IGA INTERNATIONAL GAUCHER ALLIANCE
Driven by passion for patients

International advocacy for Gaucher patients since 1994

IGA Impact Report 2025

www.gaucheralliance.org

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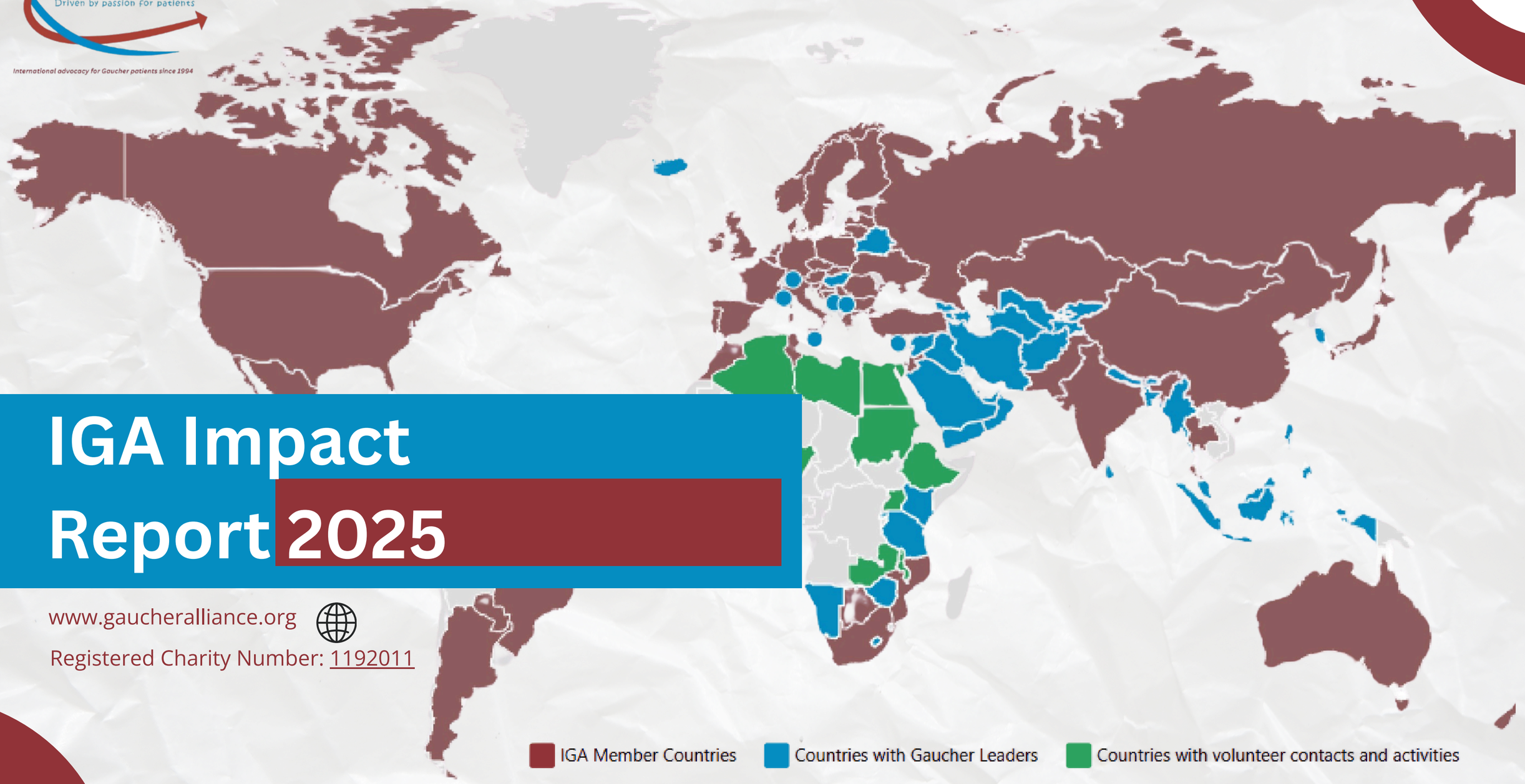


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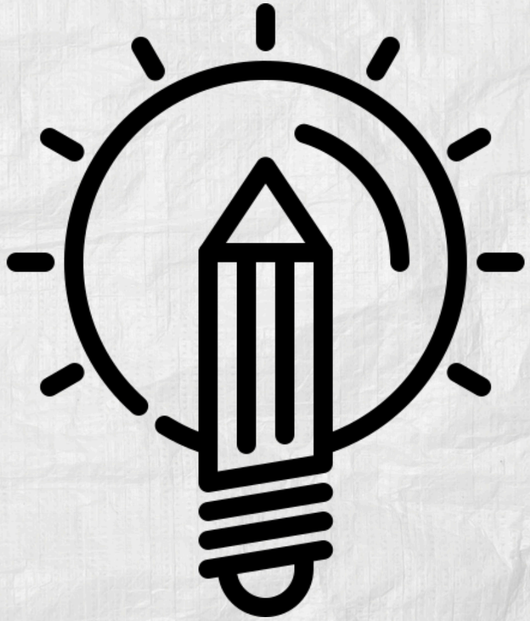
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A message from our CEO and Chair

Standing alongside patients in 2025
Across the world, too many individuals still face long and difficult journeys to diagnosis. Our global survey shows that more than half of respondents waited over a year for a diagnosis—representing prolonged uncertainty, avoidable complications, and missed opportunities for early intervention. Inequity in access to treatment also persists, with patients often lacking sustainable access to therapy, and even those on treatment facing insecurity due to fragile healthcare systems, financial pressures, or supply disruptions.

