IGA Strategic plan 2024-2029

The IGA's Strategic imperatives seek to achieve a **strong voice** for Gaucher patients and their families through collaboration and partnership.

Strategic Imperative 1:
Improve Gaucher patients' access to optimal diagnosis, treatment, and care.

Enhance the provision of diagnostic services to improve the timeliness of diagnosis of Gaucher disease by:

1. Developing an online resource on the IGA website that identifies diagnostic centres for Gaucher disease globally.
2. Developing in collaboration with other stakeholders a roadmap of diagnostic pathways in Africa and work to support the implementation of a sustainable diagnostic service in Africa appropriate to each region/country.
3. Using the platform of International Gaucher Day (IGD) to raise awareness about Gaucher disease among those health professionals who have an impact on patients and their disease journey.
4. Under the umbrella of the regional manager programme, we will:
    a. increase the opportunities for the education of doctors on the signs and symptoms of Gaucher disease in conjunction with the IWGGD, pharmaceutical company preceptorships and other conferences.
    b. ensure access to diagnostic services through collaboration with laboratories that are keen to provide charitable services for diagnosis.
    c. raise awareness of the work of Medics 4 Rare Disease (M4RD) where appropriate for that country/region.
5. We will promote the International Working Group on Gaucher Disease (IWGGD) guidelines in the regional manager programmes to share best practice and ensure patients get the best care possible.
6. Roll-out an awareness campaign about diagnosis called "Gaucher red flags" to alert clinicians to the signs and symptoms of Gaucher disease in collaboration with:
   - Healthcare professionals including those working in the fields of haematology, metabolic, orthopaedics, medical genetics, primary care and oncology.
   - IWGGD, European Haematology Association (EHA), International Society of Haematology, Society for the Study of Inborn Errors of Metabolism (SSIEM), International Congress of Inborn Errors of Metabolism (ICIEM) and other organisations identified to ensure global coverage.
   - IGA Member organisations and regional managers.

7. Increasing awareness of Gaucher Disease to the European Haematology Association and the International Society of Haematology through the inclusion of Gaucher disease in their programmes and educational syllabus.
8. Developing a position paper on Newborn screening in Gaucher disease and ensuring that the IGA are aware of, or where appropriate are members of, European and International rare disease screening projects i.e., Screen4Care.

Improve the standards of clinical care available to all Gaucher patients globally by:

1. Continuing to identify areas of care and management that need to be developed and promoted and provide support to implement the IWGGD/IGA guidelines.
2. Taking the lead in advocating home therapy by publicity and, together with the IWGGD and involving other professionals, the development of guiding materials.
3. Publishing the IWGGD/IGA Best Practice Switching and Monitoring Consensus and supporting IGA Members to use it as a tool for ensuring the best patient care in their country.
4. Actively promote the work and benefits of IWGGD membership to clinicians in areas not currently represented in the membership with a focus on Asia, Africa, and the Far East.
5. Continuing to collaborate and be a member of Gaucher registry advisory groups including but not limited to International Collaborative Gaucher Group (**ICGG**) **Gaucher** registry; Gaucher Outcome Survey (GOS) and the Rare Disease Patient Registries Council, to promote the value of the registries, ensure that patients/caregivers have access to information published from the registries, that they address the current needs of patients and raise and answer new research questions.
6. Using the data from GaRDIAN to ensure that current clinical care and guidelines address the areas identified by the patients and caregivers that impact their everyday lives to improve patient outcomes.
7. Identifying the unmet research needs and the unanswered research questions of patients/caregivers through our member organisations and the wider Gaucher community and in collaboration with other stakeholders i.e., the IWGGD, EHA, Industry, Rare Disease International (RDI), Eurordis, Metabolic European Reference Networks (MetabERN), SSIEM, ICIEM and regulators to drive forward research into these areas.
8. Updating the current UK Gaucher Associations' nGD supportive care information booklet and making it available in several different languages and formats, based on our community's needs.
9. Using the IGA website to provide information on all licensed medicines for Gaucher disease with a link to a search facility to published papers. Providing information on other Gaucher prescribed treatments that are not licensed but for which published information is available.
10. Collecting data on treating hospitals, clinicians, and diagnostics services from all our regional manager programmes, including a registry of Gaucher patients in each region/country to be clearer about the numbers of patients.

