INFORMATION AND CONSENT FORM

Study Title: A Phase 1/2, Open-label, Single-arm Study to Assess the Safety, Tolerability, and Efficacy of ST-400 Autologous Hematopoietic Stem Cell Transplant for Treatment of Transfusion-dependent Beta-thalassemia (TDT)

Study #: ST-400-01

Sponsor: Sangamo Therapeutics, Inc.;

Study Doctor: <Investigator Name>
<Site name>
<Site address>

Telephone Number: <XXX-XXX-XXXX>

Only for sites located in California - otherwise please delete:

For California participants: Before you read this consent form, you should read and sign a copy of the California Experimental Subject's Bill of Rights. Ask the study staff for a copy of this document if you haven't already received one.

The study doctor wants to know if you would like to be part of a research study.

If you have any questions about or do not understand something in this form, you should ask the study doctor or study staff. You should also discuss your participation with anyone you choose to better understand this study and your options.

You may or may not personally benefit from being in this study. However, by serving as a subject, you may help us gain new information that will benefit beta-thalassemia patients in the future.

Your participation is voluntary which means you can choose whether or not you want to participate. If you choose not to participate, you will not lose any benefits that you will have otherwise. If you decide to participate, you will be asked to sign the end of this form.

If you join this study, you should continue to see your regular doctor and any specialists that help you care for and manage your treatment for transfusion-dependent Beta-thalassemia (TDT).

WHY AM I BEING ASKED TO PARTICIPATE?

You are being asked to participate in this experimental research study because you have transfusion-dependent beta-thalassemia, are 18-40 years old, and needed at least eight red blood cell transfusions per year on average, over the last 2 years. This study is experimental. Experimental means that this is the first study in which the investigational product the study doctor will give to you will be given to human volunteers. The study team will be doing ongoing tests and exams to see if it is safe and if it has any benefit to people with beta-thalassemia.

A company called Sangamo Therapeutics, Inc. is studying beta-thalassemia and is trying to find new ways to treat this disease. Sangamo has asked the study doctor and the research team to conduct the study for them.

Sanofi Genzyme ("Sanofi") is collaborating with Sangamo for this study. Sanofi may conduct additional exploratory research to evaluate some of your biological samples collected in this study.

WHAT IS THIS STUDY ABOUT?

This research study is being done to study if the investigational product being studied is effective for beta-thalassemia. Beta-thalassemia is a rare inherited disorder in which there is a defect in the adult type of hemoglobin in your red blood cells. Hemoglobin carries oxygen to all tissues of the body, including the brain, heart, lungs and kidneys. It is possible to replace the abnormal red blood cells in beta-thalassemia patients with normal red blood cells by a bone marrow transplant from a matched donor. We know this from recent studies where bone marrow from a tissue matched donor has replaced abnormal blood cell production with normal blood cell production.

The investigational product to be tested in the present study is known by its code name ST-400.

An "investigational product" is an agent that is being tested and is not approved for sale in the United States. ST-400 is made from stem cells collected from a person's own blood stream. Stem cells are the primitive cells that make all your blood cells. The stem cells contain the genetic code for making hemoglobin. A portion of that code decides which kind of hemoglobin is made: adult or fetal (baby hemoglobin). Sangamo has found a way to change this code to make more fetal hemoglobin, which works like adult hemoglobin in your body.

The main purpose of this research study is to see if ST-400 is safe and well tolerated, and to find out if there are any side effects of ST-400.

People enrolled into research studies are referred to as "subjects". In this study, up to 6 subjects who are at 18-40 years of age and who have beta-thalassemia will be enrolled. All study subjects will receive ST-400. The first 3 subjects will receive ST-400 sequentially (one after another). The first subject will receive ST-400 and will be monitored for safety. If there are no significant concerns with the first subject, then the second subject will receive ST-400 and will be monitored for safety. If there are no significant concerns with the second subject, the third subject will be given ST-400 and will be monitored for safety. Depending upon the outcome in the first 3 subjects who receive ST-400, the last three subjects may receive ST-400 all at the same time.

The study will be overseen by an independent group of medical experts in beta-thalassemia and bone marrow transplantation.

Before you decide whether or not to join the study, please read all the information in this consent form. Feel free to ask questions to understand your rights and protections. The choice to take part in this study is completely voluntary.

HOW DOES ST-400 WORK?

There is a gene in your stem cells that stops production of a type of hemoglobin called fetal hemoglobin. The body makes fetal hemoglobin until soon after birth, at which time the gene stops production of fetal hemoglobin. This gene is called “BCL11A”.

Thalassemia patients cannot make adult hemoglobin and develop severe anemia (not enough red blood cells in the blood, leading to weakness or tiredness) after the fetal hemoglobin is no longer made. The purpose of this treatment is to modify the BCL11A gene to allow the body to continue to make fetal hemoglobin. This is based on the finding that some patients with beta-thalassemia who naturally make a lot of fetal hemoglobin have less severe disease and require fewer transfusions.

If you agree to participate in the study, you will undergo a blood stem cell mobilization and collection procedure called apheresis to collect stem cells from your blood. The medications used for mobilization coax the stem cells into the blood stream from their usual residence, the bone marrow. Those cells will then be shipped to a lab where the DNA (the genetic code) of those cells will be precisely changed using a technology called zinc finger nucleases (ZFN).

These ZFNs will make a specific cut and modify the DNA at a targeted location in the BCL11A gene to reduce its effectiveness. The goal is to reduce the effectiveness of the BCL11A gene without affecting other DNA, so your stem cells can make fetal hemoglobin. After the DNA is modified, the stem cells will be grown in the lab, tested to make sure they are safe and then shipped back to your doctor. Once your doctor receives the gene edited stem cells (ST-400), you will be admitted to the hospital and undergo chemotherapy. This is to remove the existing stem cells from your body. The treated gene edited stem cells will then be put back into you via a catheter so they can re-populate your bone marrow.

IS THERE ANYTHING ELSE I CAN DO FOR MY BETA-THALASSEMIA ILLNESS?

You do not have to be in this study to get help for your beta-thalassemia illness. If you do not want to be in this study you can:

- Continue your current transfusion therapy and supportive care
- Participate in a different study
- Undergo an allogeneic bone marrow transplant. An allogeneic bone marrow transplant from a donor is a complex medical procedure that your study doctor can describe.

You should discuss your alternatives to participating in this research study with the study doctor or study staff. In addition, you may discuss your options with your regular health care provider.

HOW LONG WILL I BE IN THE STUDY?

If you decide to be in this study and the study doctor says you can be in the study, your participation in the study could last about 3.5 years (up to 182 weeks) including the following periods:

Screening, Mobilization, Apheresis and Conditioning: About 26 weeks for screening, blood stem cell mobilization and collection, and chemotherapy conditioning.

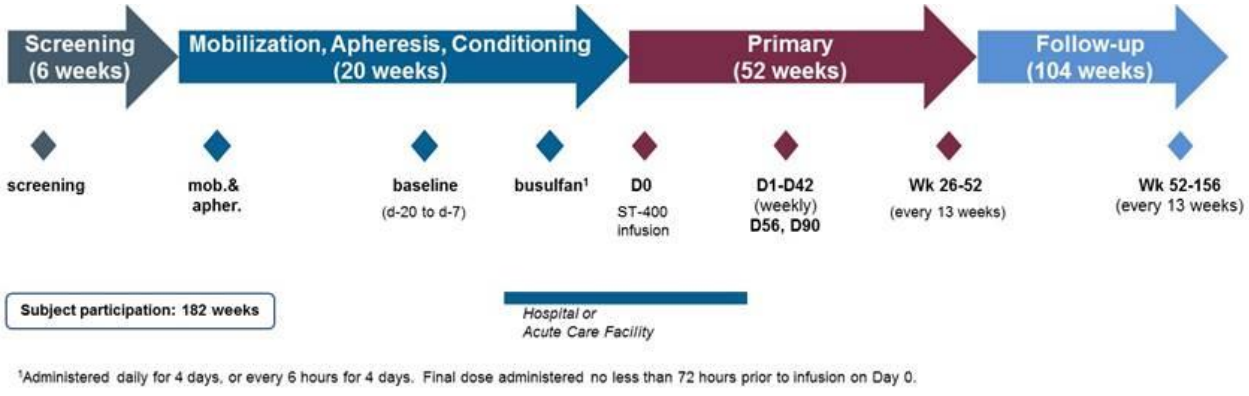
Primary Study Period: Starting with ST-400 infusion (Day 0), and followed by 12 months (52 weeks) of follow-up after treatment.

