IGA INTERNATIONAL GAUCHER ALLIANCE
Driven by passion for patients

International advocacy for Gaucher patients since 1994

28th May 2026

Bringing together the latest updates, patient perspectives, research highlights, and global community news from IWGGD.

MAY 2026 Newsletter

WELCOME

Welcome to the May 2026 edition of the International Gaucher Alliance newsletter. This month's newsletter focuses on highlights from the 3rd International Working Group on Gaucher Disease (IWGGD) Symposium held in Trieste, Italy. The meeting brought together clinicians, researchers, patient advocates, industry representatives, and families from across the global Gaucher community to discuss the latest advances in research, patient care, collaboration, and advocacy. We are delighted to share reflections, scientific updates, working group developments, and community voices from the meeting, alongside updates from across the International Gaucher Alliance network.

IWGGD 2026 – Bringing the Global Gaucher Community Together

By Tanya Collin-Histed
Chief Executive Officer, IGA

The International Gaucher Alliance would like to warmly congratulate the IWGGD Board on the success of the 3rd IWGGD Meeting held in Trieste, Italy. The meeting highlighted the strength of the global Gaucher community, bringing together clinicians, researchers, patient advocates, and organisations with a shared commitment to improving the lives of people living with Gaucher disease. The first session of this year's meeting placed a strong focus on Africa, helping raise awareness of the ongoing challenges surrounding diagnosis, treatment access, and healthcare inequality while also highlighting the growing collaboration and advocacy efforts taking place across the continent.

The symposium created valuable opportunities for scientific exchange, international collaboration, and stronger engagement between healthcare professionals, researchers, and patient organisations worldwide.

Working groups on Gaucher disease

By Vesna Aleksovska

The symposium began with the International Working Group on Gaucher Disease (IWGGD) Working Groups in action, bringing together international experts to discuss key challenges and define future directions in Gaucher disease. These sessions are a cornerstone of IWGGD, fostering collaboration, knowledge exchange, and the development of strategies that directly impact research and clinical practice. IGA representatives participated in different working groups, as patients have a chair at the table at IWGGD. The Supportive Care Working Group was focusing on ongoing projects and the need for new people to take the lead on different subjects.

Key developments:

The group reaffirmed its commitment to patient-centred care, particularly for those living with neuronopathic Gaucher disease (nGD).
Two vital clinical documents have been updated and are moving toward final review:

- Women's Health: Comprehensive updates on supportive care for women with Gaucher disease.
- Paediatric Care: New insights into symptomatic care for children with non-neuronopathic GD.

IWGGD The International Working Group on Gaucher Disease

Several initiatives are nearing the finish line to make managing Gaucher disease more accessible:

- **Home Treatment**: New international guidelines for home therapy are in the final stages, aiming to standardise and safeguard treatment outside of hospital settings.
- **Self-Management**: A full guide on self-management is now complete. Our next step is creating shorter, "easy-read" versions so every patient can feel empowered to manage their health journey.
- **Mental Health and Transition**: Work is ongoing to support adolescents as they transition to adult care, including new tools to help monitor and address anxiety and depression.

Tools for the Community

- **Digital Innovation**: The Huma app pilot is currently underway with IGA members, exploring how digital health can better track our symptoms and well-being.
- **Early Detection**: New Pre-GD clinic questionnaires are available online in several languages, helping clinicians identify needs earlier than ever before.
- **Lifelong Support**: Dedicated research is moving forward regarding care for the elderly living with GD, as well as strategies for managing irreversible manifestations like pulmonary disease or the long-term effects of splenectomy.

From Trieste With Purpose: An African Patient Advocate's Reflections on IWGGD 2026

