

Largest Global Studies in Gaucher Disease Underway

Research Studies for Investigational Oral Medication Now Enrolling Participants

Volunteers are needed across the country to participate in one of three new clinical studies to evaluate if an investigational oral medication can help manage Gaucher disease type 1. The three studies — ENGAGE, ENCORE and EDGE — are the Explorer Studies and, together, they represent the largest studies of Gaucher disease ever conducted.

These trials are designed to evaluate if the investigational oral medication, eliglustat, is safe and effective in the treatment of Gaucher disease type 1 signs and symptoms.

ENCORE Study

The primary goal of the ENCORE study is to assess the safety and effectiveness of eliglustat compared to imiglucerase, a commercially available enzyme replacement therapy (ERT) made by Genzyme®, after 12 months of treatment in patients previously treated with any ERT who have met therapeutic goals. Participants will be assigned to one of two groups: those who will receive the investigational oral medication, eliglustat, or those who will be treated with imiglucerase. After 1 year in the study, all participants will receive eliglustat twice daily until it becomes commercially available or the study closes.

ENGAGE Study

The primary goal of the ENGAGE study is to assess the safety and effectiveness of eliglustat compared to placebo after nine months of treatment in untreated patients with Gaucher disease type 1. Participants will be assigned to one of two groups: eliglustat or placebo (a capsule with no active ingredient), twice daily. After nine months in the study, all participants will receive eliglustat twice daily until it becomes commercially available or the study closes.

EDGE Study

The EDGE study is comparing once-daily to twice-daily dosing of eliglustat. Eligible participants will be treated with eliglustat twice daily for at least six months and then be randomly assigned to receive either once-daily or twice-daily dosing for 12 months.

To learn more about the Explorer Studies, please visit www.explorerstudies.com. Participation in these studies may help us more effectively treat patients with Gaucher disease in the future.

About Eliglustat

The investigational oral medicine (eliglustat) being studied in the Explorer Studies is a substrate reduction therapy. The investigational oral medicine is believed to decrease the production of the fatty substance (glucosylceramide) that accumulates in Gaucher disease by decreasing the activity of the enzyme (glucosylceramide synthase) that produces the fatty substance. Eliglustat was designed to specifically target this enzyme.

Data from a Phase 2 study showed that a majority of patients experienced a reduction in spleen and liver size, as well as an increase in healthy red blood cells, platelet counts and an increase in bone strength. One patient experienced irregular heartbeats; other drug-related side effects included infrequent abdominal discomfort, diarrhea, headache and transient heart palpitations (sensation of a pounding increase in heart beat). Each of these events occurred in 1 or 2 patients, were mild and lasted only a short time.

Although taken orally, eliglustat should not be confused with Zavesca® (miglustat), another oral compound already commercially available in some countries.

The Explorer Studies are sponsored by Genzyme. Genzyme has a history of research for, and commitment to, the Gaucher Disease community.

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