The wider global context
We are also working within a world shaped by broader challenges. Political instability, conflict, and humanitarian crises continue to affect continuity of care for rare disease patients. While not the focus of our work, these realities directly impact the communities we serve and cannot be ignored.

The power of people in 2025
Despite these challenges, what stands out most strongly in 2025 is the power of people. In 2025, we continued to stand alongside Gaucher patients and families in an increasingly complex and unequal global landscape. While meaningful progress has been made, this report is a reminder that significant challenges remain.

The power of volunteers
Our global network of volunteers is the foundation of everything we do. From supporting patients and caregivers to leading local initiatives, contributing to international working groups, and shaping awareness campaigns, their dedication enables the IGA to reach communities that would otherwise remain unsupported. Their lived experience ensures the patient voice is embedded in research, policy, and care.



Expert collaboration
We are deeply grateful to the global network of Gaucher experts who generously contribute their time and expertise. Through steering groups, research initiatives, educational platforms, and clinical guidance, they strengthen our programmes and help ensure patients and caregivers receive informed support. Their voluntary collaboration is a cornerstone of the progress we continue to make.

Partnerships driving progress
We are equally grateful to our partners and stakeholders who continue to believe in our mission. Collaborations such as our partnership with A Rare Cause have helped advance progress in Africa, supporting diagnostic pathways, strengthening clinical networks, and building sustainable care models. While challenges remain, these partnerships show what is possible through shared purpose and trust.

Strengthening global collaboration
A defining strength of our work is the collaboration between the IGA and the International Working Group on Gaucher Disease (IWGGD). Together, we are advancing research, shaping clinical guidance, and ensuring patient perspectives are embedded throughout—from clinical trials to long-term care strategies. This partnership strengthens confidence that progress is coordinated, transparent, and patient-centred.

Looking ahead
As this report shows, the IGA continues to expand its global reach, strengthen scientific engagement, and support patients in increasingly complex environments. Yet our mission remains unchanged: to improve the lives of all those affected by Gaucher disease, wherever they are in the world. We move forward with determination—guided by our community, strengthened by collaboration, and committed to a future where no patient is left without diagnosis, care, or treatment.

Tanya Collin-Histed, CEO
Aimee-Kate Bosch , Chairperson

In 2025, the IGA supported cases across more than 18 countries, including Afghanistan, Bangladesh, Sri Lanka, Sudan, Gaza, Morocco, Uzbekistan, India, Jordan, Rwanda, Zimbabwe, Zambia and Ethiopia.

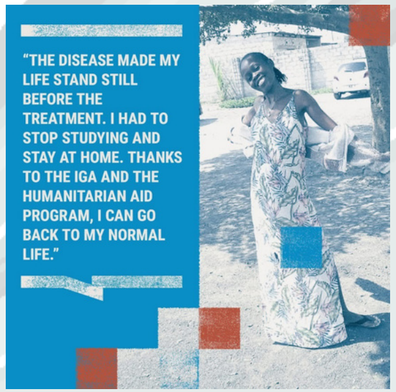
The IGA works closely in partnership with Sanofi through their ICAP programme, and with Takeda through its Charitable Access Programme (of which the IGA is a member of the Medical Expert Committee). In 2025, 24 patients were approved through Takeda's programme. Pfizer supported one new Gaucher patient in India and continues to provide treatment globally through PfizerCares. Sanofi's charitable access programme continues to support many patients globally during the year, including cases referred by the IGA alongside referrals received through wider clinical and humanitarian pathways.
In Uzbekistan, up to 10 patients were identified as potentially without treatment.
In Gaza, coordination continued for 3 siblings seeking treatment transfer; tragically, one sibling passed away due to complications.

The IGA is operating in an increasingly complex global environment. In some countries, no reimbursement systems exist for rare disease medicines, leaving families entirely dependent on charitable access. In others, reimbursement systems technically exist, yet financial instability, political disruption, supply interruptions, or administrative delays create treatment insecurity. Increasingly, patients and caregivers are turning to the IGA when national systems break down.

This trend is growing. The IGA is seeing more cases where reimbursement disruption, insurance gaps, conflict, or regulatory barriers threaten continuity of care — even in countries with established healthcare systems.

Through partnership with Sanofi, Takeda, PfizerCares, Direct Relief, Project HOPE, clinicians, and local advocates, the IGA continues to navigate humanitarian, regulatory, and political barriers to protect patient access.

STANDING WITH PATIENTS IN A COMPLEX GLOBAL ACCESS ENVIRONMENT









DRIVING INNOVATION:
ENSURING THE PATIENT VOICE SHAPES THE GAUCHER PIPELINE







Throughout 2025, the IGA engaged with more than 20 biotechnology and pharmaceutical companies across the Gaucher development landscape — from early discovery programmes to Phase III clinical trials.

At the 2025 WORLD meeting, the IGA contributed to discussions on trial design, endpoint development, recruitment feasibility, and meaningful patient engagement. The IGA facilitated introductions between industry teams and five international neuronopathic Gaucher experts, strengthening advisory development and clinical alignment.

The IGA also provided strategic support to researchers applying for competitive funding by identifying clearly defined unmet needs and linking these directly to research proposals. In 2025, the IGA supported three major funding applications submitted to the Medical Research Council (MRC), The European Rare Diseases Research Alliance (ERDERA), and C-Path, ensuring patient priorities were embedded within scientific proposals.

