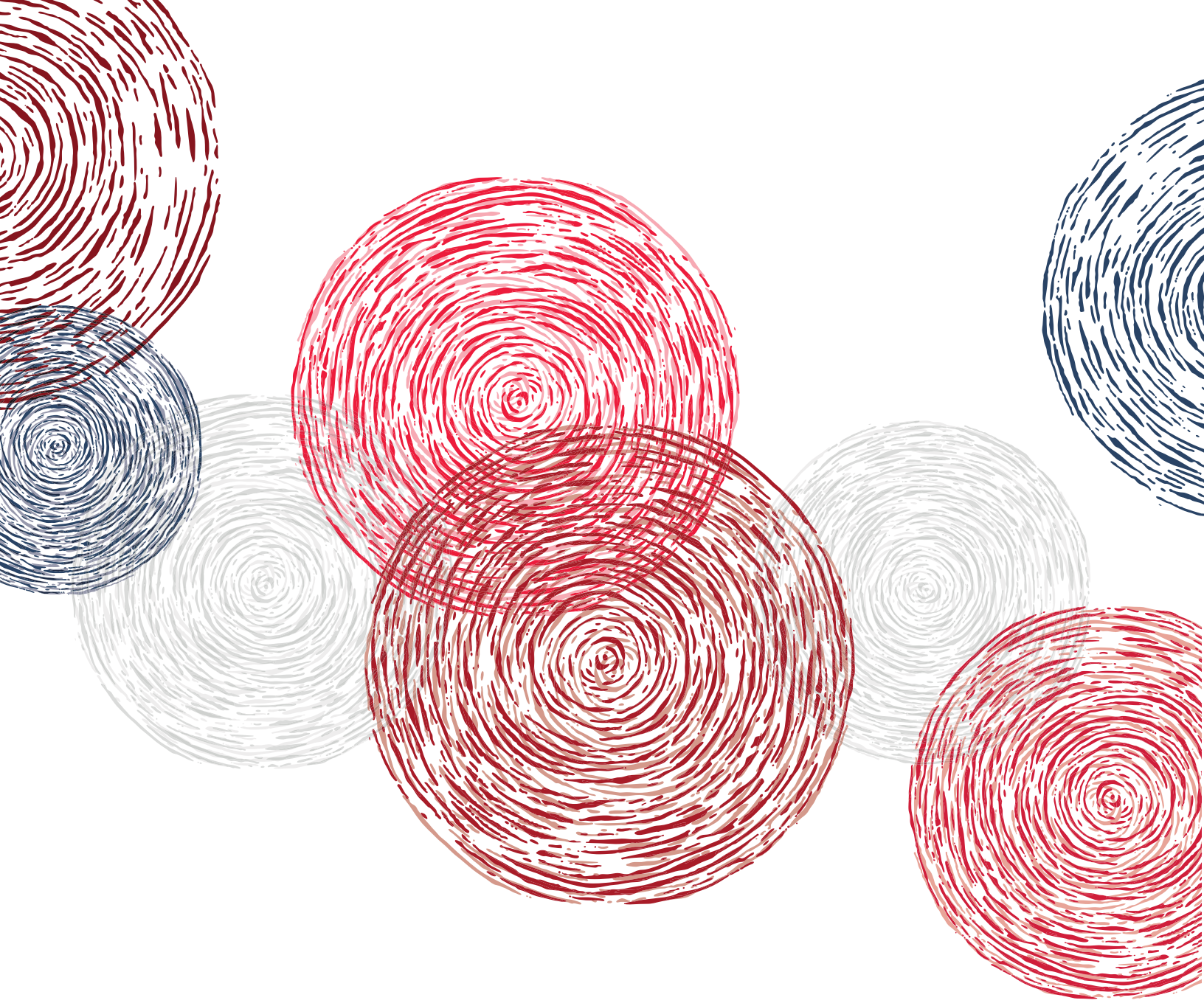

GBT
HOPE Study

gbt

INTRODUCTION

Global Blood Therapeutics is launching the GBT HOPE Study – a Phase 3 clinical research study that will evaluate if an investigational medication called GBT440 will be safe and effective in reducing anemia, pain crises, fatigue, and other day-to-day symptoms in adolescents and adults with SCD.

