Neuronopathic Gaucher Disease (nGD) has a high unmet need, but with an increasing number of pharma companies now developing treatment options, it brings hope to many patients and families. A collaborative disease registry would offer important insight for understanding natural history, validating new outcomes and support clinical trial designs, recruitment, as well as regulatory and reimbursement reviews.

**Technical Requirements**
- 1 Meeting
  - 5 KOLs from 3 countries
  - On-line Delphi
- Input from 12 KOLs in 5 countries
- 132 clinical data fields identified
- Compliance and regulation a mandatory component (e.g. GDPR, GCP, Part 11)

**Pharma Partners**
The IGA would like to thank the four pharmaceutical companies that invested in the development of phase 1 through their participation in the pharma group element of the project and also for their financial support through a unrestricted grant.

**Recommendation for next steps:**
There was high level of enthusiasm to support the initiative by all stakeholders.
- SUSTAINABLE BUSINESS MODEL THROUGH A PARTNERSHIP AGREEMENT
- COLLABORATIVE, GLOBAL FEDERATED REGISTRY (IGAR)
- CLEAR AND TRANSPARENT GOVERNANCE
- SEEK EMA/FDA BUY-IN AND APPROVALS
- STAGGER OVER THREE STAGES TO BUILD TRUST
- INCORPORATE FAIR DATA PRINCIPLES

**Technical Architecture**
- PIS / consent
- IGA PORTAL (CRA MANAGED)
- PATIENT VERIFICATION
- IGAR PLATFORM
- DOC / LAB
- MULTIPLE REGIONS

**Pharma Requirements & Priorities**
- Improve disease understanding / define natural history
- Convey patient / carer disease impact
- Validate new outcomes / endpoints
- Support the identification of patients for clinical trials
- Provide Real World Data for regulatory
- Provide real world data for reimbursement submissions
- Pharmacovigilance requirements post MA for each new drug on the market
- Anything else (free text)
- High Importance
  - 5
  - 4
  - 3
- Low Importance
  - 1
  - 2

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The IGA is an international umbrella group representing the interest of Gaucher patients and those of non-profit Gaucher patient groups as well as rare disease groups throughout the world.