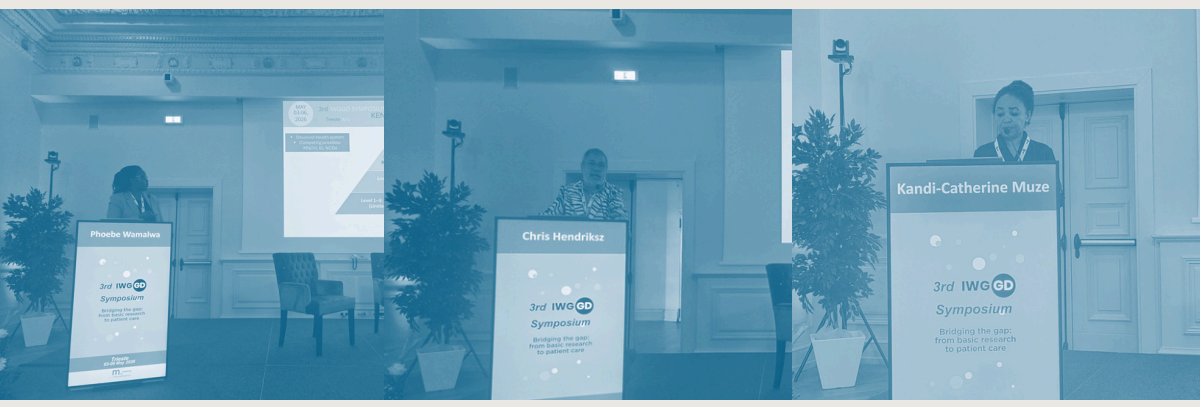
From May 3 to 6, 2026, the city of Trieste, Italy, became a gathering point for some of the world's most dedicated minds in Gaucher Disease research and advocacy. I had the privilege of attending the International Working Group on Gaucher Disease (IWGGD) 2026 Scientific Symposium, not only as an Africa patient advocate but also as a speaker, representing the voice and realities of Gaucher patients across our continent. It was an experience that stayed with me long after I boarded my flight home.

The Power of a Shared Purpose

What struck me most in Trieste was not a single session or a landmark scientific finding but the people. The camaraderie in that room was genuine and deeply moving. Researchers, clinicians, patient advocates, and policy voices from across the globe sat together, each bringing their piece of the puzzle, united by a shared commitment to improve the lives of people living with Gaucher disease.

Discussions throughout the symposium underscored critical themes that resonate globally: the importance of early diagnosis, collaborative models of care, patient-centered approaches, and the urgent need to strengthen health systems, including within low- and middle-income countries. For me, hearing these themes articulated by global experts validated what we see and live every day in Africa.

I was fortunate to travel to Trieste alongside three fellow delegates from Africa who also contributed meaningfully to the programme. Dr. Phoebe Wamalwa, Consultant Pediatric Endocrinologist and Chair of the Pediatric Endocrine Society Kenya, and Dr. Kandi-Catherine Muze, Pediatrician and Endocrinologist from Tanzania, jointly presented case studies sharing firsthand experiences and challenges in diagnosing and managing Gaucher disease from an African clinical perspective.



Professor Christian Hendriksz, Chief Community Impact Officer at A Rare Cause, delivered a plenary lecture providing an overview of the current situation of rare diseases in Africa, bringing both global expertise and a deep understanding of the African context to the main stage.

Dr. Phoebe Wamalwa, captured it well: sharing experiences from clinical practice in Kenya while learning from global experts was both enriching and inspiring, reinforcing the need for continued collaboration, research, and advocacy to improve outcomes for patients living with Gaucher disease and other rare disorders.

Dr. Kandi-Catherine Muze, who coordinates national care pathways for children living with Gaucher disease in Tanzania, reflected that despite the many barriers including limited diagnostic facilities and restricted access to treatment, the symposium was an opportunity to highlight the important milestones already achieved in Tanzania. She noted that these achievements have been made possible through strong collaborations with international partners, donors, and healthcare organisations, and expressed hope that future symposiums will bring even greater progress to share.

Dr. Phoebe Wamalwa

Professor Christian Hendriksz, noted that the programme moved coherently from basic science through clinical insights to emerging therapies, and that it was particularly encouraging to see strong interest in the African updates and growing recognition of the importance of integrating underserved regions into the global Gaucher community.

Professor Hendriksz also highlighted the scientific depth of the symposium. Sessions spanned the full journey from laboratory research to patient care, with notable discussions on the link between Gaucher disease and Parkinson's disease, advances in understanding disease variation across patients, and the growing use of biomarkers and real-world data to improve diagnosis and monitoring.

