IGA INTERNATIONAL GAUCHER ALLIANCE
Driven by passion for patients

August 2023 Newsletter

Switching and Monitoring Best Practice for Gaucher Disease

The International Working Group on Gaucher Disease (IWGGD) and International Gaucher Alliance (IGA) have prepared a consensus document on Switching and Monitoring Best Practice for Gaucher Disease.

Recently, patients living with Gaucher disease, their caregivers and families, and patient organisations, have increasing concerns about the circumstances of switching medicines used to treat this rare condition.

This document sets out the issues involved and outlines best practice in various areas relating to switching and monitoring.

Read the document

The power of knowledge in a timely diagnosis

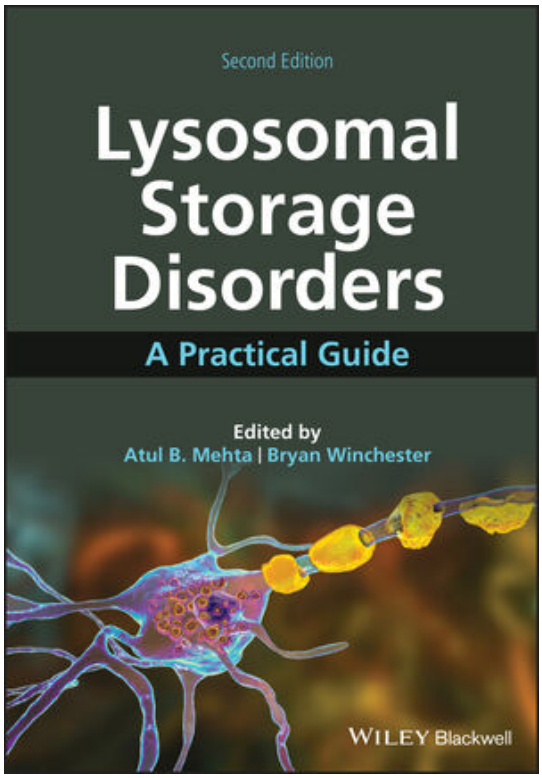
Building a better future

INTERNATIONAL GAUCHER DAY
1 OCTOBER
International Gaucher Alliance

Save the date

and watch for our exciting new video in which Gaucher patients, their carers and physicians speak about their diagnostic journeys.

Launching on 1 October



IGA contributes to the education of physicians treating Gaucher patients by distributing new Lysosomal disorders book

The IGA has recently gifted 63 copies of the book ***Lysosomal Storage Disorders: A practical guide*** to support treating physician of patients of the Takeda charitable access programme as part of their ongoing education. The IGA hopes the book will be a useful resource in the physicians' work and encourages them share it with other colleagues that they feel would benefit from it.

The books are being delivered to charitable access programme physicians in the following countries: Belarus, Egypt, Ghana, India, Kenya, Morocco, Pakistan, Paraguay, Tanzania and Tunisia.

This book is the 2nd edition and when the first edition was published in 2005, the then EGA were given a considerable number of copies free of charge to distribute to doctors in Eastern Europe and other countries. We believe this was of great benefit to those doctors and wanted to be able to do this again with this latest version.

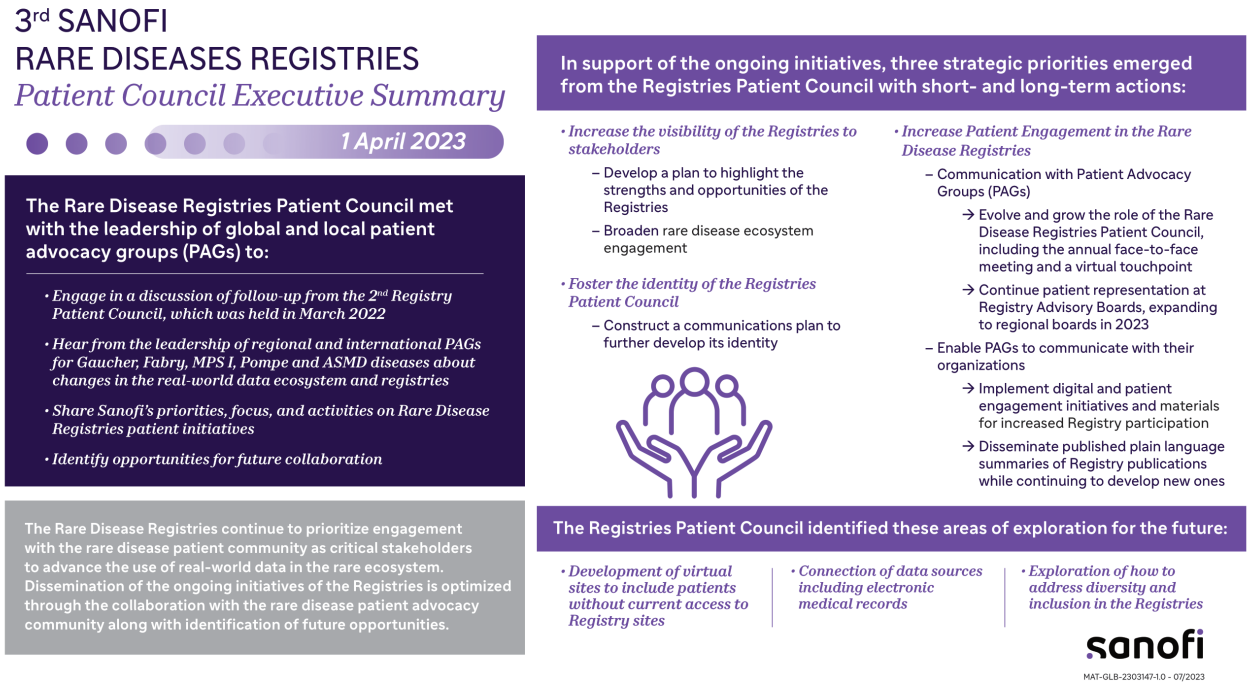
As an international umbrella organisation representing the global Gaucher patient community, the IGA's vision is 'A world where all Gaucher patients have the best possible quality of life, access to the treatment and care they need, looking forward to the development of a cure.' The gifting of this book supports improved education and the care and support to Gaucher patients and other lysosomal patients.