Follow-up Study Period: An additional 24 months (104 weeks) of follow-up after completion of the primary study period.

You will have to come to the clinic approximately 15 times (not including the 2-4 week hospitalization period) or more during the study. The number of study visits you have to complete depends on whether or not you need to have more than one procedure to collect your blood cells. Depending on your health, you may also have to come to the clinic for additional follow-up visits. The study staff will tell you when to come in for your study visits.

You should ask the study staff how long your visits will last. At the end of this study, you will be asked to participate in a separate long-term follow-up study to monitor for safety of ST-400. The long-term follow-up study is also voluntary. You don't have to be part of this long-term follow-up study if you don't want to participate.

WHAT WILL HAPPEN DURING THIS STUDY?



[1]Administered daily for 4 days, or every 6 hours for 4 days. Final dose administered no less than 72 hours prior to infusion on Day 0.

**** D= Day, wk= weeks**

If you decide to be in this study, you will first enter the study's screening period:

Screening Period: About 6 weeks in duration

During the screening period, your medical history, including medications you are currently taking and your blood transfusion history will be reviewed, and the following tests and procedures will be evaluated over one or more screening visits:

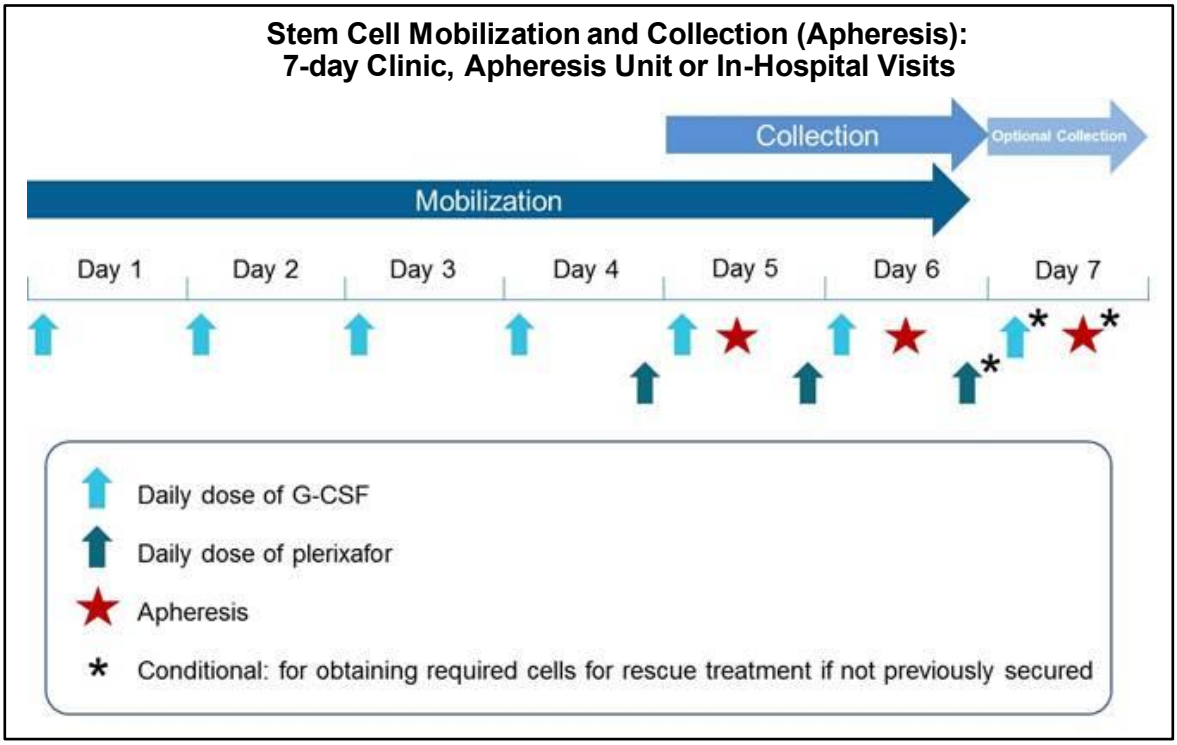
- Physical exam
- Collect age, gender, race, and ethnicity information
- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure
- Height and weight

- Blood and urine tests:
  - To see if you have been exposed to viruses such as HIV (human immunodeficiency virus, the virus that causes AIDS), hepatitis and other infections. You will not be able to be in the study if you have a diagnosis of HIV or active hepatitis B or C infections. Depending on state law, you may have to give consent before HIV and hepatitis B and C testing can start and you will be asked to sign a separate form. The HIV and hepatitis tests are confidential between you and your doctor, and the study doctor or study staff will not share your results outside this study unless state law requires it. If required by state law, the study doctor or study staff may report positive test results to the local health department (Infectious Disease Panel)
  - A blood test to check your blood count, serum chemistries, and liver function (Clinical Labs)
  - Urine test to check kidney function (Clinical Labs)
  - Blood pregnancy test: For females of childbearing potential to find out if you are pregnant
  - A blood test to count your fetal hemoglobin levels (HbF Quantification)
  - Genetic blood test to check your hemoglobin genes (Hemoglobin Analysis)
  - Blood test to check the condition of specific organs, such as pancreas and adrenal glands (Endocrine Lab Testing)
  - Blood test to determine how your body is making blood and storing iron (Thalassemia-related Disease Biomarkers)
- Heart function tests including an electrocardiogram (ECG, to record the rhythm of the heart) and echocardiogram (ECHO, a picture of the heart and its structures using sound waves, or ultrasound)
- Chest x-ray to look at the image of the chest and internal organs like lung and heart
- Lung function tests: a group of tests that measure breath or air moving in and out of your lungs during various respiratory (breathing) maneuvers and the ability of your lungs to move oxygen into the body
- Liver and cardiac (heart) magnetic resonance imaging (MRI): MRI is a test that uses a magnetic field and pulses of radio wave energy to make pictures of organs and structures inside the body. These images can help to determine iron levels within the heart and liver.
- Liver biopsy (may not be required at screening, your doctor will tell you if this is needed): under anesthesia, a needle will be inserted into your liver to collect a tissue sample to determine if it is safe to proceed with the conditioning therapy
- A short health form to collect information about your overall health and assess the level of your function to perform daily tasks (SF-36 Survey)
- You will be asked about your health since beginning the study

The total amount of blood drawn during the screening period is approximately 2 tablespoons.

Even if you have passed all of the screening tests, there is a possibility that you will not be enrolled in the study. If you are told by your physician that you may enroll and wish to continue your participation, you will begin the following study periods. Depending upon how much time has passed between your initial screening studies and when you are scheduled to begin chemotherapy conditioning, your doctor may need to repeat some of the screening studies at the Baseline Assessment to confirm that you are healthy to proceed to chemotherapy conditioning (see Baseline Assessment).

Mobilization, Apheresis, Conditioning (about 20 weeks)



The following tests and procedures will be performed within one week before you are given the first dose of medicine to mobilize your stem cells:

- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs)
- Urine test to check kidney function (Clinical Labs)
- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure
- Blood pregnancy test: For females of childbearing potential to find out if you are pregnant
- Review and record all medications you are taking and record the number of blood transfusions since your last visit
- You'll be asked about your health since the last visit
- Physical exam

- Weight

The total amount of blood drawn for lab tests during the first day of the Stem Cell Mobilization and Collection (Apheresis) period is approximately 1 tablespoon.

Stem Cell Mobilization: You will be treated with two medications: granulocyte colony stimulating factor (G-CSF) and plerixafor. This combination of medications will mobilize or move your stem cells from your bone marrow into your bloodstream. G-CSF will be given as an injection under your skin or in your vein every day for 6 or 7 days. Plerixafor will be given as an injection under your skin or in your vein as a daily dose for 2 or 3 days. The timing of the medicines, G-CSF and plerixafor, will be determined by your doctor and explained to you. Daily blood tests may be obtained while you are receiving G-CSF and plerixafor to monitor your response to these medications.

Blood stem cell collection (apheresis): This will be done on Days 5 and 6 (and possibly on Day 7) following the start of stem cell mobilization. A special hollow tube called a central venous catheter (CVC) will be inserted into a vein in your chest, arm or neck by a surgeon. The purpose of inserting the central venous catheter is to have a way to collect the blood stem cells from the bloodstream. Surgery with anesthesia is needed to place a central venous catheter for apheresis collection. In addition, you may need to remain in the hospital as long as the apheresis catheter is in place.