Exciting progress in gene therapy, genome editing, and next-generation treatments illustrated how rapidly the field is moving. Taken together, the science presented in Trieste reflected a field increasingly driven by molecular insight, clinical evidence, and global collaboration, all aimed at delivering more effective and equitable care for patients.
The scientific poster sessions added further depth, showcasing emerging data, early-stage studies, and innovative approaches across the field. Combined with the rich networking opportunities with international experts, it made for a truly memorable and impactful meeting.

Speaking Up for Africa: What I Shared

During my session, IGA in Africa: Highlights of Ongoing Initiatives, I had the opportunity to bring the African perspective to the global stage. I structured my talk around four key areas.

1. Why IGA in Africa Matters
Gaucher disease does not discriminate by geography. Yet the resources, awareness, and infrastructure to diagnose and manage it are deeply unequal across the world. It is almost certainly more prevalent in Africa than current numbers suggest, with many cases going undetected and undiagnosed. For them, IGA's presence and engagement in Africa is not just welcome. It is essential.

2. IGA's Approach in Africa
IGA's work in Africa focuses on education, patient and family support, advocacy for equitable access to treatment, and strengthening collaboration across the continent. Through initiatives such as the Africa Roadmap Project and the Africa Gaucher WhatsApp Support Group, IGA is helping improve diagnosis, connect patients and clinicians, and build stronger support networks for families affected by Gaucher disease.

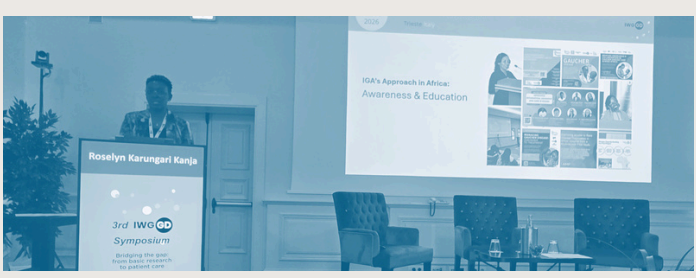
3. What Still Needs to Change
Many patients across Africa continue to experience delayed diagnosis, limited access to enzyme and genetic testing, healthcare inequality, and inconsistent access to treatment. Rare diseases remain under-recognised within many healthcare systems, while limited funding, awareness, and local data continue to create barriers to diagnosis, policy change, and long-term patient support.

4. The Opportunity for Africa
Despite these challenges, there is real opportunity for progress. By strengthening advocacy, expanding collaboration, improving access to diagnosis and treatment, and including African patients in research and clinical trials, Africa has the potential to build more accessible and patient-centred approaches to rare disease care for the future.

The Opportunity for Africa

I did not come to Trieste only to speak of challenges. I also came to speak of hope, because there is real and genuine reason for it. Africa has a unique opportunity to leapfrog traditional models and adopt more affordable, scalable diagnostic approaches, including by building decentralised diagnostic networks that bring testing closer to patients rather than requiring them to travel long distances or out of the country.

We do not need to repeat the slow journey others have taken. We can learn from it and move faster. The current global attention on rare diseases creates momentum we can harness. Digital health solutions offer powerful ways to bridge geographical gaps and address specialist shortages across our vast and diverse continent.



Young people and caregivers are increasingly leading advocacy efforts, raising awareness, influencing policy, and supporting one another. This movement is already taking root in several African countries and represents one of our greatest assets.

Africa also does not need to start from scratch. Existing systems such as laboratory networks, community health programmes, and referral structures used for diseases like HIV, TB, and malaria can be adapted to support rare disease diagnosis and care. Leveraging what already works offers a pragmatic, cost-effective path forward. What we need is a collaborative model that brings together diagnosis, regulation, treatment access, and ethics into coherent national and regional plans. And underpinning all of this is a principle I feel strongly about: ethical use of data. Data always belongs to the patient. Any data collected from African patients must directly benefit those patients, not serve the interests of others at their expense.
I also called for something long overdue: the inclusion of African patients in clinical trials. Scientific progress that excludes Africa is incomplete progress.

A CALL TO ACTION

I returned from Trieste energised, but more aware than ever of how much work remains. To patient advocates and community members: your voice matters — engage, connect, and make yourselves known. To healthcare providers: earlier awareness of Gaucher disease can prevent years of suffering caused by delayed diagnosis. To researchers, policymakers, and funders: Africa must be included through research partnerships, funding, and patient participation in clinical trials. And to the global Gaucher community: thank you for your solidarity. Together, we are working toward a world where every person living with Gaucher disease has a fair chance at diagnosis, treatment, and a full life,writes Vesna

3rd IWGGD Symposium in Trieste: Scientific Exchange, Collaboration, and Patient-Centered Advances in Gaucher Disease.