Global Blood Therapeutics, Inc., or GBT for short, is a clinical-stage biopharmaceutical company dedicated to discovering, developing and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet need. GBT is committed to working closely with patients, their families and community organizations to maintain an open dialogue with the sickle cell community to understand patients' unique needs and share the latest information on ongoing clinical research studies and other research.

Frequently Asked Questions

About GBT440

Q: What is GBT440? How does GBT440 work?

A: GBT440 is a potential therapy for patients with sickle cell disease (SCD) that is designed to attack the cause of the disease. GBT440 is taken by mouth daily. It works by helping hemoglobin, the molecules inside red blood cells, hold onto more oxygen as the red blood cells travel throughout the body, which prevents them from sticking together. This may help red blood cells keep their normal shape and stop sickling.

Q: How has GBT440 been studied?

A: To date, GBT440 has been studied in Phase 1 and Phase 2 clinical research studies, and enrollment is currently underway for a large Phase 3 clinical research study in adolescents and adults with SCD. For more information, please visit the Global Blood Therapeutics website at www.globalbloodtx.com.

Q: Is GBT440 currently available to people living with SCD?

A: At this time, GBT440 is only available to eligible individuals living with SCD through the HOPE clinical research study and is not available by prescription.

Q: Is GBT440 safe?

A: To date, studies have shown that GBT440 is well-tolerated, and helps reduce the damage SCD causes to red blood cells. A Phase 3 clinical research study called the GBT HOPE study (more information below) will further evaluate the safety and efficacy of GBT440 in a larger number of patients living with SCD.

About Clinical Research Studies

Q: What is a clinical research study?

A: A clinical research study, also referred to as a clinical trial, is a study designed to evaluate whether an investigational medication or treatment is safe to use and works to improve the health of people. Before any medication can be approved and made available to the general public, it has to go through several phases of clinical research. Many treatments available today to people are the result of past clinical research studies. Participation in any clinical research study is completely voluntary, and participants can stop participating at any time for any reason - or no reason at all. While individual participants may or may not benefit directly from being in a clinical research study, the information gathered in the study may help others in the future.

Q: Is participating in a clinical research study safe?

A: All clinical research studies in the United States are approved and monitored by a group of independent medical and research experts called an Institutional Review Board (IRB). In addition to scientists, the IRB also includes laypeople who are non-experts in the field such as lawyers, patients, patient advocates, clergy or bioethicists who review and evaluate the study from a neutral perspective. The IRB ensures that the risks involved in a study are minimal and enforces strict ethical guidelines. At any time during a clinical research study, a participant may stop participating in the study, for any reason.

Q: Where can I go for more information on clinical research studies?

A: For more information on clinical research studies, please visit www.clinicaltrials.gov or www.nih.gov.

About the GBT HOPE Study

Q: What is the HOPE study?

A: The HOPE (**H**emoglobin **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 SCD causes to red blood cells, and therefore, decrease day-to-day symptoms of SCD, including anemia, pain crises, and fatigue, in adolescents and adults with SCD. The study will also evaluate the effects of different doses of the investigational medication to determine the most appropriate dose for those suffering from SCD. An investigational medicine means that it has not been approved for use by the U.S. Food and Drug Administration (FDA) or other regulatory agencies.

Q: What is the purpose of the HOPE study?

A: The HOPE study will evaluate whether GBT440 will be effective in reducing the damage SCD causes to red blood cells and therefore, decrease the day-to-day symptoms of SCD in adults and teenagers. It will also examine the effects of different doses of GBT440 to determine the best dose to give to adults and teenagers with SCD.

Q: Who can participate in the HOPE study?

A: Individuals may qualify to participate in the HOPE study if they:

- Have been diagnosed with SCD
- 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 a screening process to determine if they meet all the requirements for participation. Study doctors and staff will evaluate potential participants and explain other requirements for enrollment.

Q: If I don't qualify for the HOPE study, are there any other GBT440 clinical research studies?

A: The HOPE study is part of a series of clinical research studies that GBT will undertake to evaluate the safety and efficacy of GBT440. The goal of these studies will be to examine GBT440 in other age groups, including infants. As these studies are initiated, additional information will become available at GBTsicklecellstudy.com.

Q: Where are the HOPE study sites located?

