

**CONSENT FORM**

HIPAA Compliant

**Institutional Review Board for Baylor College of Medicine and Affiliated Hospitals****ICON 3**

H-42131- A PHASE 3 STUDY OF ELTROMBOPAG VS. STANDARD FIRST-LINE MANAGEMENT FOR NEWLY DIAGNOSED IMMUNE THROMBOCYTOPENIA (ITP) IN CHILDREN

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**Concise and Focused Presentation**

This is a research study for patients diagnosed with immune thrombocytopenia (ITP), a condition that results in low platelets and possibly bleeding due to antibodies your body makes against your platelets. You are being invited to participate in this study because you have been identified to as having ITP .

The purpose of this study is to investigate the safety and effectiveness of eltrombopag in treating children and adolescents with newly diagnosed ITP.

If you choose to participate, you will be on study for about 1 year. You will be randomly assigned to receive the study drug eltrombopag or standard therapy.

During your participation you will have study procedures weekly for 12 weeks and then about monthly or less often for up to 1 year.

The following procedures will be performed:

- Physical exam
- Complete a Bleeding assessment
- Review your medical record.
- Ask how you are feeling and if you have had any side effects from therapy
- Ask you to complete some questionnaires regarding how you are feeling and your ITP
- Collect blood samples to assess your general health
- If you are a girl and have had your period, a blood or urine sample will be collected to see if you are pregnant
- Ask you about if you have been taking your study medication.

You will be asked to participate in an optional blood collection to collect blood one time for future research including genetic research.

The most common risks associated with eltrombopag treatment are headache, muscle/extremity pain, runny nose, cough, vomiting. Some more serious, but less common risks are liver enzyme elevation, blood clots.

Your participation in this study is voluntary. you may choose not to participate in this study. You may choose to receive routine care or participate in other studies.

The benefits of participating in this study may be an improvement to your ITP and increasing the general knowledge and understanding of ITP and treatment. However, you may receive no benefit from participating.

Please find a more detailed description of procedures and risks below.

**Background**

When reading this form, please note that the words, "you" and "your" refer to the person in the study rather than to a parent or guardian, or legal representative who might sign this form on behalf of the person in the study.

Patient Name/ID: \_\_\_\_\_

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This form contains the purpose of the research being conducted, the procedures involved, your responsibilities, and the risks and benefits associated with participation in the study. Please read this form and ask the Study Doctor and Study Staff any questions you may have. If there are any words or information that you do not know, ask them to explain. Feel free to take notes, write questions or mark any part of this form.

This research is being done by members of the Pediatric ITP Consortium of North America (ICON). ICON is a group of pediatric doctors throughout the United States who in a collaborative research effort are dedicated to improving the understanding, treatment, and quality of life of pediatric patients with ITP.

This is a research study for patients diagnosed with immune thrombocytopenia (ITP), a condition that results in low platelets and possibly bleeding due to antibodies your body makes against your platelets. Platelets enable your blood to clot and stop bleeding. You are being invited to participate in this study because you have been identified as having ITP.

If you join this study, you will be treated with one of four treatment plans. Three of the treatment plans are the standard treatments for newly diagnosed ITP in children and adolescents. The fourth treatment plan involves a drug named eltrombopag.

Eltrombopag is a drug which is approved in over 40 countries including the United States and European Union for treatment of chronic ITP (lasting longer than 6-12 months) and other types of blood diseases, including adults and children with low platelets. Over 5000 patients have been treated with eltrombopag in clinical studies to date. Its use in this study is considered "investigational" because eltrombopag is approved by the Food and Drug Administration (FDA) to treat children with chronic ITP, but has not been studied in children with newly diagnosed ITP.

Your participation in this study is voluntary. You are free to say yes or no. If you do not want to participate, your regular medical care and legal rights will not be affected. Even if you join this study, you may stop at any time.

This research study is sponsored by Baylor College of Medicine and is funded by Novartis. The investigational drug, eltrombopag, is supplied by Novartis.

A description of this clinical trial will be available on <http://www.ClinicalTrials.gov>, 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.