Apheresis or stem cell collection uses a specialized machine that circulates your blood outside your body. It removes the blood stem cells and returns the remaining fluid and other blood cells to your bloodstream. You will be watched very carefully during this process for any side effects. You will have blood drawn to make sure your body is not having any side effects from the procedure. It is possible you may receive a blood transfusion before or during the procedure. Your blood pressure and heart rate will be taken many times during this procedure. The procedure will be performed in a clinic, hospital or apheresis unit. Each apheresis procedure will take about 6-8 hours to complete. If certain kinds of central venous catheters are used, you may need to stay in the hospital overnight to care for that catheter.

The collection of blood stem cells will be divided into 2 portions: One portion will be shipped to the manufacturer to make the ST-400 investigational product. The other portion will be frozen and stored by your doctor to be given to you later, only if needed as a rescue treatment.

Extra days of collection: It is possible that if not enough cells are collected on Days 5 and 6 that another day of apheresis will be needed on Day 7 to collect cells to freeze and store.

Repeat of mobilization and apheresis: It is also possible that you may be asked to return to repeat the stem cell mobilization and collection procedure, at least 2 weeks after the first procedure. If stem cell mobilization and collection is repeated, the tests and assessments listed above will need to be repeated.

Cell disposal: If your collected stem or gene-edited cells (ST-400) cannot be used, or if you decide to leave the study before you receive ST-400, you may provide your consent for the cells or ST-400 to be used for research purposes. If you do not provide that consent, the cells will be destroyed.

Baseline Assessments: Clinic visit

Assessments will be performed approximately two weeks prior to chemotherapy conditioning with busulfan. The following tests and procedures are performed at that visit:

- Review and record all medications you are taking and record the number of blood transfusions since your last visit
- You'll be asked about your health since the last visit
- Physical exam
- Weight
- A short health form to collect information about your overall health and assess the level of your function to perform daily tasks (SF-36)
- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure
- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs)
- Urine test to check kidney function (Urinalysis)
- Blood pregnancy test: For females of childbearing potential to find out if you are pregnant (within 1 week prior to first busulfan infusion)
- An electrocardiogram (ECG) to record the rhythm of the heart
- Genetic blood tests to determine the effectiveness of ST-400 (BCL11A Gene Modification)
- A blood test to count your fetal hemoglobin levels (HbF Quantification)
- Blood test to look at function of the immune system before ST-400 treatment (Immunological Assays)
- Blood test to determine how your body is making blood and storing iron (Thalassemia-related Disease Biomarkers)
- Blood test to determine the percent of your circulating red blood cells that make fetal hemoglobin (%F cells)
- Blood test to check the condition of specific organs, such as pancreas and adrenal glands (Endocrine Lab Testing)
- DXA: a type of imaging test to determine the health of your overall bone mineral density
- Bone marrow aspiration: A test to determine the health of your bone marrow
- Genetic blood test to be conducted only if your doctor finds possible evidence of cancer. (Sampling for Potential Retrospective Analysis in the Event of Hematological Malignancy)

The total amount of blood drawn at baseline is approximately 4 tablespoons. The total amount of bone marrow aspirate collected at the baseline is approximately one (1) teaspoon

Chemotherapy Conditioning, ST-400 Infusion and Follow up:

The ST-400 investigational product made from the blood stem cells collected from you and sent to the manufacturer will be tested before it can be given to you. It will take about 6-8 weeks from the time your cells are collected to the time those tests are complete.

If the ST-400 investigational product passes all the tests, you will be scheduled for the chemotherapy conditioning treatment. You will stay in the hospital or acute care clinic for approximately 2-4 weeks total for the following: chemotherapy conditioning, modified blood stem cell (ST-400) infusion and follow-up tests as described below. To help with giving medicines, blood transfusions and getting blood for lab tests, a central venous catheter will be placed before ST-400 infusion. This is a hollow tube that is inserted by a surgeon or radiologist in the operating room.

Chemotherapy conditioning:

Chemotherapy conditioning is needed to make room for the new ST-400 stem cells. Chemotherapy conditioning will get rid of most of your blood and bone marrow stem cells. This will temporarily cause low counts of white blood cells, red blood cells, and platelets. ST-400 is meant to re-populate your bone marrow and help your body produce new blood cells to replace the destroyed cells.

The chemotherapy medicine used is busulfan. Busulfan has many different side effects, which are discussed in the risk section.

Busulfan will be given through your central venous catheter over 4 days when you are in the hospital or acute care clinic. Busulfan may be given once daily (a total of 4 doses over 4 days), or 4 times daily (a total of 16 doses over 4 days). Your last dose of busulfan will be followed by at least 3 “rest days” (at least 72 hours) when you will not get any chemotherapy medicine. Following this rest period, ST-400 will be infused, as described below.

Hospitalization	**Treatment**
4 consecutive days	Busulfan (given IV once daily or 4 times daily; ask your doctor)
At least 72 hours	Rest
Day 0	ST-400 infusion

In addition, the following tests and procedures will be performed daily:

- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure
- Review and record all medications you are taking and record the number of blood transfusions since your last visit
- You’ll be asked about your health since the last visit
- Physical exam

Samples for central laboratory analysis ("Clinical Labs") should be collected at least once during conditioning, with timing at the discretion of the study doctor:

- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs)
- Urine test to check kidney function (Clinical Labs)

The total amount of blood drawn during chemotherapy conditioning is approximately 1 tablespoon.

ST-400 Infusion and Follow-up Period

You will stay in the hospital or acute care clinic for the ST-400 infusion and remain there for at least 2-4 weeks, and possibly longer. Your doctor will determine when it is safe for you to go home. While you are in the hospital or acute care clinic, you will be carefully watched for signs of infection and other problems, and blood tests will be done daily at first, then weekly. Additional blood tests, medicines, and procedures may be required if there are any problems.

After leaving the hospital or acute care clinic, you will need to visit the study clinic for check-ups, blood tests and urine tests to make sure that you are doing well medically.

If you and the study doctor agree, select visits procedures post ST-400 infusion may be carried out off-site by a home health care provider at your home or another safe location (such as your workplace or hotel) while there are social distancing and/or travel restrictions in place. During such visits, a mobile research nurse will visit you at an agreed upon off-site location and complete procedures according to the protocol.

Your health and personal data (name, address, telephone number, etc.) will be collected by the off-site nursing provider and will used by the home health care provider personnel, as well as any samples couriers, as necessary only for the purpose of carrying out the off-site visits.

You will be required to confirm your permission for the home health service provider nurse to perform assessments and to collect blood and urine samples, and access and use your personal data associated with this study in the end of this consent form.

ST-400 infusion (Day 0)

ST-400 will be given through your central venous catheter.

Vital signs (temperature, heart rate, breathing, and blood pressure) will be monitored before, during, and after the infusion.

The following tests and procedures will be performed:

- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs)
- Urine test to check kidney function (Clinical Labs)
- Review and record all medications you are taking and record the number of blood transfusions since your last visit
- You'll be asked about your health since the last visit

- Physical exam
- An electrocardiogram (ECG) to record the rhythm of the heart
- A blood test to count your fetal hemoglobin levels (HbF Quantification)
- Blood test to determine how your body is making blood and storing iron (Thalassemia-related Disease Biomarkers)

The total amount of blood drawn at Day 0 is approximately 2 tablespoons.