The IGA participated in a rare disease investment roundtable hosted by Forbion, engaging with venture capital representatives to highlight the importance of sustained investment in rare disease innovation. The IGA also supported a parent listening session focused on the future development of clinical trials for GD2 patients, ensuring caregiver insight informs trial design and feasibility.
Through active communication, the IGA continues to raise awareness of clinical trials and research opportunities across the global Gaucher community, promoting informed participation and inclusive recruitment.

STRENGTHENING GARDIAN:
VALIDATING TOOLS FOR FUTURE CLINICAL TRIALS

GARDIAN
THE NEURONOPATHIC GAUCHER REGISTRY
OWNED AND GOVERNED BY THE GLOBAL PATIENT COMMUNITY

GARDIAN IS THE FIRST GLOBAL REGISTRY TO FOCUS SPECIFICALLY ON GAUCHER DISEASE TYPES 2 & 3 (NGD).
WWW.GARDIANREGISTRY.ORG

The GARDIAN registry (Gaucher Registry for the Development, Innovation and Analysis of Neuronopathic Disease) remains the only global registry dedicated to neuronopathic Gaucher disease.

In 2025, collaboration continued with Aparito to maintain registry infrastructure and analyse early implementation data. A manuscript outlining the methodology and development of GARDIAN is in preparation, with publication anticipated in early 2026.
A key priority has been formal validation of the PRO and ObsRO tools. Work has focused on assessing sensitivity, responsiveness, and measurement robustness to ensure they meaningfully capture patient and caregiver experience. This includes examining their practical utility and suitability as potential endpoints in future interventional trials.



ADVANCING GD3 RESEARCH: FROM CONSENSUS TO CLINICAL READINESS

In partnership with the IWGGD and international experts, 2025 marked continued progress toward harmonising GD3 research globally.

Standardising GD3 Clinical Trial Design: The Global Delphi Initiative

Improving research for neuronopathic Gaucher disease (GD3) is essential to ensuring better treatments and outcomes for our community. However, differences in how clinical trials are designed have made it difficult to compare results and move research forward effectively.

The GD3 Global Delphi Consensus is advancing the standardisation of clinical trial design, addressing long-standing challenges in endpoints, cohort stratification, and methodology.

Through a structured global Delphi process, the IGA is working alongside international clinicians, researchers, and partners to build consensus on more meaningful and aligned approaches to GD3 research. The initiative is guided by a steering group comprising the IGA, the IWGGD, and representatives from regulators and payers.
This work is strengthening collaboration across the field and laying the groundwork for more robust, comparable studies—ensuring that future clinical trials better reflect the needs and realities of the global GD3 community, in line with the IGA's commitment to driving equitable and impactful research.

The GD3 Community Advisory Board (CAB)

Established in 2025 and chaired by IGA Board Member Andrė Balžekienė, the CAB brings together GD3 patients and caregivers from the US, UK, Czech Republic, Sweden, Lithuania, Germany, and Pakistan, providing a platform for connection, shared learning, and advocacy.



The Advisory Board provides a forum for GD3 patients and caregivers to identify challenges, unmet needs, and raise awareness among the community, policymakers, researchers, and industry. Despite decades of enzyme replacement therapy, neurological symptoms remain untreated a critical
ongoing need.

In 2025, the Board met twice: the first meeting produced a paper on GD3 unmet needs, scheduled for publication in 2026; the second addressed educational challenges, forming the basis of a resource for educational policymakers.

Recognising and Managing Gaucher Disease Type 3

Africa GD3 Webinar Series
Improving awareness and understanding of GD3 in under-resourced regions is critical to achieving earlier diagnosis and better care. In response, the IGA launched a four-part educational webinar series focused on recognising and managing Gaucher disease type 3 in Africa.

Delivered in partnership with the IWGGD, A Rare Cause, and African Gaucher leaders, the programme brings together regional and international expertise to address key challenges in diagnosis, disease progression, and clinical management.

Two webinars were successfully delivered in 2025, covering diagnosis, management, and disease course. Alongside this, a GD3 checklist was developed and distributed to support clinicians in recognising and managing the condition, with plans to share this resource globally.









The remaining sessions are scheduled for 2026, with plans to expand the series into a global roadshow in partnership with local patient groups and treating doctors, extending its reach and impact across other regions.
Click here to watch these on our youtube channel

Voice Biomarker Feasibility Study
The IGA is advancing innovative approaches to monitoring neuronopathic Gaucher disease (GD3) through a new Voice Biomarker Feasibility Study, developed in collaboration with the IWGGD, SymetryML, and Canary Speech.

This project explores the potential of voice analysis as a non-invasive, accessible tool for tracking neurological progression and as a novel biomarker for use in clinical trials—addressing a critical gap in how GD3 is currently monitored.

Building on the IGA's broader work to strengthen GD3 research infrastructure, including the Global Delphi Initiative on clinical trial design, this study represents an important step towards more sensitive, patient-friendly, and scalable outcome measures.

A pilot study is planned for early 2026, with the potential to enhance trial readiness, improve disease monitoring, and accelerate progress towards effective therapies for the global GD3 community.

EXPLORING DELAYED DIAGNOSIS IN GAUCHER DISEASE

The diagnosis of Gaucher Disease is often a challenge, as described by many patients. To better understand those challenges the International Gaucher Alliance (IGA) conducted a survey between November 2024 and February 2025 among individuals living with Gaucher disease, including patients, families, and caregivers, primarily in English language, with a Spanish translation for relevant IGA members, the survey was completed by 142 respondents from 40 different countries.

