

Communicating about GARDIAN

Frequently asked questions

What is GARDIAN and why is it needed?

What is GARDIAN?

GARDIAN (the Gaucher Registry for Development, Innovation and Analysis of Neuronopathic disease) is a patient registry launched by the International Gaucher Alliance (IGA). The registry will gather information directly from patients with type 2 and 3 Gaucher disease (often referred to as nGD) and their caregivers to provide a clearer picture of the disease and how it affects patients and caregivers. The aim is to highlight the challenges of living with this ultra-rare and under-resourced disease and to improve the understanding and management of nGD among medical professionals. The ultimate goals for GARDIAN are to improve standards of care and to help design safer and more effective treatments.

What is a patient registry?

A registry patient is designed to gather uniform data about the disease. It tracks information about the health status of patients and the care they receive with the aim of increasing understanding of a disease and improving outcomes for patients.

For GARDIAN, patients and their caregivers will report on their health status, care, challenges, and other aspects of nGD. The collection of clinical data will be limited and minimal.

How can a patient registry improve patient care?

Gathering information in a standardized and consistent manner directly from a group of patients and caregivers can help to build a more detailed picture of the impact of a disease, which disease-related characteristics are important to consider, what health related outcomes are of importance to patients and caregivers and how the patient experience and care could be improved.

This is particularly important in the case of ultra-rare diseases such as nGD, whereby increasing awareness and sharing experiences, patients and caregivers can make a difference in the future management of nGD.

Equally importantly, GARDIAN will provide those affected by nGD with a much-needed public voice. GARDIAN will offer the opportunity to patients and caregivers to communicate the reality of living with this ultra-rare and under-resourced disease and to become an advocate for the nGD community.

Why has GARDIAN been developed?

There is an urgent need to increase awareness of nGD, to improve patient care and to develop safer and more effective treatments for the neuronopathic aspects of Gaucher disease. Currently, there are no comprehensive, uniform, and reliable sources of knowledge for nGD. GARDIAN will be the first global registry to focus specifically on nGD. It will collect data in a systematic and consistent, nonbiased way.

What are the aims of the GARDIAN registry?

GARDIAN will act as a research platform for nGD. It will collate detailed information on who is affected by nGD and the impact it has on patients and caregivers. The ultimate aims are to improve standards of care and to help design safer and more effective treatments.

What information will GARDIAN collect?

GARDIAN will capture disease related health data directly from patients or caregivers with a special focus on symptoms and impact of nGD. The information gathered will be limited to patient generated health data and caregiver reported data but will also include minimal clinical data.

Has anything similar been set up before?

GARDIAN will be the first global disease registry to focus specifically on nGD. It will collect data in a systematic and consistent way. This data will be used to gain a better understanding of who is affected by nGD, to evaluate the impact it has and to highlight unmet needs. Prior registries were focused predominantly on Gaucher Disease Type 1 and run by pharmaceutical companies; GARDIAN is created and run by the Gaucher patient advocacy group.

Who can take part?

GARDIAN will collect data directly from patients or caregivers. Patients (or caregivers on behalf of patients, where applicable) must meet all of the following inclusion criteria to be eligible for the study:

- Patient with confirmed GD3 diagnosis or primary or co-primary caregiver of a patient with a confirmed GD2 or GD3 diagnosis
- Access to an internet-connected mobile device (e.g., smartphone, tablet) or computer
- A working email account
- Able to read and understand one of the study languages
- Signed opt-in consent document indicating that the patient has been informed of all pertinent aspects of the registry.

How will GARDIAN benefit the type 2 and type 3 (nGD) community?

GARDIAN has the potential to significantly increase understanding of nGD and to improve standards of care for patients. The ultimate aims are to encourage the development of safer and more effective treatments leading to improved patient care and address caregiver burden.

Equally importantly, GARDIAN will provide those affected by nGD with a much-needed public voice. It will help to communicate the reality of living with this ultra-rare and under-resourced disease and to allow patients and caregivers to become advocates for the nGD community.

Why should I take part?

GARDIAN has the potential to transform standards of care for people with nGD. It will help to improve understanding of this ultra-rare and under-resourced disease among the medical community, health authorities seeking to approve new treatments and the general public.

Equally importantly, it provides those affected by the disease with a much-needed public voice. It offers the chance to communicate the reality of living with this disease. Sharing experiences will help to ensure that relevant parties have a clearer understanding of the needs of nGD patients and their caregivers and this has the potential to shape future management of the disease. Those taking part will also have the satisfaction of knowing that they are making a difference and helping others as well as themselves.

GARDIAN will only succeed in its aims if it receives widespread support from nGD community.

How will GARDIAN work?

When does GARDIAN open?

The content of GARDIAN is currently being tested and finalised. GARDIAN will open for registration in December 2021.

How long will GARDIAN run for?

GARDIAN will initially run for three years. At the time of launching GARDIAN there is secured funding for this timeframe. We hope to secure further funding for GARDIAN beyond three years.

When will results become available?

Our target is the end of 2022.

What information will GARDIAN gather?