Days 7, 14, 21, 28, 35, 42, 56, and 90; and Weeks 26, 39, and 52

The following tests and procedures are performed:

- Review and record all medications you are taking and record the number of blood transfusions since your last visit
- You'll be asked about your health since the last visit
- Physical exam
- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure
- A short health form to collect information about your overall health and assess the level of your function to perform daily tasks (SF-36) (weeks 26 and 52 only)
- Lung function tests: a group of tests that measure breath or air moving in and out of your lungs during various respiratory (breathing) maneuvers and the ability of your lungs to move gases such as oxygen from the atmosphere into the body (week 52 only)
- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs)

  Urine test to check kidney function (Clinical Labs)
- Urine pregnancy test: For females of childbearing potential to find out if you are pregnant (days 28, 56, and 90; weeks 26, 39 and 52 only)
- An electrocardiogram (ECG) to record the rhythm of the heart (day 28; weeks 26 and 52 only)

  Genetic blood tests to determine the effectiveness of ST-400 (BCL11A Gene Modification) (days 14, 28, 42, 56, and 90; weeks 26, 39 and 52 only) (Subset Analysis of Gene Modification) (week 39 only)
- A blood test to count your fetal hemoglobin levels (HbF Quantification) (days 14, 28, 42, 56, and 90; weeks 26, 39 and 52 only)
- Blood test to look at function of the immune system after ST-400 treatment (day 90; week 52 only)
- Blood test to determine how your body is making blood and storing iron (Thalassemia-related Disease Biomarkers) (day 90; weeks 26, 39 and 52)
- Blood test to determine the percent of your circulating red blood cells that make fetal hemoglobin (%F cells) (days 28, 56, and 90; weeks 26 and 52)

- Blood test to check the condition of specific organs, such as pancreas and adrenal glands (Endocrine Lab Testing) (week 52 only)
- Echocardiogram (ECHO) (week 52 only)
- Liver and cardiac (heart) magnetic resonance imaging (MRI). MRI is a test that uses a magnetic field and pulses of radio wave energy to make pictures of organs and structures inside the body. These images can help to determine iron levels within the heart and liver. (week 52 only)
- DXA: a type of imaging test to determine the health of your overall bone mineral density (week 52 only)
- Bone marrow aspiration: A bone marrow aspirate will be collected for tests to determine the health of your bone marrow and to assess the persistence and effect of ST-400 edited cells in the bone marrow including research on sub-groups of the edited cells (day 90; either week 26 or week 39; week 52 only).

  Genetic blood test to be conducted only if your doctor finds possible evidence of cancer. (Sampling for Potential Retrospective Analysis in the Event of Hematological Malignancy) (day 90; weeks 26, 39 and 52 only)

The approximate total amount of blood and bone marrow aspirate drawn for each visit is in the table below:

Visit	Amount of blood in tablespoons	Amount of bone marrow aspirate in teaspoons
Days 7-28	5	0
Days 35-56	4	0
Day 90	4	2
Week 26	3	2
Week 39	5	2
Week 52	4	2

Follow-up Study Period

Weeks 65, 78, 91, 104, 117, 130, 143 and 156/End of Study Visit following ST-400 infusion

You will be evaluated approximately every 3 months (13 weeks) during the follow-up period.

The following test and procedures will be performed:

- You'll be asked about your health since the last visit

- Review and record all medications you are taking and record the number of blood transfusions since your last visit
- Physical exam
- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure
- A short health form to collect information about your overall health and assess the level of your function to perform daily tasks (SF-36) (weeks 104 and 156 only)
- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs).
- Urine test to check kidney function (Clinical Labs).
- Heart function tests including an electrocardiogram (ECG) and echocardiogram (ECHO) (weeks 104 and 156 only)
- Genetic blood tests to determine the effectiveness of ST-400 (BCL11A Gene Modification) (weeks 78, 104,130 and 156 only) (Subset Analysis of Gene Modification) (weeks 91 and 143 only).
- A blood test to count your fetal hemoglobin levels (HbF Quantification).
- Blood test to look at function of the immune system after ST-400 treatment (Immunological Assays) (weeks 65 and 117 only).
- Blood test to determine how your body is making blood and storing iron (Thalassemia-related Disease Biomarkers) (weeks 78,104,130, and 156 only).
- Blood test to check the condition of specific organs, such as pancreas and adrenal glands (Endocrine Lab Testing) (weeks 65 and 117 only).
- Liver and cardiac (heart) magnetic resonance imaging (MRI). MRI is a test that uses a magnetic field and pulses of radio wave energy to make pictures of organs and structures inside the body. These images can help to determine iron levels within the heart and liver. (weeks 104 and 156 only)
- DXA: a type of imaging test to determine the health of your overall bone mineral density (weeks 104 and 156 only)
- Bone marrow aspiration: A bone marrow aspirate will be collected for tests to determine the health of your bone marrow and to assess the persistence and effect of ST-400 edited cells in the bone marrow including research on sub-groups of the edited cells (week 104 only).
- Genetic blood test to be conducted only if your doctor finds possible evidence of cancer. (Sampling for Potential Retrospective Analysis in the Event of Hematological Malignancy) (weeks 78, 104, 130, and 156 only).

An End of Study (EOS) visit will be conducted at Week 156. At the EOS visit you may be asked to participate in the Long-Term Follow-up Study. If you choose to participate, you will be asked to review and sign a separate informed consent prior to participating in the Long-Term Follow-up Study.

The approximate total amount of blood and bone marrow aspirate drawn for each visit is in the table below:

Visit	Amount of blood in tablespoons	Amount of bone marrow aspirate in teaspoons
Week 65	2	0
Week 78	3	0
Week 91	3	0
Week 104	3	2
Week 117	2	0
Week 130	3	0
Week 143	3	0
Week 156	3	0

Early Termination Visit (ETV)

If you decide to withdraw from the study after receiving ST-400, the gene modified cells cannot be removed from your body and can potentially persist indefinitely. By withdrawing from the study, you are withdrawing from further monitoring.

If you are discontinued from the study early or withdraw from the study, you will be asked to return to the clinic for an ETV.

The following tests and procedures may be performed at the ETV:

- Review and record all medications you are taking and record the number of blood transfusions since your last visit.
- You'll be asked about your health since the last visit.
- Physical exam
- Vital signs: temperature, heart rate, oxygen level in blood, breathing (respiratory rate), and blood pressure.
- A short health forms to collect information about your overall health and assess the level of your function to perform daily tasks (SF-36)
- Blood draw for blood count, serum chemistries, and liver function (Clinical Labs).
- Urine test to check kidney function (Clinical Labs).
- Urine pregnancy test: For females of childbearing potential to find out if you are pregnant.
- Heart function tests including an electrocardiogram (ECG) and echocardiogram (ECHO)
- Genetic blood tests to see how your stems cells genetic material has changed before and after ST-400 (BCL11A Gene Modification).
- A blood test to count your fetal hemoglobin levels (HbF Quantification).

- Blood test to look at function of the immune system before and after ST-400 treatment (Immunological Assays).
- Blood test to determine how your body is making blood and storing iron (Thalassemia-related Disease Biomarkers).
- Blood test to check the condition of specific organs, such as pancreas and adrenal glands (Endocrine Lab Testing).
- Liver and cardiac (heart) magnetic resonance imaging (MRI). MRI is a test that uses a magnetic field and pulses of radio wave energy to make pictures of organs and structures inside the body. These images can help to determine iron levels within the heart and liver.
- Bone marrow aspiration: A bone marrow aspirate will be collected for tests to determine the health of your bone marrow and to assess the persistence and effect of ST-400 edited cells in the bone marrow including research on sub-groups of the edited cells.
- Genetic blood test to be conducted only if your doctor finds possible evidence of cancer. (Sampling for Potential Retrospective Analysis in the Event of Hematological Malignancy).

The total amount of blood drawn at the early termination visit (ETV) is approximately 4 tablespoons. The total amount of bone marrow aspirate collected at the ETV is approximately 2 teaspoons.
All of the visits are summarized in the table below:

Study Period/Visit	**Visit Type**
Screening (approximately 6 weeks)	Clinic Visit
Stem cell mobilization and apheresis	Clinic, Apheresis Unit, or Hospital Visit
Baseline	Clinic visit
Busulfan Chemotherapy treatment	Hospitalization
ST-400 Infusion and Observation Day 0 through Day 28	Hospitalization for infusion and observation
Days 35, 42, 56, and 90 Weeks 26, 39, and 52	Clinic visits
Follow-up Study Period (104 weeks) Week 65, 78, 91,104,117,130,143 and 156	Every 13 weeks clinic visits

Unmodified blood stem cell infusion (Rescue Treatment):

After receiving the ST-400, you will be monitored closely for the re-population of your bone marrow (engraftment) by ST-400. Engraftment is when the new modified blood stem cells start to grow and show up in your blood. In the event no blood stem cells re-grow after the

chemotherapy treatment, you will receive the rescue treatment with your own unmodified stem cells.

While you are in the study, you must:

- Follow the instructions you are given.
- Come to the clinic for visits with the study doctor or study staff.
- Tell the study doctor or study staff about any changes in your health or the way you feel.
- Tell the study doctor or study staff if you want to stop being in the study at any time. If you decide to not receive ST-400, but you have received busulfan, you will need to remain in the hospital and receive the rescue treatment with your own unmodified stem cells before leaving the study.
- See your regular doctor for your medical care.

After the study is over, you should talk to the study doctor and your regular doctor about the long-term follow-up study and your future treatment for beta-thalassemia.