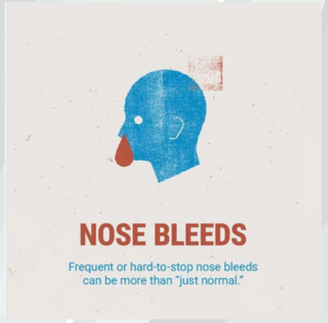
The survey revealed that diagnostic delays remain prevalent, with 58% of respondents waiting more than a year for diagnosis. While key contributors to delays included low awareness among physicians and inadequate communication between medical specialties. Common "red flag" symptoms identified in guidelines were often linked to eventual diagnosis, highlighting the need to educate healthcare professionals, especially those likely to encounter patients but not consider Gaucher disease.

Respondents suggested that increased awareness among healthcare professionals, expanded access to newborn screening, and greater availability of genetic and enzymatic testing could significantly accelerate the process.

The main conclusions where that continuing to engage in education and awareness activities for specialists that commonly diagnose Gaucher, targeted awareness campaigns for secondary care clinicians that do not commonly diagnose Gaucher but are regularly seen in the journey to diagnosis could reduce diagnostic delays. Country-specific surveys to understand local diagnostic experiences could shape tailored interventions.













Download the Publication

142 respondents

40 different countries

58% of respondents waiting more than a year for diagnosis

Africa

GLOBAL
GAUCHER CONNECT PROGRAMME

PODCAST: LIVING WITH GAUCHER DISEASE IN AFRICA

In 2025 we dedicated an episode of our podcast to Africa. Prof. Christian Hendriksz talked about the challenges and progress made in Africa regarding diagnosis, management and treatment, together with Emma (GD patient from Kenya), Laurence (GD parent from Rwanda) and Roselyn Kanja Odero (Gaucher Leader at IGA and a patient advocate from Kenya).

THE POWER OF CONNECTION WITHIN THE GAUCHER DISEASE COMMUNITY!

Roselyn Kanja-Odero's inspiring initiative to establish a WhatsApp group for patients and families in Kenya, Zambia, Rwanda, Mozambique, Zimbabwe and Tanzania – with more countries on the horizon – promises to be an invaluable source of mutual support and shared understanding as they navigate the unique challenges of Gaucher disease.
Statement Roselyn Kanja-Odero:

As an IGA Gaucher Leader in Africa, helping form a support group across countries has been incredibly meaningful. It's allowed patients and families to connect, share experiences, and find solutions beyond borders. More than anything, it's created a sense of community and hope for people who often feel alone in their journey. I hope it will grow into a platform for sharing resources, engaging with experts, and contributing to research.





Similarly, Dr. Magy Abdel Wahab's creation of a dedicated WhatsApp group for Arabic-speaking individuals in Egypt, Gaza, and Sudan is a wonderful step towards fostering connection within that linguistic community.

The fact that these vital support networks emerged from a recommendation at the Kenya Gaucher meeting in December 2024 speaks volumes about the proactive and collaborative spirit within the IGA Gaucher community. By providing these platforms, Roselyn and Dr. Magy are creating a much-needed opportunity for families to come together, share their experiences, and uplift one another on their Gaucher journey. This collaborative approach truly embodies the strength and resilience of the human spirit in the face of adversity.

PUBLICATIONS

We published a poster for Ethiopia and Sri Lanka (Global Collaborative Effort for Gaucher Disease: A step towards improving healthcare) for the WORLD symposium 2025 and Tanya presented it and gave an interview.

THE AFRICA ROADMAP: A COLLABORATIVE PATHWAY TO SUSTAINABLE CARE AND DIAGNOSIS

IGARD
Building the future, driven by patients

The Africa Roadmap is a strategic, joint initiative between the International Gaucher Alliance (IGA) and A Rare Cause (ARC), designed to overcome the systemic barriers to diagnosis and care across the African continent. Utilising an innovative hub-and-spoke model, the programme connects a central laboratory in South Africa with eight regional centres of excellence to provide vital access to diagnostics, clinical support, and professional education.

This partnership between the IGA and ARC is fundamental to the project's success, combining global advocacy expertise with specialised technical resources to empower local healthcare professionals. By fostering sustainable clinical networks and decentralising expert knowledge, the Africa Roadmap ensures that the path to diagnosis and long-term management is both accessible and tailored to the unique needs of African Gaucher communities.

Africa

GLOBAL
GAUCHER CONNECT PROGRAMME

"GAUCHER DISEASE IN LIBYA: DIAGNOSIS, MANAGEMENT, AND THE AFRICA ROADMAP"

On May 5, 2025, the International Gaucher Alliance (IGA) hosted a targeted educational webinar, "Gaucher Disease in Libya: Diagnosis, Management, and the Africa Roadmap," to address the specific needs of the Libyan medical community.



This session featured expert presentations from Dr Magy Abdel Wahab on the symptoms and management of Gaucher types 1 and 3, and Dr Elne Conradie, who introduced the Africa Roadmap for testing lysosomal storage disorders. A highlight of the webinar was the contribution from Dr Fatma Elamari Ali, Dr. Maryem Ismail, and Dr. Nadya Omran, local physicians with extensive experience in internal medicine, who provided a critical perspective on managing adult Gaucher patients within the Libyan healthcare system.

By combining global expertise with local insights, the webinar served as a vital platform for improving collaboration for Gaucher Disease in Libya.

BRIDGING KNOWLEDGE AND HOPE: A LANDMARK FOR GAUCHER CARE IN AFRICA

The Africa Gaucher Family Conference 2025, held from 28th to 30th of November in Nairobi, marked a historic turning point for rare disease advocacy across the continent. This meeting featured a hybrid CME Dinner on Gaucher Disease Management for healthcare professionals on 28 November, followed by a two-day hybrid Gaucher Family Conference for patients, caregivers, and families from 29 to 30 November.

