

www.gaucherregistry.com
www.gauchercare.com

Genzyme Corporation
500 Kendall Street
Cambridge, MA 02142 U.S.A.
800-745-4447 ext.15500 or
617-768-9000 ext.15500
Monday - Friday 8 am - 6 pm EST
help@gaucherregistry.com

Genzyme Europe B.V.
Gooimeer 10
1411 DD Naarden
The Netherlands
+31 35 699 1232
Fax +31 35 699 8688
europe@gaucherregistry.com

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Working Toward A More Complete Understanding of Gaucher Disease



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Patient Guide

Increasing the Understanding of Gaucher Disease

The Gaucher Registry was created to gather information to improve the understanding and treatment of Gaucher disease.

Because Gaucher disease is rare, it can be difficult to collect information on how the disease and its treatment affect different people. The Gaucher Registry is a database for gathering this critical information.

All people who have Gaucher disease are eligible to participate, regardless of whether they are on disease-specific treatment. Your physician is the one who will enroll you in the Gaucher Registry. He or she will explain the program and will ask you to sign an authorization to participate in the Registry. Your participation is completely voluntary.

When you volunteer to participate in the Gaucher Registry, your medical information is pooled with other participants' and is used to assist physicians in studying

trends and addressing specific questions about treating Gaucher disease. This information may contribute to better care for you and other people living with Gaucher disease.

About Gaucher Disease

Gaucher disease is an inherited disorder caused by a defective gene. A person who inherits this gene is unable to produce enough of an essential enzyme called glucocerebrosidase, which breaks down a fatty substance called glucocerebroside. Since a person with Gaucher disease does not produce enough of this enzyme, the fatty substance is not broken down, but instead builds up in the cells. These swollen cells are called Gaucher cells.

Over time, as Gaucher cells build up in the bone marrow and organs, they cause increasing damage, possibly including bone pain, skeletal damage, enlarged liver and spleen, and anemia. However, if the disease is recognized early, treatment can begin.

Benefits of the Gaucher Registry

In addition to helping physicians better understand Gaucher disease, the Registry can also benefit you. For instance, it can help you and your physician monitor your disease, whether or not you are currently on treatment.

When you are enrolled in the Registry, your physician can produce a Patient Case Report every time you have an appointment. This report uses charts, graphs, and text to show how you are progressing on important measures, including:

- Your bones
- Your blood
- Your liver and spleen
- Your quality of life

The report contains an overview of your health status as it relates to Gaucher disease. Your physician can review it with you to give you a picture of how your health progresses over time.

Take an Active Role

When you take an active role in your health care, you feel more

in control. Talking to your doctor is an important first step. Here are some ways you can use the Registry to keep the lines of communication open:

- Ask your physician if he or she is enrolled in the Gaucher Registry
- Let your physician know you are interested in participating in the Registry
- Ask your physician or nurse to share your Patient Case Report with you at your next visit

Your Privacy is Protected

Patient privacy is a top concern for the Gaucher Registry. Information about you can only be submitted following your written authorization. You will not be identified by name, as patient names are not collected by the Registry. Instead, all enrolled patients are randomly assigned an identification number. Only your physician will know what number is assigned to you.

Any information that would identify you as a patient of your physician is also kept confidential. All information in the Registry is collected in a way that prevents the identification of specific patients.

Learn more

To learn more about the Gaucher Registry, talk to your doctor or visit www.gaucherregistry.com.