WHAT ELSE SHOULD I KNOW ABOUT THE STUDY PROCEDURES?

You may have to sign a separate consent form before you have some of the procedures listed above.

Your regular medical care might include some of the study tests and procedures (these are called "standard of care" tests and procedures). The study doctor or a member of the study staff can answer any questions you may have about which tests and procedures are not part of your regular medical care (these are called "research" tests and procedures). Ask the study doctor or study staff for the estimated recovery time of your participation in this study.

WILL BEING IN THIS STUDY HELP ME?

You should not expect to receive any benefit from this study. This study is being done to see if ST-400 is tolerated and to check for any potential side effects. ST-400 may not help your beta-thalassemia. Your beta-thalassemia might not get better or may even get worse.

ARE THERE RISKS TO ME IF I AM IN THIS STUDY?

All experimental products and drugs carry a risk of side effects. Ask the study doctor or study staff if you have questions about the signs or symptoms of any side effects that you read about in this consent form. Below is a list of the risks that the investigators think are possible with this study:

KNOWN RISKS THAT ARE DUE TO STEM CELL TRANSPLANT PROTOCOL

Risks Associated with Stem Cell Mobilization and Apheresis

- Hematoma (bruising) or infection at the site of the needle insertion. Discomfort at the site of the needle, which will be in place for several hours, may occur.

- The medicines used for apheresis may cause tingling sensations around the mouth, hands, or other parts of the body due to low calcium levels in the blood. Muscle cramps, nausea, and/or vomiting may occur, but this does not happen often. You may need to receive calcium. There is a small risk that the anticoagulation will lead to bleeding.
- Rarely, blood pressure may fall and lightheadedness or fainting may occur. Seizures may also occur rarely. Shock, irregular heartbeats and death have been reported, but are uncommon and usually due to an underlying illness rather that the apheresis procedure.
- There is a possibility of the red blood cells rupturing (called hemolysis) due to an issue with the machine; however, this is rare and is carefully monitored. In the event of hemolysis, the procedure would be discontinued.
- Loss of red blood cells due to leakage or breakage of the plastic tubing or containers may occur and thus prevent the return of the red blood cells to your body.
- Although the machine is equipped with an air detector to prevent air bubbles, there is a remote possibility of an air bubble entering your body. The consequences of the air bubbles entering your body could be severe and result in death.
- The platelet count may fall during the apheresis procedure. This may result in a bleeding risk. Transfusion of platelets may be required.

Some medications are not permitted during the apheresis procedure. Your doctor will review with you what is safe and not safe. If you are currently taking these medications and stop your regular medication to be in the study, your health might get worse. Please tell the study doctor or study staff right away if you have any problems when you stop taking your regular medication.

Risks Associated with G-CSF and Plerixafor

Possible Side Effects of G-CSF (Filgrastim):

COMMON, SOME MAY BE SERIOUS In 100 people receiving Filgrastim, more than 20 and up to 100 may have:
• Nose bleed • Anemia which may require transfusion • Pain • Diarrhea • Fever • Tiredness • Hair loss

OCCASIONAL, SOME MAY BE SERIOUS In 100 people receiving Filgrastim, from 4 to 20 may have:
• Fluid in the organs which may cause low blood pressure, shortness of breath, swelling of ankles • Damage to the lungs which may cause shortness of breath • Internal bleeding which may cause coughing up blood • Cough

OCCASIONAL, SOME MAY BE SERIOUS In 100 people receiving Filgrastim, from 4 to 20 may have:
• Swelling or tenderness of vessels • Headache

RARE, AND SERIOUS In 100 people receiving Filgrastim, 3 or fewer may have:
• Rupture of the spleen causing sudden or severe pain in the left side of abdomen spreading up to your shoulder

Possible Side Effects of Plerixafor

COMMON, SOME MAY BE SERIOUS In 100 people receiving Plerixafor, more than 20 and up to 100 may have:
• Diarrhea • Nausea • Swelling and redness at the site of injection • Tiredness • Headache

OCCASIONAL, SOME MAY BE SERIOUS In 100 people receiving Plerixafor, from 2 to 20 may have:
• Joint and/or muscle pain • Dizziness • Vomiting, constipation, bloating, passing gas, pain in belly, heartburn • Difficulty sleeping • Increased sweating • Dry mouth, Numbness of the mouth • Rash

RARE, AND SERIOUS In 100 people receiving Plerixafor, 1 or fewer may have:
• Rupture of the spleen causing sudden or severe pain in the left side of abdomen spreading up to your shoulder • Allergic reaction which may cause rash, low blood pressure, wheezing, shortness of breath, swelling of the face or throat

Please contact the study doctor or study staff right away if you have any side effects and any other problems with your health or the way you feel during the study, whether or not you think these problems are related to the study.

<u>Risks Associated with Busulfan Chemotherapy</u>

You will be given medication before and after busulfan to reduce any side effects of nausea or vomiting, even if you have no symptoms. You will also be given medications to prevent seizures. The medications may be given in the same central venous catheter or you may be asked to swallow the medication.

Possible Side Effects of Busulfan:

COMMON, SOME MAY BE SERIOUS In 100 people receiving Busulfan, more than 20 and up to 100 may have:
• Anemia which may require blood transfusions • Infection, especially when white blood cell count is low • Abnormal heartbeat • Constipation, diarrhea, heartburn, nausea, vomiting, loss of appetite • Sores in mouth which may cause difficulty swallowing • Chills, fever • Pain • Swelling of the body • Damage to the liver • Allergic reaction which may cause rash, low blood pressure, wheezing, shortness of breath, swelling of the face or throat • Bruising, bleeding • Dizziness, headache, tiredness • Difficulty sleeping • Worry • Cough, stuffy nose • Rash • High blood pressure which may cause blurred vision

OCCASIONAL, SOME MAY BE SERIOUS In 100 people receiving Busulfan, from 4 to 20 may have:
• Cloudiness of the eye, visual disturbances • Seizure • Blood in urine • Loss or absence of sperm • Menopause • Internal bleeding which may cause coughing up blood • Damage to or scarring of the lungs

RARE, AND SERIOUS In 100 people receiving Busulfan, 3 or fewer may have:
• Fluid around heart • Heart failure which may cause shortness of breath, swelling of ankles, and tiredness • Cancer of bone marrow caused by chemotherapy • Low blood pressure which may cause feeling faint

Damage to the vital organs in the body: Busulfan can cause problems in the heart, lungs, liver, intestine, kidneys and bladder, or brain. Lung problems can be the result of infections or chemotherapy. Some patients have become jaundiced (yellowish skin), have liver function abnormalities, fluid retention, abdominal swelling, and abdominal pain. If organ damage symptoms are severe, you may have to stay in the hospital longer or be re-hospitalized after

transplant. Although many patients recover completely, these complications may cause permanent damage or even death.

Busulfan will lower your blood counts. These cells include your platelets (increased risk of bleeding), white blood cells (increased risk of infections), and red blood cells (increased anemia leading to weakness or tiredness). Patients may require transfusion of platelets and/or red blood cells.

Impact on reproductive hormone function and sexuality: High doses of chemotherapy, including busulfan, can cause sterility (inability to have children) and decreased hormone levels.

- **Gamete and Embryo Cryopreservation:**

  Busulfan is associated with a risk of harm to testis or ovary and infertility, which means that it is possible that you will be unable to have children after the treatment. Therefore, before receiving Busulfan you will be offered the opportunity to have your gametes (male sperm or female oocytes [immature egg cells]) collected and stored for future use. For some women and depending upon your circumstances, your doctor might also advise in vitro fertilization of your oocytes (immature egg cells) immediately following collection and the storage of embryos that may result. The Sponsor will pay for the initial collection and storage of gametes and/or immediate creation and storage of embryos for the duration of the study. You should discuss these options with your doctor as some procedures may need to be completed at least one month before the blood stem cell treatment.

  In order to try to achieve pregnancy in the future with the banked sperms, oocytes, or embryos, you (or your partner) may need to have artificial reproductive treatment, which does not always work. Please ask your doctor about the potential risks and costs and timing of the treatment and procedure.

RISKS THAT ARE DUE TO THE EXPERIMENTAL APPROACH

Risks of ST-400

This is the first time ST-400 is being given to humans, and side effects are not fully known. Possible side effects are listed below.

- Serious allergic reaction in association with ST-400 administration has been reported. This reaction included symptoms of abnormal taste, cold sensation, feeling of heaviness and chest tightness, difficulty breathing, slow heart rate and low blood pressure.