The conference brought together a diverse group of participants from eight different countries, with families from Kenya, Tanzania, Rwanda, Zambia, Zimbabwe, and Mozambique attending in person to share their journeys. By uniting healthcare professionals, patients, and families, the gathering transformed scientific dialogue into a powerful "environment of learning, healing, and shared purpose".

The impact of this hybrid event was best captured by the heartfelt feedback from our participants, one of whom remarked they had “**never saw an environment of hope like this,**” adding, **“wish this love spreads forever in Africa and all over the world”.**

This spirit of solidarity reinforces our core mission: we are truly stronger together. As we move forward, the connections forged in Nairobi will serve as the foundation for advocating for improved diagnosis, better treatment access, and a brighter future for every Gaucher family in Africa.



GLOBAL
GAUCHER CONNECT PROGRAMME

South-East Mediterranean

'STRENGTHENING THE GAUCHER PATIENTS' VOICE IN LEBANON'

On 19th August 2025, the International Gaucher Alliance (IGA) hosted its first-ever patient and family meeting in Beirut, Lebanon, themed 'Strengthening the Gaucher Patients' Voice in Lebanon'. This meeting was realised with the leadership of our Gaucher Leader, Carine Alsokhn, and Lebanese volunteer, Lara Haidar.

Held at the Gefinor Rotana Hotel, this landmark event brought together a diverse community of patients and families from across the country—including Beirut, Jbeil, Bekaa, and Baalback—as well as Syrian families and clinicians. The meeting served as a critical platform for addressing the unique systemic barriers in the region, such as diagnostic delays and treatment coverage gaps.

Through professional insights from Dr. Hicham Mansour, emotional testimonies from youth, and collaborative working groups, the event successfully fostered a network of mutual support and laid the strategic foundation for establishing a formal national patient organisation.

This meeting represents a turning point for the Lebanese Gaucher community, transforming individual resilience into a collective movement for better care and advocacy.



South Asia

STRATEGIC PARTNERSHIPS: BUILDING SUSTAINABILITY IN SRI LANKA

In 2025, a collaborative effort between Symmetry, A Rare Cause (ARC), Metabolic Support UK, and the International Working Group on Gaucher Disease (IWGGD) significantly advanced the support for Gaucher disease in Sri Lanka, and also for other rare diseases.

Because of this collaboration Sri Lanka got the first registry for rare diseases. In the first 6 months, 50 patients have already been registered with different rare conditions. In 2026 we will have data to analyse and use for further advocacy in improving access to diagnosis, care and treatment in Sri Lanka.

The Rare Disease Forum of the Sri Lanka College of Paediatricians organised the 9th Annual Rare Disease Day Symposium on 7th March 2025 at the New Auditorium of the Lady Ridgeway Hospital, Colombo, Sri Lanka where 150 people attended gathered on the topic: Rare Diseases in Resource-Limited Settings: Challenges and Opportunities for Improved Care.
Our support and collaboration continues in 2026.





GLOBAL
GAUCHER CONNECT PROGRAMME

Central Latin America

PODCAST "LIVING WITH GAUCHER DISEASE IN CENTRAL LATIN AMERICA"

The podcast "Living with Gaucher Disease in Central Latin America" was realised in Spanish language thanks to Maria Alejandra Tornero, a Gaucher Leader in the IGA. Dr. Gustavo Kantor began the meeting by presenting the objective of discussing the management of Gaucher disease in different countries.

Patient and caregivers shared their stories about diagnosis and treatment: Ricardo (GD patient from Argentina), Rigoberto (GD patient from Guatemala), Marta (mother of Candy-GD patient from Guatemala), Augustin (young GD patient from Argentina), Alexandra (GD patient from Peru), Elsa (mother of Jacqueline – GD patient from Guatemala), Emilse (GD patient from Argentina), Veronica (mother of Ezequel – GD patient from Argentina).

Every story is heartwarming, and it shows why is it important to have patient organisations who fight for better quality of life of families with Gaucher disease

PRIORITISING EMOTIONAL WELLBEING: COLLABORATIVE MENTAL HEALTH ADVOCACY IN LATIN AMERICA

In 2025, the International Gaucher Alliance (IGA) supported the realisation of a vital regional initiative focused on the psychological and emotional dimensions of living with a rare disease.

In a powerful collaboration with Alianza Lisosomal Argentina (A.L.A.) and ALAPA, a specialist virtual session titled "Gaucher Disease: Beyond the Symptoms" was delivered to address the "subjectivity of the patient." Led by psychologist Carolina Oliveto, the session provided a dedicated forum for the Latin American community to explore the profound impacts of diagnostic trauma, chronic pain, and the complexities of navigating adulthood and family planning with Gaucher disease.

By facilitating this dialogue, the IGA helped empower local advocates to bridge the communication gap between clinical treatment and mental health, ensuring that emotional resilience is recognised as a cornerstone of comprehensive patient care across the region.

Thanks to Maria Alejandra Tornero, a Gaucher Leader in the IGA we are realising activities in Spanish language to support the Spanish speaking Gaucher community.

LOCAL AND REGIONAL ACTIVITIES

In 2025, IGA has supported local and regional activities and initiatives in public health in Latin America.

IGA is actively pursuing treatment for those patients without coverage in Ecuador, Venezuela and Honduras, and working with regional organizations to secure proper and timely diagnosis for patients, especially in rural fields where national health systems are scarce.