The 3rd International Working Group on Gaucher Disease (IWGGD) Symposium, held in Trieste, Italy, from May 3 to 6, 2026, represented an important opportunity for scientific exchange, international collaboration, and interdisciplinary dialogue within the field of Gaucher Disease (GD). Under the theme "Bridging the Gap: From Basic Research to Patient Care," the symposium brought together researchers, healthcare professionals, patient advocates, and industry representatives from multiple countries, fostering discussions on advances in clinical management, translational research, innovative therapies, and patient-centered care.

The International Gaucher Alliance's working groups presented several research projects, which were given as posters and oral presentations, which I took part in. I had the honor of presenting the oral communication about the "Guidelines on Home Treatment in Gaucher Disease," developed through an international collaborative initiative coordinated by IGA since 2022.

The development of these guidelines constitutes a significant contribution to ongoing discussions regarding equitable access to treatment and the implementation of patient-centered healthcare models. In contexts where home infusion programs remain limited, the dissemination of evidence supporting the safety and feasibility of home treatment may contribute to improving quality of life, treatment adherence, and healthcare accessibility for individuals living with GD.

Further reinforcing the theme of patient empowerment, the IGA collaborative poster: "Beyond the Clinic: Empowering Self-Management and Living Well with Gaucher Disease", addressed the critical role of self-management in chronic illness. The work addressed the importance of self-management strategies in the context of chronic diseases, highlighting educational tools and practical resources aimed at supporting patients and families in the daily management of Gaucher Disease, aiming for a better quality of life. The discussion on self-management is particularly relevant in health systems where multidisciplinary support and specialized services are still unevenly distributed, so that the patient and their family members can feel safer in managing this illness throughout their lives.

To ensure these insights reach the wider community, the IGA team conducted a series of interviews with speakers and participants throughout the congress. These will be published gradually across IGA communication channel, contributing to a wider dissemination of the scientific knowledge shared during the congress to our patient community around the world.

When Science and Patient Voices Move Forward Together

It was encouraging to see several contributions from Brazilian researchers at the symposium. Among them, I presented my PhD research using qualitative approaches to expand discussions beyond biomedical perspectives about this disease ("Case Studies on the Illness Experience with Gaucher Disease Type 1 in Brazil: A Qualitative and Socio-anthropological Study").

The symposium program highlighted the importance of interdisciplinary and international collaboration in advancing Gaucher Disease research, while strengthening connections between science, clinical practice, and patient experience through more inclusive and participatory approaches to healthcare.

For regional patient communities, participation in international forums of this calibre is essential. It increases local visibility, strengthens international collaborative networks, and ensures that regional challenges are represented in global discussions and emerging innovations in the field of rare diseases.

The 3rd IWGGD Symposium provided a meaningful reminder that progress in Gaucher Disease research and care is built collectively—and that true advancement is achieved only when science, clinical practice, and the lived patient experience move forward together.

Raul Chertkoff
IGA Board Member

Gaucher patients are no longer alone.

When my eldest son was diagnosed with the rare Gaucher disease, we were alone in the clinic, there were no other patients, and we thought it was just us. That was a long time ago. That was at a time when the first treatment for the disease was discovered and approved. Today, 3 decades later, it seems that patients are not alone, this was significantly expressed at the recent International Working Group on Gaucher Disease (IWGGD) conference in TRIESTE, Italy, this May, which I had the privilege of attending.

A meeting of scientists, researchers, experienced treating physicians and doctors starting their journey in treating the disease, as well as pharmaceutical companies supporting the community, but not only them. Patients and families have a significant representation through the International Gaucher Alliance (IGA). The first part of the conference discussed the situation of rare diseases and Gaucher disease in Africa, an overview of the challenges for bringing the correct diagnosis and treatment of rare diseases in Africa.

The discussion raised and deepened the awareness of the attendees on this important subject, with Gaucher disease being an example.The treatment of Gaucher disease has advanced, with more than four treatments approved today, and research into new treatments is underway to address the challenges of the disease, such as the treatment for NGD (Neuronopathic Gaucher disease).

