveries made possible with this impactful data.

[Apply for Research Grants](#)[Foundation Funded Research](#)[Research News](#)[Clinical Trials](#)[Academic Research Partners](#)[GBS|CIDP Patient Registry](#)[Learn About Walk & Roll to Support Research](#)[Donate to Support Research](#)[COVID-19 UPDATES](#)[WALK & ROLL FOR RESEARCH](#)

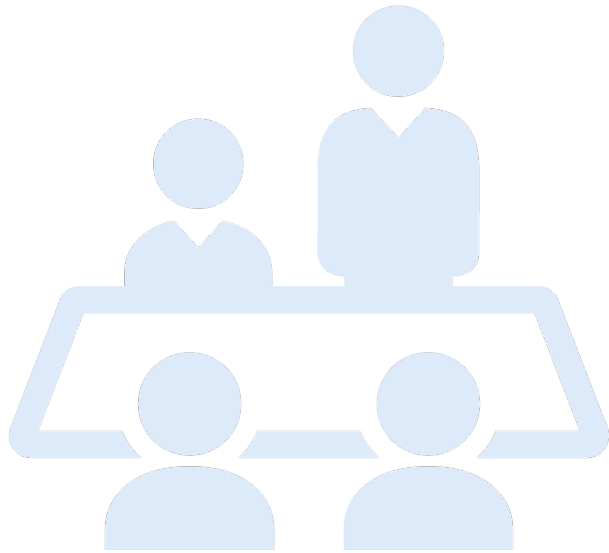


GBS|CIDP FOUNDATION INTERNATIONAL



- Mission
- Foundations
- Infrastructure





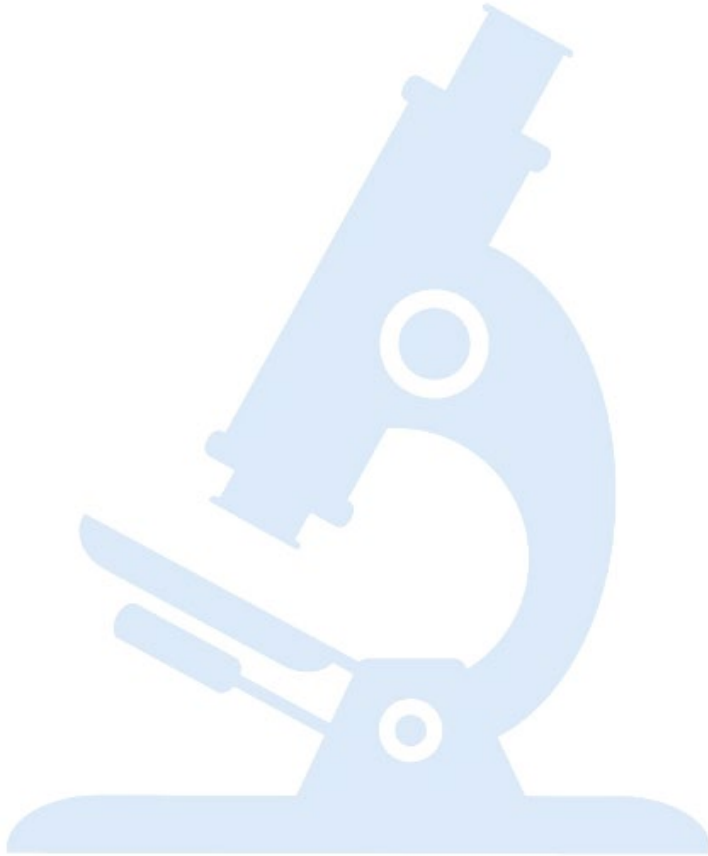
MEDICAL ADVISORY BOARD

- Defining the role
- Establishing Committees





RESEARCH



- Intent
- Engagement
- Funding



PATIENT REPORTED REGISTRIES

GBS|CIDP Patient Registry

The patient registry is a collection of standardized information regarding patients who share a condition and is used for a variety of purposes such as conducting natural history studies, supporting disease specific clinical trial recruitment, and developing patient resources.

Enroll Now!

- Intent
- Platform: IAMRARE
- Patient reported v. physician reported
- Marketing, marketing, marketing



CONCLUSION

- Mission
- Transparency
- Connections
- People, people, people!



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