IGA had the opportunity to present the case of patients with Gaucher and other rare diseases to the Honourable Members of the Central America Parliament, explaining the need for regional actions to improve conditions for the community. The IGA Chairperson attended remotely and explained the work IGA does for Gaucher patients around the world. Jackeline Emilse Peña de Leon, regional Gaucher Leader, addressed the assembly, as well as IGA Secretary Patricia Lucki and Luis Penados, member of the ANGEL board.

As part of activities delivered with partners in Central America, seminars were conducted and activities organised with patients and caregivers, particularly focusing on the wellbeing of the community.

At a Latin American level, IGA is part of the organisation of the 3rd CEPCAL Congress in Antigua Guatemala, where researchers, medical practitioners and patients are expected to gather and share knowledge on diagnosis, care and technology.

VOLUNTEER PROGRAMME

Empowering the Global Community through volunteering

The International Gaucher Alliance (IGA) facilitates essential professional and patient-led support by coordinating a diverse network of volunteers across several key programmes. A cornerstone of this effort is the Global Gaucher Connect Programme (GGCP), where dedicated Gaucher Leaders (GLs) and volunteers provide local expertise, facilitate regular communication between patients and clinical experts, and develop vital resources—such as translated materials in different languages.

Furthermore, the IGA's collaboration with the International Working Group on Gaucher Disease (IWGGD) has led to the participation of patients and caregivers in the IWGGD working groups on different aspects of Gaucher Disease. Notable participation of volunteers in the working groups include the development of self-management guidelines and a comprehensive patient/caregiver leaflet – Living Well with Gaucher, as well as critical work on home treatment recommendations. These volunteers, ensure that the patient voice is heard and translated into practical, accessible tools for the international Gaucher community.

Furthermore, volunteers remain the heartbeat of International Gaucher Day, contributing the personal stories that drive our global advocacy. By providing the platform and administrative framework, the IGA ensures that the patient voice is not only heard but translated into practical, accessible tools that improve daily life for the international Gaucher community.







INTERNATIONAL GAUCHER DAY (IGD) 2025



In 2025, the International Gaucher Alliance (IGA) delivered the global campaign

See the Signs, Shorten the Journey

The campaign focused on raising awareness of early symptoms ("Red Flags") and reducing diagnostic delay globally. The messaging aligned directly with the findings from the IGA's global delayed diagnosis survey, reinforcing the importance of recognising warning signs and accelerating referral pathways.

The campaign included:

- A coordinated global social media campaign with a structured monthly content calendar
- Engagement and participation from international member organisations
- Amplification of patient stories to humanise the diagnostic journey
- Educational materials aligned with Red Flags messaging
- Integration across newsletters, website, and podcast platforms
- Board-level involvement in campaign planning and strategic messaging

Through unified global participation, IGD 2025 strengthened awareness, reinforced the importance of early diagnosis, and positioned the IGA as a central coordinating voice for international advocacy.

Volunteers remained the heartbeat of International Gaucher Day, contributing the personal stories that drive global advocacy. By providing the platform and administrative framework, the IGA ensured that the patient voice was not only heard but translated into practical, accessible awareness tools.

International Gaucher Day statistics

Impressions:	Reach:	Frequency:
2.46 million	**1.82 million**	**1.35**

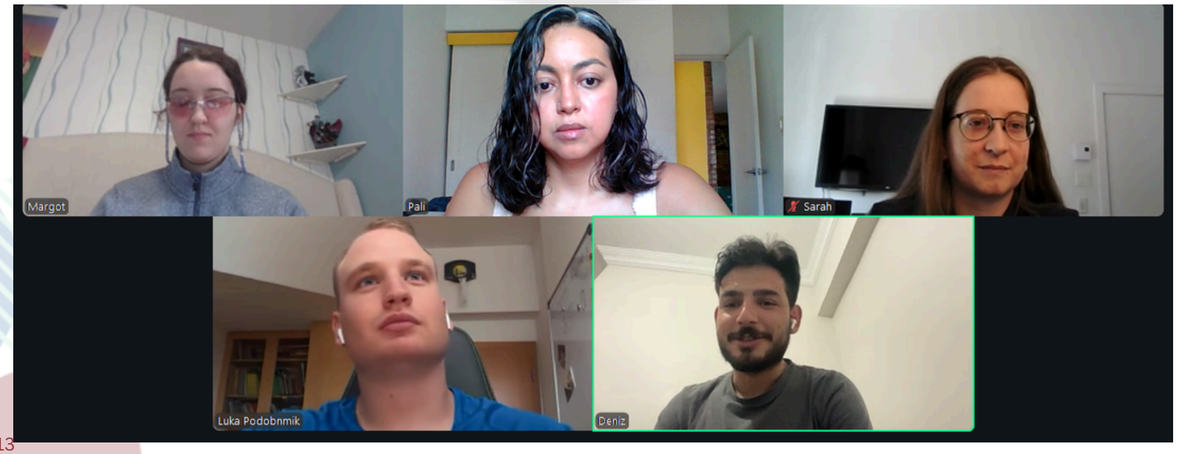
IGA YOUTH COMMUNITY

Since late 2023, following the Go with Gaucher meeting, the IGA has built a community of young patients with Gaucher disease who have shown a strong commitment to activism and mutual support. Participants from the Istanbul meeting have been joined by other young people who share the goal of creating a space for meeting and learning.