**Purpose**

The purpose of this study is to investigate the safety and effectiveness of eltrombopag in treating

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children and adolescents with newly diagnosed ITP.

**Procedures**

The research will be conducted at the following location(s):

Baylor College of Medicine, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Children's Hospital of Chicago, Nationwide Children's Hospital, Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, and University of Florida.

A total of up to 162 subjects will be enrolled on this protocol. Approximately 20 of those subjects will be enrolled at our local site.

**DURATION.**

There will be a total of approximately 16 study visits over a 1 year period. In addition, we will follow you for thirty days after your last dose for this study to see how you are doing.

**STUDY DESIGN**

If you agree to take part in the study, you will be assigned randomly (like rolling a dice) to be treated with:

- Eltrombopag
- Standard Therapy

Two out of three subjects taking part in the study will be given eltrombopag and one out of three subjects will be treated per standard therapy.

You and your study doctor(s)/team will know whether you will receive eltrombopag or standard therapy.

If you are randomized to receive the standard therapy you will be treated with one of the following three options depending on which treatment method your study doctor thinks is best for you.

- Intravenous immunoglobulin (IVIG)
- Steroids
- Anti-D immune globulin (Anti-D)

All three are standard front-line treatments for treating ITP for pediatric and adolescent patients. If your study doctor determines that IVIG or Anti-D globulin is the best treatment option for you, you will receive one dose of IVIG or Anti-D through an IV. If you receive steroids, you will take them twice daily for four days by mouth.

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If you are randomized to receive eltrombopag, you will take it once daily. Your dose may be modified depending on your platelet count. You will take eltrombopag for 12 weeks, with the possibility to continue therapy for up to 1 year depending on how your body responds to taking eltrombopag.

You will be required to take eltrombopag on an empty stomach, 1 hour before or 2 hours after a meal. Eltrombopag is available as a liquid and as a tablet. If you take the tablet form, you must be able to swallow the tablet(s) whole with a glass of water without chewing. The tablet should not be crushed or broken.

It is also important that you do not take the eltrombopag in the 2 hours before and 4 hours after taking any other medications, calcium-rich foods (such as, dairy products like cheese, yogurt and milk, and calcium-fortified juices), or vitamins containing minerals such as iron, calcium, aluminum, magnesium, selenium, and/or zinc. Please discuss this further with the Study Doctors as they can advise on other foods to avoid to ensure the drug is most effective.

If you vomit within 30 minutes of taking eltrombopag, the dose should be repeated.

Regardless of whether you are randomized to one of the standard therapies or eltrombopag, all will be referred throughout this consent as study medications.

**STUDY PROCEDURES****I. SCREENING VISIT (Visit 1)**

If you agree to participate in the study, you will sign a consent form and the study doctor and study staff will:

- Confirm whether you are eligible to participate in this study
- Collect information about your ITP
- Ask you about the medicines you are currently taking
- Perform a physical examination
- Examine your eyes for cataracts (clouding of the normally clear lens of the eye).
- Draw blood (about four teaspoons) to measure your blood cell counts, iron levels, liver function, and immune function, and to assess your general health if not done as part of routine medical care. If you are a female and have started your period, a blood sample may also be drawn to see if you are pregnant.
- Take a urine sample to see how your kidneys are functioning. If you are a female, have started your period, and a blood sample was not drawn to see if you are pregnant, your urine will be checked to make sure you are not pregnant.
- Ask you to complete some questionnaires about your how you feel and about your ITP.

You will be asked come back within 72 hours (+/- 24 hours) to have your platelet count checked after this

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visit. For this approximately ½ teaspoon of blood will be drawn.

