

## How was GARDIAN developed?

Initiated by the International Gaucher alliance (IGA) four pharmaceutical companies responded to participate and fund the project: [Oxyrane](#), [Pfizer](#), [Prevail](#) and [Sanofi](#) Genzyme. Phase 1 commenced in January 2019 and lasted 21 weeks and included five workstreams:

1. Involvement of patients and parents/carers to understand the burden of the disease;
2. Input of pharmaceutical companies to define how such a registry could work for them in terms of compliance, governance and regulation;
3. Involvement of global key opinion leaders in a Delphi process to identify what data capture fields to be included in the registry;
4. Designing the technical architecture of the global registry;
5. Communication/ engagements and project management.

The outcomes from Phase 1 were shared with the partners in June 2019 and then widely shortly after through a combination of conferences and presentations. It included recommendations for the deployment and implementation of a global disease registry, which will meet the needs of all the stakeholders but offer a sustainable business plan to make the registry a viable asset for the community.

The development of a global registry driven by patients aims to facilitate patient centric clinical trials of emerging drugs for nGD.

A copy of the community report outlining the feasibility study can be found [here](#).