

Natural History Study of Infantile and Juvenile GM1 Gangliosidosis (GM1)

Why participate in this study?

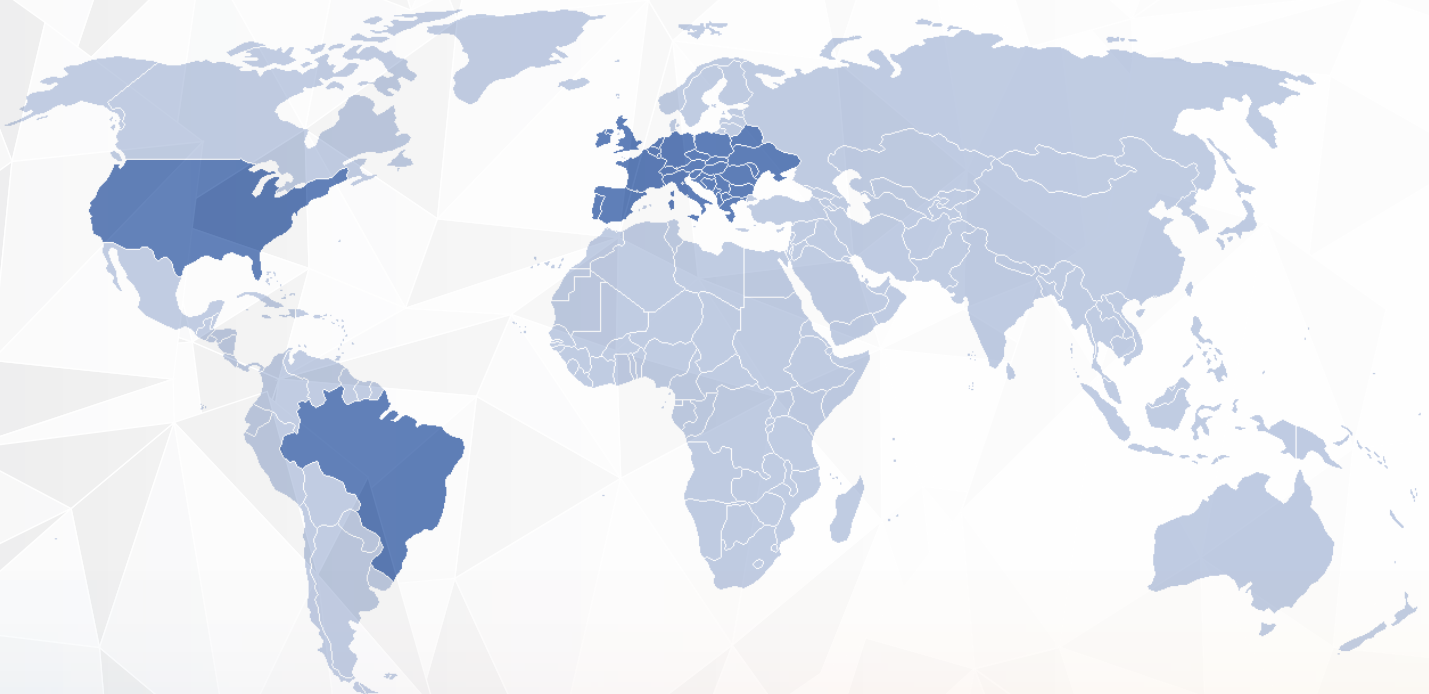
The Orphan Disease Center, with funding from Passage BIO, Inc. is launching a natural history study to understand the progression of Infantile and Juvenile GM1 Gangliosidosis. Natural history studies do not directly produce new therapies for diseases, but they allow researchers to gain a better understanding of the disease. The data from natural history studies can also act as a comparison for future clinical trials, which allows researchers to more easily show possible benefits of new therapies. Ultimately, a process will be implemented to provide access to the data from this study to the disease and scientific communities. Our hope is that this data can be used to progress the science toward an approved treatment for GM1.

Study Details

The purpose of this natural history study is to gain a better understanding of the long-term progression of both Infantile and Juvenile GM1 gangliosidosis. Enrollment will begin in the US in Mid2019. Patients and their parents/ caregivers will be asked to attend study visits every 6 months over the course of 3 years. During these visits, physicians and their teams will evaluate and examine patients at through various tests and procedures- some of which are performed standardly for GM1 patients and others that are considered optional. For patients and their families who do not live close to one of the study sites below, travel costs can be reimbursed for study visits.

Where can patients enroll in this study?

Patients can enroll at one of 4-5 centers in the United States, Brazil, and Europe



Want to learn more? Contact:

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GM1 Natural History Study FAQ

Q: Why participate in this study as well as the NIH study?

A: There is no direct benefit to you or your child to participate in this natural history study. You may want to participate in both this natural history and the NIH natural history study. These studies do include some different measurements and tests, so the data collected from your visits in either/both studies is incredibly valuable and will help scientists and researchers working on a future treatment for GM1 in the following ways: 1) the data collected from your child will help scientists to better understand the natural course of the disease (i.e. to track the various signs and symptoms of the disease over time); 2) it may help scientists choose the best measurements for future studies that test potential treatments; 3) the data collected from the natural history study are intended to be used in future studies to compare to an investigational treatment.

Q: Does participating in this study exclude me from future clinical trials?

A: No. However, participation in a clinical trial with an investigational therapy for GM1 would exclude you from further participation in the natural history study, as we would like to study the natural progression of GM1 without treatment or intervention. In this case, we would still analyze the data that was collected up to the point of your child's enrollment in the clinical trial for the investigational treatment, which is still very valuable to the Orphan Disease Center.

Q: Would I have to stop my treatment to participate if I am already enrolled in a clinical trial?

A: If you are already enrolled in a clinical trial with an investigational therapy for GM1, you would not be eligible to participate in the natural history study, unless that therapy is miglustat (a drug being studied for use in GM1), which is allowed.

Q: Would we have to keep track of things that happen outside of study visits?

A: Maybe. If you and your family live far away from the study site and are unable to travel at some point after you have enrolled in the study, we may ask your study doctor to provide us with information about you that is collected at your regular doctor visits at home. We would not ask your home doctor to do any more tests than he/she would normally do.

Q: What kinds of procedures or assessments will be done during the visits?

A: There are two groups of tests performed as part of this natural history study- a set of required tests and a set of optional tests. The **required** tests are things that you have most likely done before during visits to your doctor, such as a physical exam, vital signs, a neurological exam, blood draws, a urine test, some interview-type assessments of development and behavior, an ECG (electrocardiogram) and an ECHO (echocardiogram). The **optional** procedures are things that are a little more invasive, such as MRI scans, a spinal tap and an EEG (electroencephalogram). If you choose to undergo the optional procedures, you can also discuss the option of undergoing general anesthesia or sedation with your doctor.