

**Thank you
for your participation
in our study!**



Leukemia research is about
more than the disease.



Welcome to the XYZ study!



Dear Study Participant,

As your local research team shared with you, the purpose of the study is to evaluate the investigational medication to see if it is safe and effective for patients with XYZ. By participating, you are helping develop possible new future treatment options for this condition. We sincerely thank you for your participation. For a detailed description of the study, study procedures and known risks and potential benefits of the study drug, please refer to the Informed Consent Form.

**With much appreciation,
The Study Team**

Your responsibilities

Here are your responsibilities while participating in this study:

- Attend all scheduled appointments
- Take the study drug as directed by study staff
- Make sure no one else takes your study medication
- Follow the instructions given to you by the study doctor and study staff
- Tell the study doctor about all changes in your health and medication while you are participating in the study
- If you visit or receive medical care from another doctor, tell him or her that you're in a research study
- Inform the study doctor or study staff if you move or change your telephone number during the study
- Tell the study doctor or study staff if you want to stop being in the study
- If you become pregnant during this study, notify the study doctor as soon as possible

Please contact the study staff if you have any questions about your responsibilities in this study.

Study medication

If you qualify for the study, you'll receive the investigational medication or placebo for 1 year, after which you'll receive the investigational medication for another year.

Remember that during the first treatment phase, neither you nor your study doctor will know if you're receiving the investigational medication or placebo. Your study doctor can find out what medication you're receiving in case of an emergency.

Your study medication will be tablets that you'll take by mouth. Please take them as directed by your study staff, and please remember to bring your study drug box (even if you have taken all the study drug) to each visit. The study staff will count any remaining tablets.

If you have any questions about your study medication, please contact the study staff as soon as possible.



Glossary of Study Tests and Procedures



Adverse events: possible effects from the study medication



Assess bleeding episodes: review any bleeding episode you had and your FIX concentrates usage



Blood sample: blood is drawn and tested for multiple things related to your health



ECG, or electrocardiogram: a test that monitors your heart rate



Informed consent form (ICF): a form you sign that states you understand your rights and responsibilities in the study



Investigational therapy: SB-FIX



Liver elastography: an ultrasound test for liver tissue stiffness



Medical history and medications: any diseases, surgeries, medicines, or treatments you had in the past



MRI: an imaging test that uses magnetic fields



Physical exam: an assessment of general appearance, eyes, ears, nose, throat, neck (including thyroid), lymph nodes, chest (lungs), cardiovascular, abdominal, skin, extremities, musculoskeletal, and neurological system (including mental status)



Previous/concomitant medications: any medicine you take now, and any medicine you have taken in the past



Saliva, stool, and semen samples: to check for the presence of viral vectors














Urine samples: will be used to test multiple things related to your health












Vital signs: your blood pressure and heart rate

Screening Visits

 Sign informed consent form	 Urine samples
 Review medical history	 Blood samples
 Physical or abdominal exam	 Review medications
 Check your vital signs	 Give you liver elastography
 ECG	 Liver MRI
 Chest X-ray	








Baseline Visit

approximately 1 week before hospitalization

 Physical or abdominal exam	 Adverse events
 Check your vital signs	 Assess bleeding episodes
 Urine samples	 Take saliva, stool, and semen samples
 Blood samples	 Give you liver elastography
 Review medications	












Hospitalization — 24 hour stay

 Check your vital signs	 Adverse events
 Urine samples	 Investigational therapy
 Blood samples	 Take saliva, stool, and semen samples
 Review medications	

Visits every 2 weeks

Weeks 2, 4, 6, 8, 10, and 12










Lab assessments may be needed weekly and participants may receive a limited course of steroids.

 Physical or abdominal exam	 Assess bleeding episodes
 Urine samples (weeks 4, 8, and 12 only)	 Take saliva, stool, and semen samples
 Blood samples	 Give you liver elastography
 Review medications	 Liver MRI
 Adverse events	












Visits every 4 weeks

Weeks 16, 20, 24, 28, 32, 36, 40, 44, 48, and 52

 Physical or abdominal exam	 Assess bleeding episodes
 Urine samples	 Take saliva, stool, and semen samples
 Blood samples	 Give you liver elastography
 Review medications	 Liver MRI (weeks 24 and 52 only)
 Adverse events	

Visits every 3 months

Months 15, 18, 21, 24, 27, 30, 33, and 36

 Physical or abdominal exam	 Assess bleeding episodes
 Urine samples	 Take saliva, stool, and semen samples
 Blood samples	 Give you liver elastography
 Review medications	 Liver MRI (months 18, 24, 30, & 36 only)
 Adverse events	



Please attend all your study visits!

It's important that you attend all your study visits so that you keep receiving care and so that doctors can monitor your health. Contact your study coordinator if you can't attend an appointment. We'll work with you to find a new date and time.

Study telephone numbers and contact information:

Study doctor's name:

Study doctor's telephone number:

Study coordinator's name:

Study coordinator's telephone number:

In the event of a medical emergency, seek immediate medical attention and contact the study doctor as soon as possible.

Remember to ask questions!

Please ask us questions at any time if you don't understand something or would like more information. Make sure you read all of the information we give you, and ask a member of our study team if something isn't clear.