A: The HOPE study is a global clinical research study that includes sites throughout the United States. The first sites will open in early 2017. All clinical research study sites will be listed on the GBTsicklecellstudy.com website. If individuals are interested in more information or enrolling in the HOPE study, they are encouraged to talk to their doctor and their local community-based organization(s) for more information, or visit GBTsicklecellstudy.com.

Q: How long will individuals participate in the HOPE study?

A: Enrollment for the HOPE study is underway. Participation in the HOPE study will last at least 2.5 months and up to 1.5 years, depending on when someone is enrolled.

Q: What will participation in the HOPE study look like?

A: Up to 400 adolescents and adults with SCD are expected to participate in the HOPE study and will be divided into three different groups. Participants in each of the three groups will receive either the investigational medication or the placebo. The placebo looks like the investigational medication but does not contain any drug. The placebo group is critical to determine whether the investigational medication is effective or not. After the completion of the study, participants may be able to take the investigational medication in an extension of the HOPE study, regardless of whether they were taking the placebo or investigational medicine in their assigned group.

During the study participants will need to attend study site visits. During the visits, study staff will check on the participants' overall health and SCD symptoms using standard medical tests and procedures. When they are at home, participants will need to take their study medication each day, and spend approximately five minutes each evening completing an electronic diary about their SCD symptoms. The investigational medication and study procedures will be provided at no cost to participants.

Q: When will individuals find out if the HOPE study was successful?

A: Once the study is completed and the results have been analyzed, the results will be posted on GBTsicklecellstudy.com and on the www.clinicaltrials.gov website. A participant's HOPE study doctor or medical research study team will also be able to tell them the results of the study. Participants can also reach out to them at any time during the study to learn more about progress.

Q: Where can individuals go for more information about the HOPE study?

A: Please visit GBTsicklecellstudy.com for more information on the HOPE study.

About GBT

Q: Who is Global Blood Therapeutics?

A: Global Blood Therapeutics, or GBT for short, is a clinical-stage biopharmaceutical company dedicated to discovering, developing and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet need.

Q: Why is Global Blood Therapeutics interested in SCD?

A: GBT recognizes that the sickle cell community historically has been underserved, and is committed to working closely with patients, their families and community organizations to advance the science and transform the treatment of SCD. Through ongoing drug discovery and development work, GBT is working to identify and develop medications and treatments that they hope will address the root causes of various blood-based disorders, thus providing patients a way to manage their health, extend their life expectancy and improve their overall quality of life. GBT is deeply committed to developing meaningful therapies that transform patient lives.

Q: What other clinical research studies is Global Blood Therapeutics conducting?

A: GBT's primary focus is to evaluate the impact that GBT440 has on red blood cell sickling in patients with SCD. Beyond this current program, there are plans to investigate GBT440 as a potential therapy for acute and chronic hypoxemic pulmonary disorders, including idiopathic pulmonary fibrosis (IPF). For more information on the GBT pipeline of products, visit www.globalbloodtx.com.

GBT HOPE Study Fact Sheet

HOPE
GBT Sickle Cell Disease Clinical Study

What is Sickle Cell Disease (SCD)?

SCD is a lifelong inherited blood condition that affects the ability of red blood cells to do their job, which is to deliver oxygen to the body's tissues and organs. In people with SCD, hemoglobin- the molecule inside red blood cells- does not hold onto oxygen properly. This causes red blood cells to lose their normal round shape and become sickle-shaped and rigid. Sickle-shaped red blood cells get stuck in small blood vessels and block the flow of blood and oxygen to the body, often causing severe pain and inflammation, among other symptoms.

What is a Clinical Research Study and Why is it Important?

Clinical research studies, also called clinical trials, are done to test whether investigational medications are safe to use and work to improve the health of people. Before any medication can be approved and made available to the general public, it has to go through several phases of clinical research. Many treatments available today to people are the result of past clinical research studies.

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WHAT IS THE GBT HOPE STUDY?

The HOPE (**H**emoglobin **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 SCD causes to red blood cells, and therefore, decrease day-to-day symptoms of SCD, including anemia, pain crises, and fatigue, in adolescents and adults with SCD. The study will also evaluate the effects of different doses of the investigational medication to determine the most appropriate dose for those suffering from SCD. An investigational medicine means that it has not been approved for use by the U.S. Food and Drug Administration (FDA) or other regulatory agencies.

