

GBT HOPE STUDY

Global Blood Therapeutics has launched the <u>GBT HOPE</u> (Hemoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) study, a global clinical research study to evaluate a new potential treatment for sickle cell disease, GBT440.

What is GBT440?

GBT440 is a potential therapy for patients with sickle cell disease that is designed to attack sickle hemoglobin, the cause of the disease. GBT440 works by helping hemoglobin, the molecules inside red blood cells, hold onto more oxygen as the red blood cells travel through the body. This may help keep red blood cells in their normal shape and stop sickling.



GBT440 is taken by mouth once each day.

What is the GBT HOPE Study?

The <u>GBT HOPE</u> (Hemoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) study is a global clinical research study that will evaluate whether GBT440 will be safe and effective in reducing anemia, pain crises, fatigue and other day-to-day symptoms in adults and adolescents with sickle cell disease. It will also study the effects of different doses of GBT440 to determine the best dose to give to adolescents and adults with sickle cell disease.

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Who Can Participate in the HOPE Study?

The HOPE study will include up to 400 adults and adolescents. In order to qualify for the HOPE study, a person has to have been diagnosed with sickle cell disease, be between the ages of 12 and 65 and have had at least one pain crisis in the past year.

Before someone can participate in the HOPE study, they must go through a screening process. Study doctors and staff will evaluate potential participants and explain other requirements for enrollment.

How Will the HOPE Study Work?

The HOPE study will evaluate the safety and effectiveness of GBT440. Participants will be required to fill out a daily electronic patient diary that is designed to capture patients' experience and overall severity of symptoms due to their sickle cell disease. The electronic patient diary should take no more than 5-10 minutes to fill out each day.

The HOPE study is a randomized placebo-controlled clinical study, which means that participants will be assigned at random (like flipping a coin) to receive either the investigational medication, GBT440, or placebo. A placebo is a "dummy pill" that looks like the investigational medication but does not contain any drug. The placebo pill is critical to determine whether or not GBT440 is actually effective.

After participants complete the study, they may be able to take GBT440 in an extension of the HOPE study, regardless of whether they received GBT440 or placebo during the study.

How Do I Know if There is a Clinical Study Site Near Me?

The HOPE study is a global clinical research study that will include sites throughout the United States. To find information about study sites in your area you can visit <u>www.gbtsicklecellstudy.com</u>, talk to your doctor, or contact your local community-based organization(s).

The HOPE study is sponsored by Global Blood Therapeutics (GBT) Inc., a biopharmaceutical company committed to advancing the science and transforming the treatment of sickle cell disease. To learn more about GBT, visit <u>www.globalbloodtx.com</u>.

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News & Other Links www.clinicaltrials.gov www.gbtsicklecellstudy.com www.globalbloodtx.com





Clinical Research Information Sheet

Study Name	Hemoglobin O xygen Affinity Modulation to Inhibit HbS P olym E rization (HOPE)
Study Type	Interventional
Study Sponsor	Global Blood Therapeutics, Inc. (NASDAQ: GBT)
Study Phase	Phase 3
Recruitment Status	Actively Recruiting Patients
Study Drug(s)	GBT440 or placebo (randomly assigned)
Number of Participants Being Recruited	400
Single/Multi-Center	Multi-Center

About the HOPE Study

The HOPE (Hemoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) study is a Phase 3 clinical research study that will evaluate if an investigational medication called GBT440 will be safe and effective in reducing the damage sickle cell disease causes to red blood cells, and therefore, decrease day-to-day symptoms of sickle cell disease, including anemia, pain crises, and fatigue, in adolescents and adults with sickle cell disease. The study will also evaluate the effects of different doses of the investigational medication to determine the most appropriate dose for those suffering from sickle cell disease.

HOPE Study Eligibility

Individuals qualify to participate in the HOPE study if they:

- Have been diagnosed with sickle cell disease
- Are between 12 and 65 years of age
- Have had at least one pain crisis in the past year

Before someone can participate in the HOPE study, he or she will need to go through the screening process to determine if they meet all the requirements. Study doctors and staff will evaluate potential participants and explain other requirements for enrollment.

For more information on participation, study sites or any other inquiries about the HOPE study, please visit <u>www.gbtsicklecellstudy.com</u>.

For More Information

Sponsor Contact Information	[GBT to insert contact information]
Trial Specific Link on <u>www.ClinicalTrials.gov</u>	https://clinicaltrials.gov/ct2/show/NCT03036813?term=GBT&rank=2
Clinical Research Terms Glossary	<u>Click Here</u>

Participating Sites

For information on a HOPE study site closest to your area, please click here: HOPE Study Site Finder