  Other symptoms of an allergic reaction may include rash, dizzy or lightheaded feeling from a drop in blood pressure, a feeling of dread, wheezing, swelling of the mouth, throat or eyes, fast pulse, sweating and inability to breathe without assistance. This type of reaction may be severe and in rare cases could result in death.

  An ingredient called DMSO is present in ST-400 which helps frozen cells to thaw. Possible side effects that are associated with DMSO include abnormal taste, allergic reaction, slow heart rate, low blood pressure, and flu-like symptoms (temporary fever, chills, and/or nausea).

- Potential risk of cancer. ST-400 makes changes to the DNA in your blood stem cells to boost fetal hemoglobin levels. ST-400 also causes a very low level of DNA changes at unintended places, which could potentially increase the risk of cancer. Animal studies with ST-400 have not shown increased risk of cancer. Unintended DNA changes by ST-400 were extremely rare and were not found in cancer-causing genes. However, we do not yet know the risk of cancer from ST-400 in humans, therefore, it is a potential risk.
- Failure to engraft ST-400 into your bone marrow, which might require blood stem cell infusion (rescue) with your original unmodified stem cells. In this case you will recover your blood counts but will still have thalassemia.

There may be side effects that are not known at this time. Please tell the study doctor if you have any side effects or any other problems with your health or the way you feel during the study, whether or not you think they are related to ST-400 or the study procedures.

To reduce any side effects from the ST-400 infusion, you may be given medications such as corticosteroids (like hydrocortisone), acetaminophen (Tylenol), and/or diphenhydramine hydrochloride (Benadryl). You should ask the study doctor about the risks of using medication you receive to help treat or prevent side effects.

It is possible that receiving ST-400 with your regular medications or supplements may change how ST-400, your regular medications, or your regular supplements work. It is very important that you tell the study doctor about all medications or supplements you are taking during the study.

Risks and Toxicities Related to Transplant Procedures

There are some risks and potential problems due to transplant procedure that can include the following:

Serious infections: Full and complete recovery of your immune system may take many months following the initial recovery of your blood cell counts. During this time, there is an increased risk of infections. You will be prescribed certain medicines to reduce the chance of those infections. However, preventive treatments are not always effective. If you have an infection, you may have to stay in the hospital longer, or be re-hospitalized after blood stem cell infusion. Although most infections can be successfully treated, some infections may be fatal.

Recurrence of disease: Thalassemia may persist or come back even if the blood stem cell infusion is initially successful. There is a chance that the number or quality of blood stem cells collected from you will not be enough to cause engraftment or will delay engraftment. A delay in engraftment means that the chance of infection and disease recurrence increases.

Central venous catheter complications: Pain at insertion site, minor bleeding and infection may happen. The most common complications related to central venous catheters are blood clots in the catheter and infection. If a clot forms, a medicine will be injected to dissolve the clot. If it does not dissolve, the catheter may need to be replaced. Infections will be treated with medicines; sometimes, removal of the infected catheter is required and a new catheter will need to be placed.

Risk of death: Some of the side effects of this treatment may be very severe and may cause death despite using all supportive care. Though all precautions will be taken to make the treatment as safe as possible, based on experience with autologous transplants for other disorders, there is a very small chance of the patient's death following the treatment.

Reproductive Risks

Because of the unknown possible effects of ST-400, there could be serious harm to both the mother and unborn or breast-feeding child.

Busulfan or plerixafor may also cause serious harm to both the mother and unborn or breast-feeding child.

If you are currently pregnant, it is important that you inform the study doctor because you will not be able participate in the study. If you have the potential to become pregnant, you will be tested for pregnancy while you are in the study. If you are considering pregnancy after treatment, you should not become pregnant until at least 1 year after receiving ST-400 at minimum. If you do become pregnant, you must tell the study doctor immediately and consult an obstetrician or maternal-fetal specialist.

If you are a man, there may be risks to an unborn baby you father during the study. If you think your partner is pregnant during the study, you must tell the study doctor immediately.

If you have the potential to become pregnant or father a child, you must agree to use an effective method of contraception during the study, from signing the informed consent through the Primary Study Period (1 year after receiving ST-400). Effective methods of contraception include abstinence; intrauterine device (IUD); male condom with spermicide *plus* a diaphragm or cervical cap or sponge; or hormonal-based contraception such as oral contraceptive pills.

Females will be tested for pregnancy periodically throughout study participation. Subjects who become pregnant before receiving ST-400 will be excluded or discontinued from the study procedures to avoid potential risks to the developing fetus.

If you or your partner become pregnant, the study doctor may ask for information about the pregnancy and the birth of the baby. The study doctor may share this information with the sponsor and IRB (the group of people who reviewed this study).

Additional Risks

You may be excluded from future gene therapy or vaccine trials as a result of your participation in this study.

General Infusion Risks

You will receive study product intravenously, which means you will receive it directly into your vein. This may cause the following problems:

- Damage to your vein
- Damage to the skin or tissue around the injection site

- Increase or decrease in electrolyte levels (the amount of certain salts and other chemicals in your blood), causing health problems
- A blood clot or an air bubble could form, which could block a blood vessel in another part of your body

Some of these problems could be very serious.

Other side effects may occur.

Risks Associated with Drawing your Blood

Occasionally there are risks associated with collecting your blood such as pain, bruising, swelling, black and blue marks, dizziness, fainting and/or infection at the site.

Risks Associated with Bone Marrow Aspiration

Bone marrow aspiration is a clinical procedure to draw fluid from your bone marrow using a needle inserted into your hip. Possible side effects of taking bone marrow samples include pain, bruising, bleeding, swelling, or scarring of the skin. Very rarely, an infection can occur at the place the needle was put into the skin. A reaction to the medicine used to numb the skin and bone before the bone marrow aspiration is also rare but could be fatal.

Risks Associated with Liver Biopsy

You may require a liver biopsy at screening to ensure it is safe to proceed with the conditioning regimen and ST-400 treatment. Liver biopsy requires a needle or catheter (tube) to enter the body. There is a small risk of bleeding or infection after having this procedure, which may require an operation to stop the bleeding or medicine to stop infection. In rare cases, taking liver tissue this way may injure an organ next to the liver. Even though you will receive some numbing medicine, you may experience pain during or right after the procedure. You could also have an allergic reaction to the numbing medicine, but this is rare. In very rare cases, it can be fatal.

Risk of Magnetic Resonance Imaging (MRI)

Heart and liver problems are common in beta-thalassemia. Cardiac and liver MRI are standard tests to evaluate iron deposition in your heart and liver. MRI uses high powered magnets to generate a picture of the inside of the body. There are no known harmful side-effects associated with temporary exposure to the strong magnetic field used by MRI scanners. However, MRI can be uncomfortable or frightening for some people. You cannot move while you are having an MRI so if you are uncomfortable with small spaces, or don't think you can lay still, please let the study doctor know right away. The presence of metal in your body may be a safety hazard or affect a portion of the MRI image. Before receiving an MRI, tell the doctor or technologist if you have any metal or electronic devices in your body.

Risk of X-Rays

You will need to have one chest x-ray at the beginning of the study to verify that you do not have any medical problems or infections, such as pneumonia, that would prevent the infusion of ST-400.

X-rays involve exposure to radiation. This radiation exposure is not necessary for your medical care and is for research purposes only. This exposure involves minimal risk and is necessary to obtain the research information desired.

Risk of Electrocardiograms (ECG)

ECGs measure the electrical activity of the heart. Sticky pads and wires will be attached to the chest. These sticky pads may cause a temporary skin reaction or skin irritation. In addition, these pads may cause some discomfort when they are removed, similar to the pulling sensation associated with the removal of an adhesive bandage.

Risks of Genetic Testing

In order to be in this study, the study doctor will need to know which gene mutation you have that caused your thalassemia. Mutations are the errors in the gene that causes disease. You will need to have a blood sample sent for mutation analysis to participate in this study.

Mutation analysis involves a process called genetic testing.

There are risks associated with research involving genetic or related testing. Such risks could include the possibility of reduced access to or retention of benefits or entitlements (e.g. insurance, educational opportunities, employment, etc.); stigmatization; psychological distress in response to information; or detection of biological relationships within a family.