GBT440 is a potential therapy for patients with SCD that is designed to attack the cause of the disease. It works by helping hemoglobin, the molecules inside red blood cells, hold onto more oxygen as the red blood cells travel throughout the body, which prevents them from sticking together. By preventing red blood cells from sticking together, this may help them keep their normal shape and may stop sickling from occurring.

Regulatory agencies like the U.S. Food & Drug Administration (FDA) or European Medicines Agency (EMA) and others oversee how clinical research studies are designed, and ultimately determine whether an investigational drug is approved. Clinical research studies are conducted in a series of phases that each help researchers answer different questions about a drug or treatment.

- **Phase 1**: Researchers test a new drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range and identify side effects.
- **Phase 2**: The drug or treatment is given to a larger group of people to see if it works and to further evaluate its safety.
- **Phase 3**: The drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the drug or treatment to be used safely.

Participation in any clinical research study is completely voluntary, and participants are allowed to stop participating at any time for any reason, or no reason at all. While individual participants may or may not benefit directly from being in a clinical research study, the information gathered in the study may help others in the future.

For more information on clinical research studies, please visit www.clinicaltrials.gov or www.nih.gov.

WHO CAN PARTICIPATE IN THE HOPE STUDY?

Individuals may qualify to participate in the HOPE study if they:

- Have been diagnosed with SCD
- 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 a screening process to determine if they meet all the requirements for participation. Study doctors and staff will evaluate potential participants and explain other requirements for enrollment.

HOW LONG WILL THE HOPE STUDY BE?

Enrollment for the HOPE study is underway. Participation in the HOPE study will last at least 2.5 months and up to 1.5 years, depending on when someone is enrolled.

3 HOW WILL THE HOPE STUDY WORK?

Up to 400 adolescents and adults with SCD are expected to participate in the HOPE study, and will be divided into three different groups. Participants in each of the three groups will receive either the investigational medication or the placebo. The placebo is a "dummy" pill. It looks like the investigational medication but has no active ingredients. After the completion of the study, participants may be able to take the investigational medication in an extension of the HOPE study, regardless of whether they were taking the placebo or investigational medicine in their assigned group.

During the study participants will need to attend study site visits. During the visits, study staff will check on the participants' overall health and SCD symptoms using standard medical tests and procedures. When they are at home, participants will need to take their study medication each day, and spend approximately five minutes each evening completing an electronic diary about their SCD symptoms. The investigational medication and study procedures will be provided at no cost to participants.

WHERE CAN I DIRECT INTERESTED INDIVIDUALS FOR MORE INFORMATION?

Individuals interested in more information, or participating in the HOPE study can visit GBTsicklecellstudy.com, or talk to their doctor.

GBT440 Fact Sheet

GBT440 is a potential new therapy for patients with sickle cell disease (SCD) that is designed to attack the cause of the disease. GBT440 is taken by mouth daily. To date, clinical research studies have shown that GBT440 is well-tolerated, and helps reduce the damage SCD causes to red blood cells. GBT440 is currently being studied in a Phase 3 clinical research study, called HOPE (**H**emoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization).

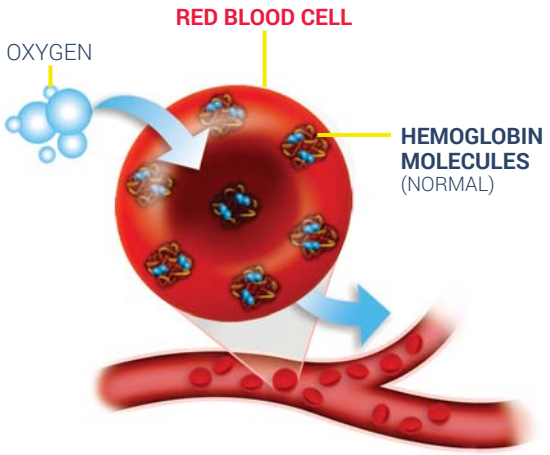
SCD is a lifelong, inherited blood condition that affects 100,000 Americans each year, half of whom are children. The disease is most common in African Americans, occurring in one in every 365 births. Hispanic Americans also have a higher rate of SCD than the general population.[1]

GBT440 has received orphan drug and fast track designations from the U.S. Food and Drug Administration. Together, these programs help speed up the availability of drugs that treat serious but rare conditions.