The IGA has worked to strengthen the community, recognising the regional and cultural differences that living with Gaucher disease entails. As a result of this effort, a book aimed at children and youth with Gaucher disease is currently under development, exploring the diverse perspectives and experiences of living with this condition. The book will address not only medical aspects, but also the cultural, institutional, and social impact of diagnosis and treatment in different contexts.

Through this initiative, the IGA seeks to promote understanding and support for patients with different types of Gaucher disease, highlighting the importance of comprehensive and equitable care. The IGA Youth Community remains committed to this project and to the wellbeing of the global youth community.

In 2025, the Youth Community also strengthened its engagement in Red Flags awareness activities, contributing to broader efforts to reduce diagnostic delay and improve early recognition of Gaucher disease.



COMMUNICATION, VISIBILITY AND ORGANISATIONAL RESILIENCE

In 2025, the IGA strengthened its communications infrastructure through the implementation of a structured communications plan and a coordinated working group approach.
During the year, the IGA delivered:

- **A new quarterly newsletter**
- **Weekly news briefs**
- **Continued production of the Gaucher Voices Podcast**
- **Dedicated regional podcast episodes in Africa and Central Latin America**
- **Website technical and content review**
- **Engagement with website redevelopment agencies**
- **Coordinated digital campaigns aligned with strategic priorities**

The Communications Working Group contributed to the development of the IGA's renewed communications strategy, including brainstorming and ideation across International Gaucher Day, newsletters, webinars, podcasts, and social media.

A comprehensive technical and content review of the existing IGA website was conducted, identifying areas for improvement and recommending next steps. Discussions were initiated with website development agencies regarding redevelopment.

This structured communications approach strengthened global visibility, reinforced leadership within the rare disease community, and ensured alignment between advocacy messaging, research initiatives, and patient engagement.

GLOBAL REPRESENTATION AND SCIENTIFIC LEADERSHIP



CEO Tanya Collin-Histed with representatives from the Sri Lankan clinical team.



CEO Tanya Collin-Histed with Representatives from the Japanese Gaucher patient group. Meeting was ICIEM, toto, Japan, 2025

International Working Group on Gaucher Disease (IWGGD) Representation

Throughout 2025, the International Gaucher Alliance (IGA) maintained active participation within the International Working Group on Gaucher Disease (IWGGD), contributing across multiple research and guideline initiatives.

IGA representation included involvement in the following IWGGD Working Groups:

- Epilepsy in GD3
- Ambroxol
- As part of the IWGGD Clinical Guidelines for GD1 – Supportive Care sub-group, IGA representatives actively contributed to:
- Home Therapy Working Group
- Self-Management Working Group
- Transition Working Group
- Coordination of Care Working Group

A scientific paper arising from the Home Therapy Working Group was published in the Journal of Clinical Medicine:

Kieć-Wilk B, Guijt P, Dan M, Abdelwahab M, Revel-Vilk S, Serratrice C on behalf of the International Gaucher Alliance (IGA) and the International Working Group on Gaucher Disease (IWGGD).

Home Enzyme Replacement Therapy in Gaucher Disease: A Review. Journal of Clinical Medicine. 2025; 14(3):842.
Click here

The guidelines on Transition and Coordination of Care have been published on the IWGGD website:
Click here

Further information on IWGGD working groups, including IGA representatives, is available at:
Click here

Other representation and leadership:

- Takeda Gaucher Outcome Study (GOS) Steering Committee
- Sanofi Registries Patient Council
- EURORDIS Screen4Care Patient Advisory Board
- European Haematology Association Gaucher guidelines steering group
- HTAi Rare Disease Interest Group Steering Committee

The IGA attended the 2025 ICIEM meeting in Japan, strengthening Asia-Pacific engagement.

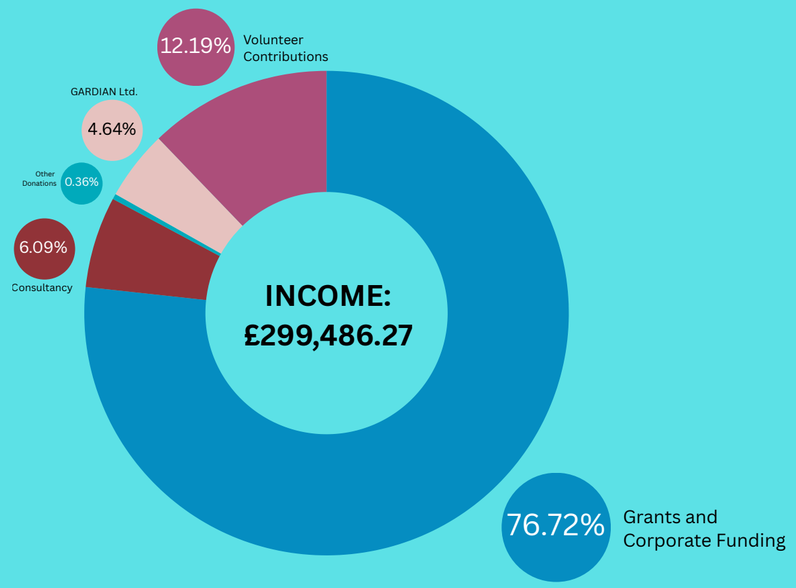
The IGA also participated as a speaker at an Early Access conference in London, highlighting the value of humanitarian aid programmes in enabling early treatment access in complex global settings.

Additionally, the IGA was invited by Rare Diseases International to present on rare disease registries, reinforcing leadership in patient-centred registry development and global data strategy.