GARDIAN will gather data from patients and caregivers on quality of life for those affected by the disease.

Different questionnaires will be used in GARDIAN. Some have been developed specifically for neuronopathic Gaucher disease and others are more general questionnaires that have been designed to collect information about the health-related issues and are used in research to collect data in a systematic and consistent way and have been tried, tested and approved for use.

In which languages will GARDIAN be available?

Initially GARDIAN will be available in 8 languages: UK English, US English, Arabic, Japanese, Simplified Chinese, German, Spanish and French.

How do I take part in GARDIAN?

How do I register?

To register your interest, click on the link on the [IGA website](#) and follow the instructions.

Do I need a smart phone/device?

To join the registry, you need access to an internet-connected mobile device (e.g., smartphone, tablet) or computer.

Do I have to answer all the questions?

To enable GARDIAN to collect the data we need it is important that you answer all of the questions, this will ensure that there are no data gaps, and that all participants' data can be used to enable a comprehensive understanding of the burden of disease on patients and their caregivers. Data gaps will mean less data which will leave gaps in our understanding. Also note that for most of the questions, you will be offered the possibility to select "I don't know/I don't remember" or "I prefer not to answer".

Is it a one-off or will I need to add information on a regular basis?

It is expected that you will be asked to enter your information when you first enrol into GARDIAN and then every six months after that. At pre-set intervals, specific self-report measures will be offered back to GARDIAN participants. Please note that you will receive a follow-up questionnaire between day 10 and 18 after you have joined GARDIAN that may ask you to complete a few of the same questionnaires that you completed when you joined. This is important as it is a validation process and will highlight any challenges that we may need to be aware of in collecting the data.

Do I need to tell my physician I am taking part?

It is not mandatory, but we would encourage you to tell your physician as they may not be aware of GARDIAN, and they may have other patients/caregivers who may be interested in joining the registry.

What happens if my physician is not aware of GARDIAN?

You can still take part in GARDIAN by accessing the link on the [IGA website](#). You may wish to suggest that your physician accesses the information so that they can learn about the initiative.

Can I get help completing the online questionnaire?

GARDIAN is working with the global Gaucher community and will be identifying GARDIAN champions, volunteers who will be willing to help you complete the questionnaire or answer any questions that you may have.

What happens if I have problems completing the questionnaire?

Please email, GARDIAN@gaucheralliance.org

How will my data be used?

Who will own the data generated by GARDIAN?

The data collected in GARDIAN will belong to the patient community through the International GARDIAN Limited. This is a company wholly owned by the International Gaucher Alliance, a registered charity.

How can I be sure my data will be safe?

GARDIAN will be overseen by a steering committee, which will ensure that the registry complies with all relevant regulations (such as Institutional Board directives). It will protect patient data safety and confidentiality and ensure that GARDIAN remains independent and free from influence from third parties.

Will my data be anonymous?

Yes, all information you provide as part of your participation in the GARDIAN registry will be held in the strictest confidence and will be anonymised. A code number will be generated automatically when you enter the information in the registry application. This code number will be used in place of your name and other information that can identify you.

Please note that prior to your participation in the registry, you will be asked to upload some documents as a proof that you are a nGD patient/caregiver. Once you have been verified and received an email to confirm you are eligible for the registry, the documents that you sent as a proof will be automatically destroyed and your data will be coded using a unique identification number (code number).

Who will have access to the results of the research registry?

Before participating to the study, you will be requested to provide your consent to authorize your coded information, aggregated with that of the other participants, to potentially be shared with the following entities for data analysis, quality assurance and to ensure that the study rules are followed.

- The International Gaucher Alliance (IGA), the International GARDIAN Limited (IGL), and those working for or with IGA, which may include other IGA entities located in your country or other countries; services providers of the IGA.
- Ethics committees that oversee the research.
- Health Authorities or worldwide agencies

All participants' aggregated information may also be shared with stakeholders engaged in nGD research including clinicians, pharmaceutical companies and advocacy organizations as approved by the Registry team. Your individual identifiable information will never be sold.

The overall findings of the study may be published in a scientific journal to contribute to raising awareness in the scientific community and beyond, but these will never mention or identify you individually, in any way.

Can I opt out of any sections or change my mind at any stage?

Your participation is voluntary (your choice). You may refuse to take part or withdraw from the study at any time, without giving a reason and without any penalty or loss of benefits to which you are otherwise entitled and without any effect on your future medical care.



You can change your mind or opt out, and/or decide to stop your participation in the GARDIAN registry at any stage/sections of the survey. In that case, please contact the IGA by emailing GARDIAN@gaucheralliance.org.

Your account will be then permanently deleted.

Please note that any information already entered by you, until your withdrawal, will be kept in the registry and will continue to be processed for the purposes of scientific research.

Will I have access to the results?

The results will also be shared with you via the IGA, in an aggregated anonymised format.

However, if you want specific information on the results, you can send your request to IGA, by emailing to: GARDIAN@gaucheralliance.org

[For further information about GARDIAN, click on the link on the IGA website.](#)