**II. Treatment Period I(Weeks 1- 12)**

You will be asked to come complete study visits during the twelve weeks after your screening visit . Only week 4 and week 12 will be required to be done at the study site, and in some cases these may be done by telehealth. At certain time points, labs may be done locally and questions can be asked over the phone. At these visits the study team or doctor will do all or some of the following:

- Perform a Physical exam
- Complete a Bleeding assessment
- Review your medical record and collection information from your medical record that is related to your health and/or disease history. Some examples include test results, medical procedures, pathology reports, medicines you take.
- Ask how you are feeling and if you have had any side effects from therapy
- Ask you to complete some questionnaires regarding how you are feeling and your ITP
- Collect blood samples to assess your general health
- If you are a girl and have had your period, a blood or urine sample will be collected to see if you are pregnant
- Ask you about if you have been taking your study medication. Collect your medication bottles/vials and dispense your study medication

Your platelet count will be checked at weeks 1, 2, 4, 6, 8, 10, and 12. If you are taking eltrombopag, your blood will be tested every two weeks to assess your liver function.

If you are receiving eltrombopag, your dosing maybe altered depending on your platelet count.

**III. Treatment Period II (Weeks 13 to 52)**

If you complete twelve weeks of eltrombopag, the study team will start to decrease your dose over this treatment period depending on your platelet count. If you are still taking eltrombopag at the one year mark, you will be transitioned off study, and you will continue to be treated according to your primary treating doctor.

If you are taking eltrombopag your blood will be tested monthly to assess your liver function and iron levels in your body.

If you are girl and have had your period, your urine or blood will be tested every two months if you are taking eltrombopag to see if you are pregnant.

Regardless of what medication you are receiving or received for this study, you will be asked to come for a visit at 6 month and 1 year mark. At these visits, the study team or doctor will do all or some of the

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following:

- Perform a physical exam
- Complete a bleeding assessment
- Ask you questions and review your medical record, and collect information from your medical record that is related to your health and/or disease history. Some examples include test results, medical procedures, pathology reports, and medicines you take.
- Ask you to complete questionnaires regarding your ITP and how you are feeling
- Ask you how you are feeling and if you have had any side effects
- Collect your study medication
- Dispense your study medication
- Ask you about any medications you are currently taking
- Collect blood samples to assess your general health, clotting, iron levels, immune function and liver function.

**III. UNSCHEDULED VISITS**

You may be asked to come in for additional visit(s). If you come in for an unscheduled visit, the study team or doctor will:

- Complete a physical exam
- Complete a bleeding assessment
- Collect a blood sample to assess your blood counts
- Ask you how you have been feeling and if you have had any side effects or about your medications you are currently taking

**IV. FOLLOW UP**

We would like to keep track of your medical condition after your last dose of study drug. A member of the study team will contact you via phone or email during the month after you stop the study treatment to see how you are doing.

**V. RESCUE MEDICATIONS**

If you have bleeding while participating in this study, please call your study doctor or team. If you have a bleed, platelets drop after initial response or you do not respond to treatment, the study doctor may give you a rescue medication such as IVIG, steroids, and/or Anti-D. If the study doctor decides that you need a different type of rescue medication, you will no longer receive your assigned study treatment, and you will be treated per standard of care. You will still be followed on the study, however, to collect information about your platelet counts, bleeding symptoms, and ITP.

**VI. SUBJECT RESPONSIBILITIES**

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- Come to required study visits
- Bring your empty study medication vials to each visit
- Take your study medication as prescribed
- Tell your doctor about any medications or treatments you undergo while participating on this study
- Complete study questionnaires

**VII. OPTIONAL BANKING SUB-STUDY**

If you decide to participate, the study team will collect an additional 2 ½ teaspoons of blood during your screening visit, week 12 visit, and end of study visit for future research. If in the event of sample processing failure, the study team may re-collect a sample during a later study visit.

Blood will be stored for future use in a biobank. A biobank collects, stores, and distributes biological samples and health information.

The purpose of this collection is to make your samples available for use in research for studies related to ITP and related diseases after this current study is completed. Biobanks are especially useful to learn about diseases, possible treatments, including the role that specific genes play in human diseases.

The samples will be stored at Texas Children's Hospital.