Greatly improve Gaucher Patients' access to optimal treatment and care globally by:

1. Continuously analysing the situation in IGA non-member countries with the help of the regional managers, volunteers, doctors, the pharmaceutical industry, and other key stakeholders, to develop a plan for addressing the key gaps to improve diagnosis, the education of doctors and access to treatment.
2. Working in partnership with the pharmaceutical companies' Charitable Access programmes to highlight the needs of those patients without access to treatment i.e., opening to new countries, access for adults, challenges due to nationality status and conflict zones.
3. Being an active member of the GARDAccess programme 'THINK TANK' and continuously monitoring the development of the GARDaccess programme to identify how Gaucher patients could benefit.
4. Initiating a dialogue with 'Medicines for Europe' and 'International Generic and Biosimilar Medicines Association' in conjunction with the IWGGD to address the current challenges of access to medicines by Gaucher patients across the world.
5. Implement the recommendations in the IGA's position paper on non-comparables (non-quality assured biologics).
6. Positioning the IGA as 'the go to' body to provide patient input into medicines development for Gaucher disease.
7. Continuing to engage at a high level with pharmaceutical company executives to ensure that company trials are focused on the greatest unmet needs.
8. Providing patient input early into all trials that pharmaceutical companies are conducting in the Gaucher disease setting.
9. Together with our member organisations, regional managers, volunteers, and the pharmaceutical industry, developing an online resource on our website that will identify clinicians and treatment centres in non-member countries.
10. Increasing the number of regional manager programmes in countries where there are Gaucher patients but a lack of formal structure including education, disease awareness, and access to treatment. (See strategic imperative 3)
11. Ensuring membership and collaboration with organisations such as RDI, Eurordis, World Health Organisation (WHO), United Nations (UN) to be informed of their activities, to utilise their work and disseminate reports and findings for the benefit of our global community.
12. Developing an online resource on our website on all active and recruiting clinical trials for Gaucher disease.
13. Actively participate in Health Technology Assessment international (HTAi) Rare Disease Interest Group and ISPOR Rare Disease Special Interest Group for the purpose of education regarding evidence collection and drug development to achieve better patient outcomes, successful clinical trial recruitment, and access to new medicines through faster health technology assessments.

Improve the quality-of-life of patients and caregivers/families by:

1. Researching the needs of the older generation (60+ years), to be able to empower, share and strengthen both Gaucher patients and the IGA member organisations to enable our older community to live their best lives.
2. Researching the provision of mental well-being services within our community, and in partnership with IWGGD and Eurordis seek to integrate mental well-being in Gaucher clinics.
3. Undertaking a burden of disease study to identify areas of non-medical need that are important to patients and caregivers.

4. Providing information on Gaucher disease and pregnancy involving GD1 and GD3 patients that have children and explore the best mediums and platforms to disseminate this information i.e., podcast, website.

Strategic Imperative 2:
Influence the Gaucher research agenda so that it's focused on addressing key unmet needs.

Be a critical partner in making sure research and clinical services are relevant for the Gaucher community by:

1. Early engagement through membership of advisory boards and focus groups to educate pharmaceutical companies on what it is like to live with the disease, the physical, mental, and societal impact, from a patient and caregiver perspective.
2. Actively encouraging the pharmaceutical companies to engage with the regulators and HTA early in their drug development programme and to involve the IGA as the patient voice in these discussions.
3. Encouraging and promoting clinical trials sites to be available in countries that do not currently have access to treatment, or where access is challenging whilst ensuring that all ethical challenges are fully considered.

Be a key driver of research in the Gaucher disease setting by:

1. Using the IWGGD biennial meetings as a platform to voice the unmet research needs of the community.
2. Collecting the patient/caregiver voice on the areas of unmet needs that have not yet been addressed, are not understood, or are unknown, linked to strategic imperative 1.
3. Using the IGA's Scientific Advisory Committee (SAC) to guide the IGA's thinking in relation to research and opinions on topics that face the community.

Be a driver in evidence generation of patients' unmet needs through:

1. The GARDIAN registry, on the burden of disease, comorbidities and other conditions and present findings at external meetings and develop publications to inform future research and clinical care and management.
2. The collection of data on all IGA members through Country reports.
3. Utilising the IGA's Biennial members meeting to discuss patients' unmet needs and in conjunction with strategic imperative 1; unmet research needs; unanswered research questions of patients/caregivers and non-clinical needs.

Drive the dissemination of information to educate and empower the community through:

1. The development and implementation of an educational programme on gene therapy using different forms of communication and languages, including but not limited to a website, podcasts, webinars, animations, and leaflets.