There is no doubt that the participation of all those attendees at such a conference is a winning combination to keep trying and make the diagnosis and treatment available. But the problem of access to treatment is still a barrier as it exists in so many countries around the world, and Africa is just one example, issue that needs to be solved. One of the important targets of the IGA.

If you read this short paragraph and you think you can support the IGA efforts, please contact us.

CONTACT US

IWGGD Poster Highlights

We are delighted to share that the #InternationalGaucherDay2025 poster campaign was recognised at the 3rd IWGGD Symposium in Trieste. The campaign marked a month of global awareness activities and reached more than 2 million people worldwide. We extend our sincere thanks to the global Gaucher community for contributing, participating, and sharing their stories. Click below to view.

The heart of advocacy : How your story changes the world.

A global exchange on improving the quality of life in the Gaucher community

***Moderator: Vesna Aleksovska**, Gaucher patient, IGA developmental programme, RD patient advocate.*
Speakers:
***Andre Balezkiene**, Lithuania, Mother of Gaucher patient and patient advocate, IGA board director.*
***Roselyn Kanja Odero**, Kenya, Rare disorders Kenya, Gaucher leader.*
***Dr Imalke Kankananarachchi**, Sri Lanka, Rare Disease Forum, Sri Lanka College of Paediatrician*
***Alexandra Gallesse**, Peru, Gaucher patient advocate, president of APPEL.*

Click here to watch. WATCH NOW

Exclusive IWGGD Interviews

Over the coming weeks, we'll be sharing a series of interviews and highlights from the 3rd IWGGD Symposium in Trieste, featuring experts, researchers, clinicians, and patient advocates from across the global Gaucher community. Keep an eye on our social media channels for conversations with Dr. Phoebe Wamalwa, Carolina Tonelotto, Andrea Klein, Professor Christian Hendriksz, Professor François Maillot, Ida Vanessa Doederlein Schwartz, and Dr. Yann Nguyen as we continue to share key insights, reflections, and experiences from IWGGD 2026.

Thank You to Aimee-Kate Bosch

Aimee-Kate Bosch, Chairperson

The International Gaucher Alliance would like to extend its sincere thanks to Aimee-Kate Bosch as she steps down from her role as Chair of the IGA Board. Aimee-Kate first became involved with the IGA through Go with Gaucher and later joined the Board during the Members' Meeting in Riga in 2018. From the beginning, her passion for the Gaucher community, particularly for ensuring that no young person or family feels isolated or unsupported, has been clear in everything she has done.

During her time on the Board, and particularly during her period as Chair, Aimee-Kate brought warmth and dedication to the role. Her passion for the Gaucher community and her genuine care for patients and families were evident throughout her work with the IGA, and she approached her role with kindness, professionalism, and a strong belief in the importance of connection and collaboration across the international community.

Alongside her governance role, Aimee-Kate has provided invaluable one-to-one support to members of the neuronopathic Gaucher disease community through her professional expertise as a speech and language therapist. Many families have benefited greatly from her guidance, understanding, and willingness to help them navigate the challenges associated with accessing appropriate support and care.

Aimee-Kate also played an important role in the development of International Gaucher Day activities over several years, helping strengthen social media engagement, awareness messaging, and community participation. In particular, she worked closely with the late Harry Albright, the IGA's Communications Officer, whose creativity, dedication, and friendship meant so much to the organisation and wider community.

While stepping down as Chair, we are very pleased that Aimee-Kate will remain involved within the IGA community, including continuing her support to families and remaining engaged as the representative of the South African Gaucher patient organisation.

On behalf of the Board, staff team, volunteers, and wider international Gaucher community, we would like to thank Aimee-Kate for her commitment, leadership, compassion, and continued support of the IGA and the global Gaucher community.

With sincere thanks,

Tanya Collin-Histed
Chief Executive Officer
International Gaucher Alliance

On behalf of the IGA Board

MOVING FORWARD TOGETHER

Together, through collaboration, advocacy, research, and community, we continue working toward a future where every person living with Gaucher disease has access to support, care, and opportunity, no matter where they live.Thank you for being part of the International Gaucher Alliance community.

STAY CONNECTED

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