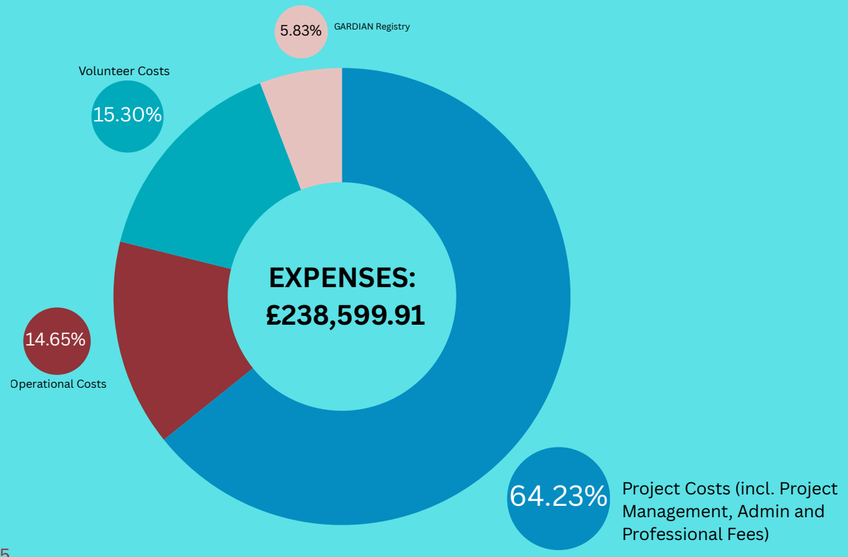
Through these roles, the IGA ensured that the Gaucher patient voice remained embedded within scientific discussions, clinical trial design, health technology assessment dialogue, and international policy frameworks.

FINANCIAL HIGHLIGHTS

INCOME:



EXPENSES:



Our board of Directors, CEO, volunteers and doctors have donated over 1785 hours of their voluntary time to our activities. **THIS IS EQUAL TO £36,499,38.** We are extremely grateful for this dedicated support.

A HUGE THANK YOU TO OUR SUPPORTERS...

... to our supporters and dedicated volunteers for believing in us and contributing to our extensive programme of activities, projects, and events throughout the year. Without your generous support and commitment, we could not work towards a better world for Gaucher patients!

sanofi · Takeda · azafaros · Ace L (Where Science and Medicine Meet) · Lilly · SPUR THERAPEUTICS · Mehta Pharmaceutical Industries · GCAN (Gout Hyperuricemia and Crystal-Associated Disease Network) · ISU ABXIS

LOOKING AHEAD TO 2026

EXPANDING ACCESS TO TREATMENT
Driving global efforts to ensure no patient is left without care.
Global Gaucher Connect Programme
Building evidence, strengthening national advocacy, and developing regional roadmaps to improve diagnosis and unlock treatment access worldwide—particularly across Africa (Algeria, Sudan, Ethiopia), South Asia (Sri Lanka, Nepal), Latin America (Chile and Central America), and Asia-Pacific (Vietnam, Indonesia, Malaysia).
Strengthening Volunteer Power
Growing and equipping a global network of volunteers to lead change locally, support patients and caregivers, and sustain impact in low-resource settings

IMPROVING DIAGNOSIS & MEDICAL EDUCATION
Accelerating earlier diagnosis and better care worldwide.
Global Medical Education Programme
Delivering CPD-accredited training, case-based learning across GD1, GD2 and GD3, and region-specific education to improve recognition and management of Gaucher disease.
GD3 Global Education Series
Expanding multilingual, multi-timezone webinars to improve understanding, diagnosis, and management of neuronopathic Gaucher disease globally.
Living Well Programme
Providing practical tools on pain, fatigue, and mental health, alongside peer-support initiatives in underserved communities.
Gaucher Voices Podcast
Amplifying global perspectives on advocacy, lived experience, and the future of the Gaucher community.

ADVANCING RESEARCH & INNOVATION
Ensuring patient voices shape the future of care.
GARDIAN & Community Advisory Boards
Expanding global patient advisory groups to inform research priorities, clinical trial design, and patient-centred care.
Voice Biomarker Innovation
Advancing digital voice biomarkers to monitor neurological progression in GD3 and support future clinical trials.
GD3 Global Delphi Initiative
Driving international consensus on clinical trial design to accelerate the development of effective treatments

STRENGTHENING GLOBAL COLLABORATION
Working together to deliver greater impact.
Collaboration with IWGGD
Advancing clinical guidelines, research, and patient-centred care through strong global partnership and shared expertise.

GLOBAL COMMUNITY & ADVOCACY
Connecting, empowering, and amplifying voices worldwide.
Equity in Action – International Gaucher Day 2026
Mobilising the global community to highlight disparities, amplify patient voices, and drive advocacy for equitable access.
Connecting a Global Community
Delivering webinars, digital resources, and global communications to ensure patients, families, and professionals remain informed and supported.

OUR FOCUS REMAINS CLEAR
A future where every person living with Gaucher disease is diagnosed earlier, has access to treatment, and is supported to live well—no matter where they are in the world.

Connect With Us

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Madeline Stoodley
Operations Officer



Patricia Lucki
Secretary



Paulina (Pali) Peña Aragón
Director



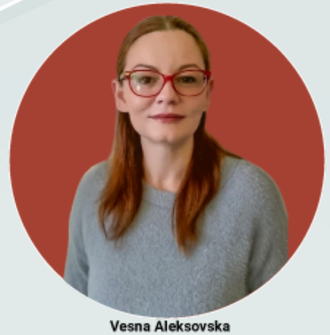
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Suyog Sathe
Director



Tanya Collin-Histed
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