2. Hosting regional webinars to raise awareness and provide a platform for community questions on new and emerging therapies, ensuring access through the provision of appropriate languages for that region.
3. Developing an online resource on our website on all active and recruiting clinical trials for Gaucher disease and raising awareness in our monthly newsletters (see strategic imperative 1)
4. To build a pool of Gaucher patient research experts who can represent the patient community on committees for research, drug development and health technology assessment.

Strategic Imperative 3:

Empower and ***support*** *its members and the wider Gaucher community around the world* **to be more effective and sustainable** advocates for the patient community.

Understand the structures that need to be put in place for the IGA to be a truly effective global organisation by:

1. Exploring the development of regional hub networks to address current challenges with language and time zones for access to activities.
2. Reviewing the IGA's Governance structure with a view to strengthening the board of Directors with non-Gaucher patients/caregivers' representatives if necessary to fill specific board needs.
3. Expanding the IGA's volunteer programme to increase our capacity and ability to deliver projects.
4. Translating our main documents into 5 languages following research to identify what would be the most useful languages to our community.
5. Providing simultaneous interpretation for 5 languages on all our webinars. The languages would be chosen depending on the topic and regions.
6. Increasing the number of regional manager programmes in countries where there are Gaucher patients but a lack of formal structure including education, disease awareness, and access to treatment. (See strategic imperative 1)

Strengthen and secure the future voice of Gaucher patients by ensuring succession planning through the Go with Gaucher programme by:

1. Establishing an IGA Youth Council through the Go with Gaucher programme to ensure the voice of young people with Gaucher disease is heard in all areas of work.
2. Identifying at least two persons from the Go with Gaucher programme to become IGA patient experts and provide them with training in the drug development process and evidence generation for health technology assessment.
3. Co-opting a member of the IGA's Youth Council onto the IGA Board of Trustees.
4. Using the Go with Gaucher programme to recruit future regional managers and volunteers for the IGA.

Understand the IGA's future resources needed to service our members and the non-member community by:

1. Securing the resources required to provide a coordinator for the volunteer and regional manager programmes, recognising the need to expand the programmes.
2. Establishing a post dedicated to support our existing members and non-member organisations.
3. Identifying new funding sources outside the traditional annual grants/sponsorship from the Pharmaceutical Industry to support our work.
4. Undertaking an annual board assessment to ensure the board are given the resources required to fulfil their role as directors and develop a plan to address any areas of need through the provision of training and information.

A strong network of Gaucher Patient organisations that is capable of taking action at local and national levels to address problems faced by Gaucher patients and their families through:

1. Providing quarterly webinars on topics chosen by the community for the purpose of education, consultation, and empowerment.
2. Expanding the IGA website to ensure that it contains as many resources as possible and provides opportunities for sharing of best practice.
3. Providing members with the opportunity to attend meetings (e.g., members' biennial meetings, the patient voice at Gaucher meetings).
4. Providing members with opportunities to be involved in the IGA i.e., IGA Board member, Go with Gaucher, Regional Manager, volunteer on a work stream.
5. Providing members with regular opportunities to speak with board members/CEO either face to face or using Zoom.
6. The development of regional hub networks, supporting the development of regional patient meetings.
7. Providing free and accessible resources for IGD for members and non-members to utilise.
8. Establishing a member's code of conduct to strengthen member engagement and ensure a voice in the IGA's work.
9. The completion of annual country reports from our members to assist in the collection of data to better understand country provision and challenges.

Expand our support for hard-to-reach communities in countries currently not IGA members by:

1. Ensuring that the necessary resources are in place to support our existing regional managers.
2. Providing more support from the board members and IGA members to the different regions in the RM program.
3. Ensuring there are incentives for the RMs and their volunteers (such as conference attendance, training, workshops, etc)
4. Utilise the data collected through the regional managers to identify where there are Gaucher patients but limited support and services and bring these countries into our regional manager programme.
5. Publishing the impact of our regional manager programme over the last five years.

Implementing the Communications Plan to support our members and make the IGA and its work more widely known through:

1. Creating and updating educational and promotional materials highlighting the work of the IGA.
2. Strengthening general communications about the IGA and Gaucher disease.

3. Using the website, social media, the e-newsletter, and other electronic methods to strengthen communications to Individual patients and their families, as well as to IGA members and potential members.
4. Expanding the scope of International Gaucher Day (IGD) to make it a fixture on the calendar, including implementing an annual lecture on the theme.
5. Identifying ways to increase our members' commitment to IGA, to ensure their input on themes and the needs of the community that they feel the IGA should be addressing, making them more proactive and strengthening the relationship with the IGA.