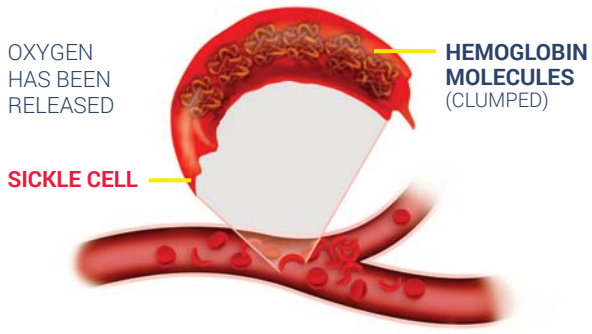
GBT440 is an investigational medication that is not currently approved by the U.S. Food and Drug Administration for the treatment of sickle cell disease.

HOW GBT440 WORKS IN THE BODY

In the body, tissue and organs need a steady supply of oxygen to work well. Oxygen is delivered to tissues and organs by hemoglobin, molecules inside red blood cells. Red blood cells that contain normal hemoglobin are round, which allows them to move easily through blood vessels to deliver oxygen.



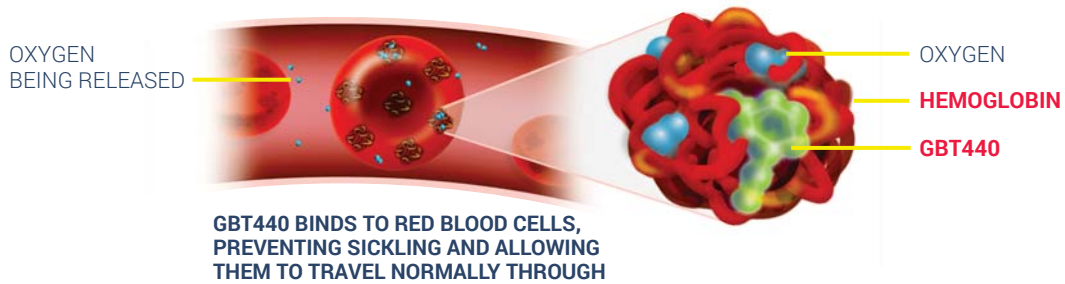
NORMAL RED BLOOD CELLS CARRYING OXYGEN FLOW FREELY IN THE BLOODSTREAM



SICKLED RED BLOOD CELLS STICK TOGETHER AND BLOCK THE FLOW OF BLOOD AND OXYGEN

In sickle cell disease, hemoglobin molecules become sticky after they release oxygen to tissues and organs. When too many hemoglobin molecules stick together, they cause red blood cells to lose their normal shape and become rigid. Sickle-shaped red blood cells get stuck in blood vessels and block the flow of blood and oxygen to the body. This can cause severe pain, called a sickle cell crisis, anemia, fatigue, organ damage, stroke, and other complications.

GBT440 works by helping hemoglobin hold onto more oxygen as the red blood cells travel through the body, which keeps these cells in their normal shape and helps stop sickling. By stopping sickling, red blood cells can move normally through the body, delivering oxygen to tissues and organs.