One of the methods researchers might use to study your samples is called whole exome or genome sequencing for analyzing your DNA and RNA expression studies. This allows them to look at some or all of your genetic code. Researchers may also use other methods as they are developed. Studying genes along with health information will help us to better understand what causes certain diseases. It may also help us to understand how different patients respond to treatment. This knowledge could help us to develop new treatments.

"DNA" is short for deoxyribonucleic acid. DNA stores information in the form of a code. This is the code that you inherit from your parents and that you pass on to your children. Parts of DNA that have complete messages are known as "genes." Genes give the instructions for building the proteins that make our bodies work.

"RNA" is short for ribonucleic acid. RNA delivers DNA's genetic code to the part of a cell that makes proteins. RNA also helps control which genes are turned on or off at one point in time.

The goal of DNA and RNA studies are to look for genetic connections which may explain how to identify, prevent, and treat health problems. For example, the data from these studies may be used to find out:

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- Who is more likely to develop a certain illness, such as asthma, cancer, or diabetes, or a condition like high blood pressure or obesity;
- What genes affect the progress of a certain disease or condition; and
- What genes may affect treatments which now may or may not work in certain people.

Genomic research will not directly benefit you, but could lead to a greater understanding of the interaction between genes and health. This knowledge could help others in the future.

Your samples may also be analyzed for certain markers and how they correlate to treatment or related diseases.

We will remove your name and any other information that could directly identify you from your materials. We will replace this information with a unique study code. We will keep a master list that links your study code to your materials. Only certain study staff can access this master list. We will keep health information and research data on secure computers. These computers have many levels of protection.

Your samples will be stored for future use. Any future research performed on your samples will not be allowed unless proof of Institutional Review Board (IRB) approval is obtained to ensure that any future research is conducted ethically, and the rights and safety of study subjects are protected. If the study is approved, we might give a part of your sample and information to the researchers.

Any data or samples that are sent to other researchers will contain only a unique identifying number; they will NOT contain personal identifiers such as your name or address. Data and samples will be kept indefinitely, allowing researchers in the future to ask new questions about blood diseases and treatment.

You should not expect to get personal results from research done through the biobank. Researchers will study samples and information from many people; it will take many years before they know if the results have any meaning.

You can revoke the use of your samples for future use at any time. Any data or information collected prior to you revoking your samples will not be destroyed; however, no further information will be collected.

**WHO WILL HAVE ACCESS TO MY GENETIC INFORMATION?**

Researchers can do more powerful studies when they share with each other the information they get from studying human samples. They share this information with each other by putting it into scientific databases. These databases store information from many studies conducted in many different places. Researchers can then study the combined information to learn even more about health and many different diseases.

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There are different kinds of databases; some are publicly accessible and some are restricted. Anyone on the Internet can access publicly accessible databases. Only researchers who apply and are approved can access restricted databases. There are many restricted databases; some are maintained by BCM, some are maintained by the federal government, and some are maintained by private companies. Some of your genetic and health information could be placed into one or more of these publicly accessible or restricted databases.

Your name and other information that could directly identify you (such as address or social security number) will not be placed into any scientific database. However, because your genetic information is unique to you, there is a chance that someone could trace the information back to you or your close biological relatives. The risk of this happening is very small, but may grow in the future.

Researchers will always have a duty to protect your privacy and to keep your information confidential.

Would you like to participate in this optional sub-study for future research?

Yes: \_\_\_\_\_ Initials: \_\_\_\_\_

No: \_\_\_\_\_ Initials: \_\_\_\_\_

**Clinically Relevant Research Results**

The results generated from this research study are not expected to have any clinical relevance to you.

**Sharing and Future Research Studies with Identifiable Private Information**

Information that identifies you may be removed from your identifiable private information collected as part of this research, and after such removal, your information may be used for future research studies or distributed to another investigator for future research studies without additional consent/authorization from you.

**Sharing and Future Research Studies with Identifiable Biospecimens**

Information that identifies you may be removed from your identifiable biospecimens collected as part of this research, and after such removal, your biospecimens may be used for future research studies or distributed to another investigator for future research studies without additional consent/authorization from you.