A Federal law, called the Genetic Information Nondiscrimination Act (GINA), generally makes it illegal for health insurance companies, group health plans and most employers to discriminate against you. This law generally will protect you in the following ways:

- Health insurance companies and group health plans may not request your genetic information that we get from this research
- Health insurance companies and group health plans may not use your genetic information when making decisions regarding your eligibility or premiums
- Employers with 15 or more employees may not use your genetic information that we get from this research when making a decision to hire, promote, or fire you or when setting the terms of your employment

All health insurance companies, group health plans and employers with 15 or more employees must follow this law.

Be aware that this Federal law does not protect you against genetic discrimination by companies that sell life insurance, disability insurance, or long-term care insurance. In addition, this Federal

law may not protect you against genetic discrimination on the basis of a genetic disease or disorder that is already obviously visible, apparent or manifest.

Confidentiality Risks and Others

There is a risk of loss of confidentiality of your information. You will read more about the protection of your information later in this form. Please ask the study doctor or study staff if you would like to know more about how your information will be protected while you are in this study.

You may not be able to have future gene therapy or participate in vaccine trials as a result of your participation in this study.

WILL I RECEIVE ANY NEW INFORMATION DURING THE STUDY?

At any time, during and after the study, if the study doctor or study staff learns any new information about the study or study investigational treatment that could potentially affect your health, the study doctor or study staff will tell you about it in a timely manner. Please remember that once you received the study investigational treatment, it cannot be changed.

WILL IT COST ANYTHING TO BE IN THIS STUDY?

The study drug will be made available to you at no charge and, as a part of the study, the study sponsor will pay for the following:

- ST-400
- Hospitalization during study investigational treatment and post infusion observation
- The costs of the study doctor or study staff giving you ST-400
- Medication you may take as part of the study to help treat or prevent side effects
- If you elect oocyte/sperm and/or embryo banking, the study sponsor will pay for the costs of the procedures and storage of banked tissues for the duration of this study
- Procedures and tests that are not part of your regular medical care ("research"), including apheresis and storage of the back-up treatment

You and/or your health-care payer/insurer will be billed for costs for the following:

- Costs of your regular medical care that are not part of this study ("standard of care")

You and/or your health-care payer/insurer may be billed for costs for the following:

- Costs of diagnosing and treating a condition or injury. This could happen if:
  - The sponsor and/or the study doctor do not think the condition or injury is a direct result of you being in the study.
  - You have not followed the directions the study doctor or study staff gave you regarding the study.

You can ask the study doctor or study staff to find out more about costs.

WHAT IF I GET HURT OR SICK WHILE I AM IN THIS STUDY?

If you are injured because of your participation in this study, treatment for the injury will be made available through [name of physician] and [institution]. Either the sponsor or [institution] will pay the costs of this treatment not paid by your medical insurance. No other payment is available from the sponsor or the [institution or study doctor] in the event of injury. You are not waiving any legal rights by signing this form, accepting medical care or accepting payment for medical expenses.

In the event that this research activity results in an injury, treatment will be available, including first aid, emergency treatment and follow-up care as needed. Care for such injuries may be billed in the ordinary manner, to you or your insurance company. The sponsor of the study has some funds available to pay for care for injuries resulting directly from being in this study. If you think that you have suffered a research related injury, let the study physicians know right away.

The sponsor does not plan to pay for an injury or illness that happens if you did not follow the directions of the study doctor or study staff or any injuries or illness that are caused from pre-existing medical conditions or natural progression of beta-thalassemia. To ask questions about this, talk to the study doctor or study staff.

Be aware that your health plan might not cover the costs of study-related injuries or illnesses. Medical treatment will be available if you need it. To ask questions about any of this, talk to the study doctor or study staff.

You do not give up any of your legal rights by signing this form.

WILL I RECEIVE PAYMENT?

You will not receive payment to be in this study. The study sponsor will cover reasonable costs associated with travel including: airfare, bus or cab fare, local transportation, and hotel. The sponsor will provide a daily allowance to cover the cost of food and miscellaneous items.

DO I HAVE TO BE IN THIS STUDY?

Your participation in this study is completely voluntary. You can decide at any time not to be in the study and you can change your mind about being in the study. There will be no penalty to you, and you won't lose any benefits. If you want to stop being in the study, tell the study doctor or study staff. If you decide to not receive ST-400, but you have received busulfan, you will need to remain in the hospital and receive the rescue treatment with your own unmodified stem cells before leaving the study.

The study doctor or study staff or sponsor can remove you from the study at any time, even if you want to stay in the study. Even if you have passed all of the screening tests, there is a possibility that you will not be enrolled in the study.

This could happen if:

- The study doctor, study staff, or sponsor believes it is best for you to stop being in the study
- You do not follow directions about the study

- The U.S. Food and Drug Administration or the study sponsor (Sangamo Therapeutics, Inc.) stops the study for any reason

If you stop being in the study early, the study doctor or study staff may ask you some questions about being in the study. The study doctor or study staff may ask you to participate in some procedures or tests to help you leave the study safely and/or to collect more information for the study. If you leave the study, the study doctor and study staff will still be able to use your information that they have already collected.

If you change your mind about being in the study later, be aware that your biological samples (blood and tissue) collected may or may not be withdrawn from the research.

HOW WILL MY INFORMATION BE KEPT CONFIDENTIAL?

Your private health information (PHI) created or received for the purposes of this study is protected under the federal regulation known as HIPAA.

Be aware that your study records (which include your medical records, your signed consent form, and other information) will be shared and copied as needed for the study. The FDA, the sponsor, the sponsor's representatives, and/or Sanofi, and IRB may look at your study materials and medical records. Please note that the study doctor or study staff may also share personal information about you if required by law (for example, if the study doctor or study staff suspects that you are going to harm someone or yourself). If you have questions about this, please ask the study doctor.

If you would like to know how the sponsor, the sponsor's representatives, and Sanofi will protect the privacy of your records, ask the study doctor or study staff how to get this information.

The study doctor, study staff, Sangamo, or Sanofi may use some facts about your being in this study in books, magazines, journals, and scientific meetings. If this happens, no one will use your name or other information that could be used to identify you.

This research study may be performed only by collecting and using your medical information. Your study records will be kept as confidential as possible. You will not be personally identified in any reports or publications that may result from this research study. Because of the research goals of this study, however, your study records cannot be kept completely confidential.

The study personnel, the sponsor, Sanofi, and sponsor's contractor, Medpace, will need to review the medical information collected from you for use in this study in order to accurately record information for this study. In addition, in order to review the study findings, the U.S. Food and Drug Administration (FDA) and other regulatory agencies may review your medical records. The following sections provide a specific description of how your information will be used and disclosed if you participate in this research study. By signing this consent form, you are authorizing such access. If you do not sign this form to authorize access, you will not be able to participate in this research study.

The medical information that will be collected from you if you participate in the study includes:

- Information obtained from procedures to determine your eligibility to participate in the study, including a routine medical history, physical exam, x-rays, electrocardiogram (ECG), blood and urine tests and MRI scans, etc.

- Information that is created or collected from you during your participation in the study, including the results of the tests included in previous bullet point and any other tests or procedures performed during the study.
- Information contained in your underlying medical records related to your medical history and treatment.

The above information may identify you by name, address, telephone number, social security number, health plan number, study number, date of birth, dates relating to various medical procedures, and/or other identifying information.

If you sign this form and participate in the study, the study personnel will be authorized to use the information described above to carry out the purposes of the research study. The study personnel will also be authorized to disclose the relevant information described above to the following parties involved in the research study:

- Sangamo Therapeutics, Inc., Medpace or other entities designated by Sangamo Therapeutics, Inc. to collect or review study data for verification of study procedures and/or adverse event reporting.
- The Institutional Review Board (IRB) or Independent Ethics Committee (IEC) that oversees the research study at your site.
- Government regulatory agencies including the FDA.
- Clinical trial recruitment company if you were referred to the study by such a company, for analytical purposes and so they may be compensated.

Once your information is disclosed to the study sponsor, Sanofi, entities that work with the sponsor, the IRB/IEC or government agencies as described above, there is a potential that your medical information will be re-disclosed and will no longer be protected by US federal privacy regulations. In addition to disclosures to the entities identified above, your coded health information may be electronically disclosed to others involved in the research study, such as:

- To laboratories or offsite testing facilities for clinical tests required by study protocols.
- To approved offsite storage facilities or cloud service providers to meet study record retention and storage requirements.
- To Sangamo Therapeutics, Inc. who directs the medical research study, or to Sanofi.
- To other third parties contracted by Medpace, and/or Sangamo Therapeutics, Inc. to provide services related to the study.

The study data may be transferred to other countries for processing, including countries not covered by data protection legislation. The laws of your state may provide further protection.