GBT440 BINDS TO RED BLOOD CELLS, PREVENTING SICKLING AND ALLOWING THEM TO TRAVEL NORMALLY THROUGH BLOOD VESSELS

FUTURE RESEARCH

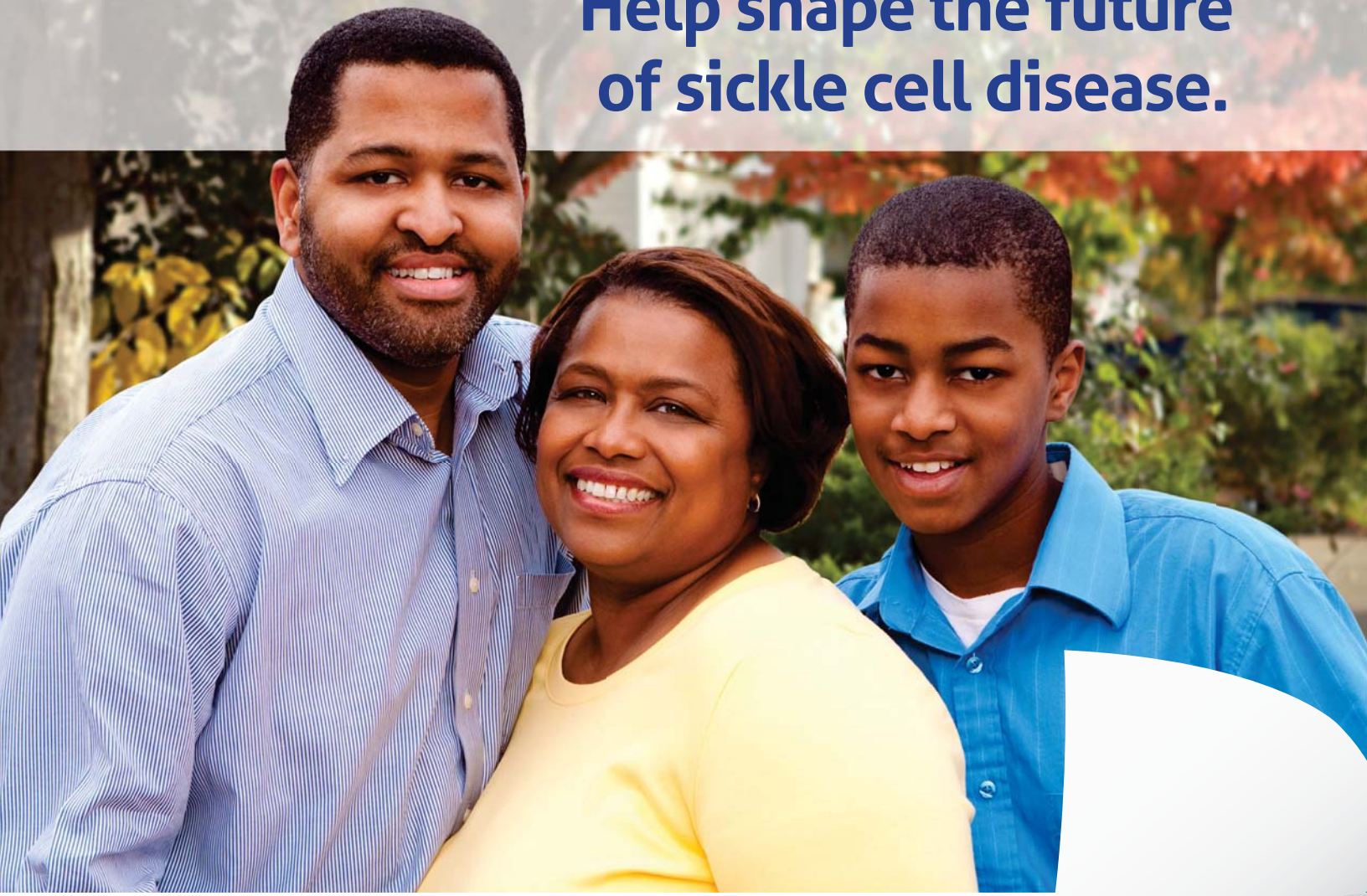
HOPE
GBT Sickle Cell Disease Clinical Study

Enrollment for the GBT HOPE Study, a Phase 3 clinical research study, is currently underway.

For more information on GBT440 or the HOPE study, visit www.globalbloodtx.com or GBTsicklecellstudy.com.

References
1. Centers for Disease Control and Prevention. Sickle Cell Disease Data & Statistics. http://www.cdc.gov/ncbddd/sicklecell/data.html

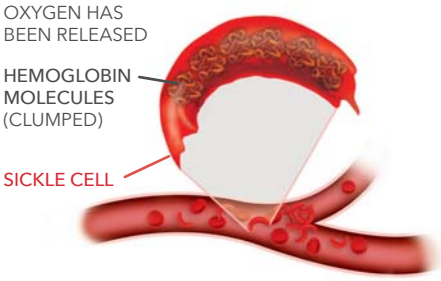
Help shape the future of sickle cell disease.



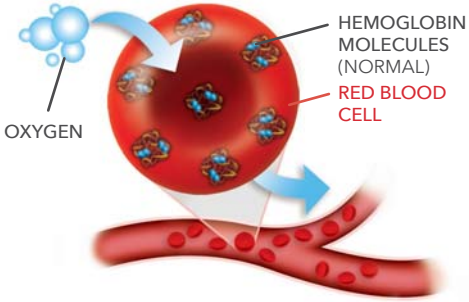
What is SCD?