**Genome Sequencing Potential**

Your identifiable biospecimens(s) will be or may be sequenced in whole or in part so that your genetic information can be compared to others' genetic information.

**Research related health information**

Authorization to Use or Disclose (Release) Health Information that Identifies You for a Research Study

If you sign this document, you give permission to people who give medical care and ensure quality from

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Baylor College of Medicine, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Children's Hospital of Chicago, Nationwide Children's Hospital, Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, and University of Florida to use or disclose (release) your health information that identifies you for the research study described in this document.

The health information that we may use or disclose (release) for this research includes:

- Information from health records such as diagnoses, progress notes, medications, lab or radiology findings, etc.
- Demographic information (name, D.O.B., age, gender, race, etc.)
- Identifiable biospecimens
- Other: Medical Record Number

The health information listed above may be used by and or disclosed (released) to researchers, their staff and their collaborators on this research project, the Institutional Review Board, Baylor College of Medicine, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Children's Hospital of Chicago, Nationwide Children's Hospital, Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, University of Florida, and NOVARTIS (SWITZERLAND) and their representatives.

Agents of the U.S. Food and Drug Administration may inspect the research records including your health information. Agents of regulatory agencies such as the U.S. Department of Health and Human Services will be permitted to inspect the research records including your health information.

The data coordinating center will have access to the research records including your health information.

A Data and Safety Monitoring Board will have access to the research records including your health information.

**Use or Disclosure Required by Law**

Your health information will be used or disclosed when required by law.

Your health information may be shared with a public health authority that is authorized by law to collect or receive such information for the purpose of preventing or controlling disease, injury, or disability and conducting public health surveillance, investigations or interventions.

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Please note that the research involves treatment. You do not have to sign this Authorization, but if you do not, you may not receive research-related treatment. To maintain the integrity of this research study, you generally will not have access to your personal health information related to this research until the study is complete. However, your health information that is necessary to your care will be provided to you or your physician. At the conclusion of the research and at your request, you generally will have access to your health information that Baylor College of Medicine, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Childrens Hospital of Chicago, Nationwide Childrens Hospital , Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, and University of Florida maintain in a designated record set, which means a set of data that includes medical information or billing records used in whole or in part by your doctors or other health care providers at Baylor College of Medicine, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Childrens Hospital of Chicago, Nationwide Childrens Hospital , Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, and University of Florida to make decisions about individuals. Access to your health information in a designated record set is described in the Notice of Privacy Practices provided to you by representatives of the specific institution where you are being enrolled into this research study which are: Baylor College of Medicine, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Childrens Hospital of Chicago, Nationwide Childrens Hospital , Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's

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Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, and University of Florida.

Please note that you may change your mind and revoke (take back) this Authorization at any time. Even if you revoke this Authorization, researchers, their staff and their collaborators on this research project, the Institutional Review Board, NOVARTIS (SWITZERLAND) and their representatives, regulatory agencies such as the U.S. Department of Health and Human Services, FDA, Baylor College of Medicine, data coordinating center, Data and Safety Monitoring Board, Boston Children's Hospital - Massachusetts, Children's Hospital Colorado, Children's Hospitals and Clinics -- Minneapolis/St. Paul - Minnesota, Duke University - North Carolina, Lurie Children's Hospital of Chicago, Nationwide Children's Hospital, Oregon Health and Science University, St. Jude Children's Research Hospital - Tennessee, TCH: Texas Children's Hospital, TCH: Texas Children's Hospital, Clinic, The Children's Hospital of Philadelphia (CHOP) - Pennsylvania, University of California: San Fran, and University of Florida may still use or disclose health information they already have obtained about you as necessary to maintain the integrity or reliability of the current research. If you revoke this Authorization, you may no longer be allowed to participate in the research described in this Authorization.

To revoke this Authorization, you must write to: Dr. Jenny Despotovic Clinical Care Center 6701 Fannin St. Suite 1580 Houston, TX 77030

This authorization does not have an expiration date. If all information that does or can identify you is removed from your health information, the remaining information will no longer be subject to this authorization and may be used or disclosed for other purposes.