While the study is in progress, you will not be able to access your study records. You will be able to access your information when the research study is completed. You have the right to see and copy the medical information collected from you in the course of the study for as long as that information is maintained by the study personnel and other entities subject to federal privacy regulation.

Study data, including your coded medical information, may be used and shared for pharmaceutical research purposes related to this study. This authorization has no expiration date. In signing this form, you authorize the use and disclosure of your information for purposes of the study at any time in the future.

You may withdraw your authorization at any time by sending a written request to [insert name of responsible study personnel] at [insert address]. If you withdraw your authorization, data collected prior to your withdrawal may still be processed along with other data collected as part of the study. Normally no new information will be collected for the study database unless you specifically authorize that. However, the law does require that any side effects that you may suffer are documented and reported. To complete the study findings, your long-term health status may also be obtained from public sources.

A description of this clinical trial will be available on *http://www.ClinicalTrials.gov* (NCT: # 03432364), as required by U.S. Law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

The study doctor and study staff will make efforts to provide protection from the media in an effort to protect your privacy. The study doctor and study staff will not give away your identity to news reporters at any time.

Use of Biological Samples for Exploratory and Future Research

Researchers working for or with Sangamo and/or Sanofi may use your biological samples including the harvested cells or the manufactured ST-400 that were or will be obtained from you during the study for other investigations. These samples may be utilized for research tests developed during the time you are on study or, in some cases, years later. These tests may provide additional information that will be helpful in understanding your disease or response to the study drug, but it is unlikely that these other research studies will have a direct benefit for you. These studies may benefit patients in the future.

In addition, biological samples obtained from you may be used to establish products that could be patented or licensed. Financial compensation will not be provided to you should this occur.

Samples will be stored by , or on behalf of Sangamo, or Sanofi indefinitely.

You will not be given results of these additional research studies, and studies data from such stored biological samples will not be placed in your medical record.

Unless otherwise allowed by law, you have the right to be informed of any plans for new analyses on your retained identifiable biological samples including future harvested cells or the manufactured ST-400 that are not currently described in this consent form . You also have the right to withdraw your samples from additional research studies by contacting the study doctor in writing. However, data from samples that have already been tested cannot be removed from these studies.

To protect your privacy, your samples will not include your name or other personal identifiers. The samples are labeled with a code that can be linked to you by the study doctor. Your samples and data associated with and generated from your samples will be made available to Sangamo, people who work with or for Sangamo, other researchers involved in this study , and Sanofi, to support research for the purposes listed in this consent.

Your confidentiality will be protected as required by law and according to any policies the clinic or sponsor may have. Be aware that your study records (which include your medical records, your signed consent form, and other information) will be shared and copied as needed for the study. For example, the FDA may look at your records.

The study doctor or sponsor can remove you from the research at any time, even if you want to stay in the research. This could happen if the sponsor stops the research for any reason.

WHO DO I CONTACT IF I HAVE QUESTIONS ABOUT THE STUDY?

In the event of an emergency, dial 911 immediately.

If you require emergency care, be sure to tell the emergency care provider about your participation in this study. Contact the study doctor or study staff as soon as possible.

In case you have questions related to your rights as a participant in a clinical study, you can contact the [IRB/EC name], at: [phones], [e-mail].

You can ask questions about the study at any time. You can call the study doctor or study staff at any time if you have any concerns or complaints. You should call the study doctor or study staff at the phone number listed on page 1 of this form if you have questions about the study procedures, study costs (if any), study payment (if any), or if you get hurt or sick during the study.

WHO WILL USE AND SHARE INFORMATION ABOUT MY BEING IN THIS STUDY?

This section explains who will use and share your private health information if you agree to be in this study. If you do not sign this form, you cannot be in the study.

During the study, the study doctor and study staff will use, collect, and share health information about you (your "records"). Your records may include any information about you that the study doctor needs to do the study and other identifying information about you, such as your name, address, phone number, or social security number. Your records will include:

- Medical records
- Medical history
- Physical exams
- Laboratory test results
- Medical images and reports
- Mental health diagnosis or treatment
- Interviews and/or questionnaires
- Information from other procedures you have as part of the study

If you sign this form:

- You allow the study doctor and study staff to use your records to carry out this study.
- You allow the study doctor to share your records with the sponsor, Sangamo Therapeutics, Inc.; people who work with or for the sponsor; and other researchers involved in this study, and Sanofi. These people will use your records to review the

study, to check the safety and results of the study, and to seek government approval for ST-400. When the study doctor sends your records to these people, your records will not have your name on them. They will have a code that links them to you. However, the sponsor can find out your name if necessary.

- You allow the study doctor or sponsor to use some facts about your being in this study in books, magazines, journals, and scientific meetings. If this happens, no one will use your name or other information that could be used to identify you.
- You allow the study doctor to share all of your records and this signed authorization form with government agencies, including the Department of Health and Human Services (DHHS), the U.S. Food and Drug Administration (FDA), and other government agencies in the United States and other countries. The study doctor may also share your records with regulatory agencies, like Institutional Review Board (IRB). These agencies may use your records to check the study information, how researchers are doing the study, participants' safety, and the results of the study.
- You allow the study doctor to share your records with your health care payer to resolve your claim if you are hurt because of being in this study. If this happens, the study doctor or the sponsor may share your records with their insurance carriers to resolve your insurance claim, and the study doctor may also request medical records from your other health care providers to learn more about your condition.

There are national and state laws that make the study doctor protect the privacy of your records. However, you do not have a guarantee of absolute privacy because of the need to share your information. After the study doctor shares your records with the sponsor and others, the laws may no longer protect the privacy of your records. The sponsor or others may share your records with other people who do not have to protect the privacy of your records. If all information that does or can identify you is removed from your records, the remaining information will no longer be subject to this authorization and may be used or shared for other purposes.

No publication or public presentation about the research described in this consent will reveal your identity without permission from you.

You might have the right to see and copy your health records related to this research. You might not be able to see or copy some of your records until after all participants finish the study. If it is necessary for your care, your records will be provided to you or your regular doctor.

You can cancel this authorization to use and share your records at any time. If you want to cancel your authorization, you must write a letter to the study doctor at the address listed on page 1 of this form. If you cancel your authorization, you will not be able to continue in the study.

Even if you cancel your authorization and leave the study early, the study doctor and study staff will still be able to use and share your records that they have already collected as described above.

This authorization to use and share your records expires in 50 years.

You should not sign this consent form unless you have had a chance to ask and have received satisfactory answers to all of your questions. You will receive a signed copy of this form.

Please indicate below whether you want us to notify your primary care physician or your specialist of your participation in this study.

_____ Yes, I want the study doctor to inform my primary care physician/specialist of my participation in this study.

_____ No, I do not want the study doctor to inform my primary care physician/specialist of my participation in this study.

_____ I do not have a primary care physician/specialist.

_____ The study doctor is my primary care physician/specialist.

PERMISSION FOR HOME HEALTH SERVICE PROVIDER

Please check and initial your permission for home health service provider:

☐ **I agree** to use a home health service nurse to perform assessments and to collect blood and urine samples associated with this study. I understand and agree that my name and contact details will be shared with the home health service provider and samples couriers involved as necessary in order to perform this service.
Initials:

☐ **I do not agree** to use the home health service and **DO NOT** give permission for my name and contact details to be shared.

STATEMENT OF CONSENT

I have read this form, and I have been able to ask questions about this study. The study doctor or study staff has talked with me about this study. They have answered all my questions. I voluntarily agree to be in this study. I agree to allow the use and sharing of my study-related records as described above.

By signing this form, I have not given up any of my legal rights as a research participant. I will get a signed copy of this consent form for my records.

Please check and initial your choice for use of your biological samples including the cells or the gene edited stem cells (ST-400) for exploratory and future research:

☐ **I agree** to let the Sangamo and Sanofi to store and use my biological samples including the cells or ST-400 for exploratory and future research.
Initials:

☐ **I do not agree** to let the Sangamo and Sanofi to store and use my biological samples including the cells or ST-400 for exploratory and future research.

Initials:

_____
Printed Name of Participant

_____ _____
Signature of Participant Date

I attest that the participant named above had enough time to consider this information, had an opportunity to ask questions, and voluntarily agreed to be in this study.

_____
Printed Name of Person Explaining Consent

_____ _____
Signature of Person Explaining Consent Date

I attest that I or my representative discussed this study with the participant named above.

_____
Signature of Principal Investigator or Sub-Investigator