SCD is a lifelong inherited blood condition that occurs when someone inherits two abnormal sickle cell genes, one from each parent.

In people with SCD, red blood cells lose their normal disc shape and become sickle-shaped and rigid. Sickle-shaped red blood cells get stuck in small blood vessels and block the flow of blood and oxygen to the body, often causing severe pain and inflammation.



Sickled red blood cells stick together and block the flow of blood and oxygen



Normal red blood cells carrying oxygen flow freely in the bloodstream

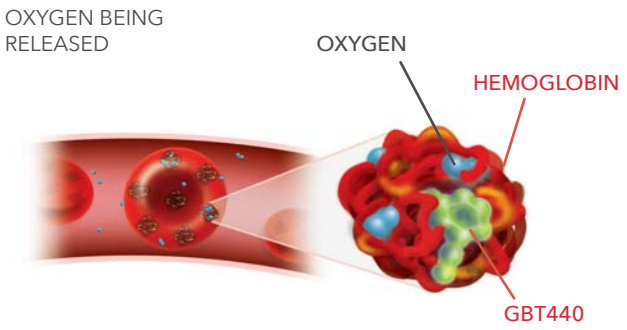
To see if you or someone you know may qualify to participate in the GBT HOPE study, visit

www.GBTSickleCellStudy.com.

HOPE
GBT Sickle Cell Disease Clinical Study

What is the investigational medication?

The investigational medication is taken by mouth each day and is designed to keep the hemoglobin molecules in red blood cells from sticking to each other. This may help red blood cells keep their normal shape and stop sickling.



GBT440 binds to red blood cells, preventing sickling and allowing them to travel normally through blood vessels

What is the purpose of the GBT HOPE study?

The GBT HOPE study will evaluate whether an investigational medication will be safe and effective in reducing anemia, pain crises, fatigue, and other day-to-day symptoms in adolescents and adults with SCD.

Who can participate in the GBT HOPE study?

To qualify for the GBT HOPE study, one must:

- Be between 12 and 65 years of age
- Be diagnosed with SCD
- Have had at least one pain crisis in the past year

The study doctor and staff will explain other requirements for participation.

What is a clinical research study?

Clinical research studies are done to evaluate whether investigational medications are safe to use and improve the health of people. Clinical studies help researchers better understand a disease and how to best treat it, and can also lead to new treatments.

Clinical studies are performed according to government regulations that protect the rights and safety of study participants. Participation in any clinical research study is completely voluntary. You or your loved one can stop participating at any time for any reason – or no reason at all. Doing so will not have a negative impact on the care provided to you or your loved one. While you may or may not benefit directly from being in a clinical research study, the information gathered in the study may also help others with Sickle Cell Disease (SCD) in the future.

What will happen during the GBT HOPE study?

If you or someone you know qualifies for the GBT HOPE study, participation will last at least 2.5 months and up to 1.5 years, depending on when you start. The study will involve up to 400 adolescents and adults with SCD.

After participants complete the GBT HOPE study, they may be able to take the investigational medication in an extension of the GBT HOPE study.

Participation in the study will be divided into three consecutive time periods:

Screening period (35 days)

Before being able to participate in the study, you or your loved one will go through a screening process to determine if you can safely participate. During this time, potential participants will be asked to fill out a 5-minute electronic diary about their SCD symptoms.

Treatment period (72 weeks)

Participants will be divided into three groups and will receive either the investigational medication or placebo. Placebo is a "dummy pill" that looks like the investigational medication but does not contain any drug. Throughout the study, participants will attend visits at the study site. When at home, participants will need to take their study medication each day and complete the electronic diary each evening. ***Study-related care and the investigational medication (or placebo) will be provided at no cost to participants.***

Follow-up period (4 weeks)

After participants take their last dose of investigational medication or placebo, they will be asked to come to the study site for a follow-up visit to check how they are doing and determine if there are any lasting effects of the investigational medication.

If you or someone you know is living with Sickle Cell Disease (SCD), you are aware that the effects of this disease span generations. Treatment options currently available are limited and may not work the same for everyone.

Clinical research studies are essential to help researchers learn more about future treatment options for people with SCD. You or someone you know can be part of this critical research by participating in the GBT HOPE clinical research study.