No publication or public presentation about the research described above will reveal your identity without another authorization from you.

**Potential Risks and Discomforts**

**SIDE EFFECTS OF ELTROMBOPAG:**

The side effects listed below have been seen in younger patients (under the age of 18) who have received eltrombopag treatment for (ITP).

Very Common Side Effects:

These may affect more than 1 in 10 people treated with eltrombopag

- Upper respiratory tract infection (runny nose, cold)
- Fever (Pyrexia)
- Abdominal Pain
- Cough

The following side effects have been reported to be associated with treatment with eltrombopag in

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patients with a different disease called severe aplastic anemia (SAA).

Very Common Side Effects:

These may affect more than 1 in 10 people treated with eltrombopag

- Rash
- Cough
- Headache
- Runny nose
- Abdominal pain
- Diarrhea
- Nausea
- Joint pain
- Increase in some liver enzymes (transaminases)
- Pain in arms, legs, hands and feet
- Dizziness
- Feeling very tired (fatigue)
- Fever

Progression of underlying disease or progression to a new myelodysplastic syndrome (MDS) and/or new acute myelogenous leukemia (AML, a type of blood cancer) has occurred in patients with MDS, AML, and severe aplastic anemia (SAA). In some patients with these diseases who are treated with eltrombopag, changes in bone marrow cells may occur and in some cases this may indicate a worsening/progression to cancer. The role of eltrombopag in these changes is not known. These changes have also been seen in patients with SAA alone, and with other drugs in the same class of compounds as eltrombopag. During this study, your blood will be periodically examined for signs of these changes.

Other possible side effects of Eltrombopag:

The following side effects have been reported to be associated with treatment with eltrombopag .

Liver problems:

Eltrombopag may damage the liver and cause serious, even life threatening, illness. This is specific to patients with hepatitis C. Blood tests will be done to check your child's liver before he or she starts taking eltrombopag and during treatment. Your doctor will order the blood tests and any other tests required. In some cases Eltrombopag treatment may need to be stopped. Tell your doctor right away if you notice any of these signs and symptoms of liver problems:

- yellowing of the skin or the whites of the eyes (jaundice)

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- unusual darkening of the urine
- unusual tiredness
- right upper stomach area pain

**Bleeding after you stop treatment:**

When you stop taking eltrombopag, your blood platelet count may temporarily drop back down to what it was before starting eltrombopag or lower. These effects are most likely to happen within 4 weeks after stopping. The lower platelet counts may increase the risk of bleeding. Tell your doctor or pharmacist if you develop any bruising or bleeding symptoms after stopping eltrombopag.

**High platelet counts with a higher chance for blood clots:**

You could have a higher chance of getting a blood clot if your platelet count is too high during treatment with eltrombopag, but blood clots can occur with normal or even low platelet counts. Blood clots are more common in adults who have other risks for developing blood clots. The Study Doctor will check the blood platelet counts, and change the dose or stop eltrombopag if the platelet counts get too high. Tell your doctor right away if you have signs and symptoms of a blood clot in the leg, such as swelling or pain/tenderness of one leg.

**Cataracts:**

In animal studies, it was found that high doses of eltrombopag caused the development of cataracts (a clouding of the lens in the eyes). Following studies on patients with immune thrombocytopenia did not confirm this finding. Regardless, you will be checked for cataracts at baseline and during the study, and a visit to a doctor specializing in cataracts will be scheduled if you are determined to be at higher risk of developing cataracts.

**Contraception and pregnancy-Female Subjects**

If you are pregnant or nursing a child you cannot participate in this research study. You must confirm, to the best of your knowledge that you are not now pregnant and do not intend to become pregnant during the research study. You will take a pregnancy test before the research begins. The results of the pregnancy test are confidential and will be given to you by one of the study nurses or doctors in private.

There are no adequate and well-controlled studies of eltrombopag in pregnant women. The effect of eltrombopag on human pregnancy is unknown. While you are on study and for 7 days after the last dose of study treatment it is important that you use a highly effective form of birth control if you are sexually active and can become pregnant.