IGA and GARDIAN at SSIEM

IGA Projects Officer Vesna Aleksovska has been representing the IGA and GARDIAN at the annual symposium of the Society for the Study of Inborn Errors of Metabolism (SSIEM) in Jerusalem.

See next month's newsletter for a full report.

Sanofi Rare Disease Registries Patient Council



Board Member Irena Žnidar and CEO Tanya Collin-Histed represented the IGA bas one of 9 global and local patient advocacy groups (PAGs) to participate in the 3rd Sanofi Rare Disease Registries Patient Council. The Council met in Amsterdam on 1 April 2023 to:

• Engage in a discussion of follow-up from the 2nd Registry Patient Council, which was held in March 2022

• Hear from the leadership of regional and international PAGs for Gaucher, Fabry, MPS I, Pompe and ASMD diseases about changes in the real-world data ecosystem and registries

• Share Sanofi's priorities, focus, and activities on Rare Disease Registries patient initiatives

• Identify opportunities for future collaboration

The meeting was an exciting day of sharing perspectives of Council members on the importance of real-world data/evidence and the ongoing collaboration with the Rare Disease Registries. We are happy to share the executive summary.

In addition, we are excited to announce that an abstract co-authored by the Council was accepted for a poster presentation at the 2023 Annual Symposium of Society for the Study of Inborn Errors of Metabolism (SSIEM). "A rare partnership: community and industry collaboration to shape the impact of real-world evidence on the rare disease ecosystem" is being presented on behalf of the entire Council at SSIEM on 29 August – 1 September 2023.

New blog post from the National Gaucher Foundation

How Systemic Inflammation Affects Your Brain & Central Nervous System

For people living with an inflammatory disease, such as Gaucher disease, a faulty immune system can wreak havoc on the body. Inflammation is one of the ways the immune system protects and heals the body. But constant immune activation can cause inflammation throughout the body, which may affect physical and mental function. This is especially true if the systemic inflammation throws the central nervous system (CNS) off-balance.

The National Gaucher Foundation in the USA spoke with Gregory Grabowski, MD, to understand how systemic inflammation affects the CNS. He is professor emeritus of Pediatrics and of Molecular Genetics, Biochemistry, and Microbiology at the University of Cincinnati College of Medicine. Dr. Grabowski is also a prolific Gaucher disease researcher. He founded of one of the world's largest clinics for Gaucher disease and the first enzyme replacement therapy center. He is a member of the National Gaucher Foundation's Medical Advisory Board.

Read the post

Volunteer Spotlight

Kachengwa Ghambi

I love supporting initiatives that reach out to improve people's lives. I develop and implement digital tools for health. I studied Computer Science and Statistics at the University of Malawi-Chancellor College. I am passionate about creating digital tools that support health systems and data analysis for decision-making. I have supported health research projects through the development of research data capture modules as well as the creation of data visualizations.

I am happy to support the work of IGA with my skills and experience. I previously helped women's health projects by programming tools in REDCap, Open Data Kit XLS/XML forms, SurveyCTO, mWater, mhealth-colposcopy tools using MobileODT EVA, and Periwinkle Smartscope as well as data analysis and visualization in Stata, SPSS, and Tableau. I am always ready to take up new challenges and learn new platforms.

I am also a freelance creative content creator. I develop for print and digital platforms using Adobe Suite (Photoshop, Illustrator, Indesign, After Effects, and Premiere Pro), CorelDraw, WordPress, and Drupal. I enjoy playing and watching football.

Please contact Vesna if you are interested in volunteering with us.

GARDIAN UNLOCKS

Enhancing outcomes for those with neuronopathic Gaucher Disease

GARDIAN

GARDIAN DATA HELPS US

TO KNOW WHERE PATIENTS ARE TO HELP RECRUITMENT IN CLINICAL TRIALS ON NEURONOPATHIC GAUCHER DISEASE

GARDIAN
is already showing results
and there is still time to play your part!
Join the GARDIAN Registry today at:
gardianregistry.org

Click here for more information and to register

International Gaucher Alliance

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