Examples of highly effective birth control methods are:

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- Total abstinence (no sexual relations), when this is in line with your preferred and usual lifestyle. Periodic abstinence methods are not acceptable! Some terms used to describe periodic abstinence methods are: calendar, ovulation, symptothermal, post-ovulation. Please note that the withdrawal method is also not acceptable.
- Female sterilization, when you have already been surgically sterilized prior the research study by surgical removal of both ovaries (woman's reproductive system that stores and releases eggs for fertilization and produces female sex hormones), total hysterectomy (surgical removal of the uterus and cervix), or tubal ligation (getting your "tubes tied") at least 6 weeks before taking study treatment.
- Your male partner has already been sterilized and has the appropriate documentation. Your sterilized male partner should be your sole partner.
- Use of oral, injected, or implanted hormonal methods of contraception or placement of an intrauterine device (IUD) or intrauterine system (IUS), or other forms of hormonal contraception that have comparable efficacy (failure rate <1%), for example hormone vaginal ring or transdermal hormone contraception (in case of oral contraception you should have been using the same pill on a stable dose for a minimum of 3 months before taking study treatment).

Please discuss with the Study Doctor the most appropriate birth control method that also respects your cultural and religious preferences. If you become pregnant or suspect you are pregnant (for example, because of a late menstrual period) during study treatment or within 7 days after completing study treatment, you must inform the Study Doctor immediately, and you have to stop ongoing study treatment immediately. You will not be allowed to continue study treatment if you are pregnant. The Study Doctor will medically follow your pregnancy until delivery to monitor safety.

**Contraception and Pregnancy- Male Subjects**

The effects of the study drug on sperm are unknown. In addition, it is unknown if participation in this research could result in harm to a fetus. You should not father a baby while taking part in this research and for the period of 7 days following stopping of study treatment. If you have a female partner who is able to become pregnant, one or both of you must use some form of highly effective birth control. During the research, if your partner becomes pregnant, or if there is a chance that she is pregnant, you should contact the Study Doctor immediately so that we may provide medical assistance and counseling.

**PROCEDURE RISKS:**

Blood Draw: Drawing blood causes discomfort. A bruise may appear for a few days at the spot where the needle was inserted. There is a slight chance of infection. This is very unlikely. There is also a small risk of dizziness and fainting with blood draws. These risks are minimized by the use of trained personnel to draw your blood.

**OPTIONAL SUB-STUDY RISKS:**

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Loss of Confidentiality:

What are the potential privacy risks?

We will take many steps to protect your privacy, but because your DNA/RNA is unique to you, it is possible but unlikely that someone could trace it back to you. There is also a risk that someone could get access to the data we have stored about you. If those data suggested something serious about your health, it could be misused. For example, it could be used to make it harder for you to get or keep a job or insurance. There are laws against this kind of misuse, but they may not give full protection. There may also be other unforeseen privacy risks.

Your privacy and the confidentiality of your data are very important to us; we will make every effort to protect them.

How will my privacy be protected?

We will not give information that identifies you to anyone without your permission, except as required by law. This project takes many steps to protect the privacy of people who take part. Research records are separate from medical records. We will not place any information from this project in your medical records.

Researchers who study your sample and information will not know who you are. We will give them only barcode numbers; we will not give them any information that directly identifies you. The researchers must sign an agreement that they will not try to find out who you are. There are laws that protect against unauthorized access to your information. There is also a Federal law called the Genetic Information Nondiscrimination Act (GINA). In general, this law makes it illegal for health insurance companies, group health plans, and most employers to discriminate against you based on your genetic information. However, it does not protect you against discrimination by companies that sell life insurance, disability insurance, or long-term care insurance. This law generally will protect you in the following ways:

- Health insurance companies and group health plans may not request your genetic information 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 from this research when making a decision to hire, promote, or fire you or when setting the terms of your employment.

There may be unknown risks or discomforts involved. Study staff will update you in a timely way on any new information that may affect your decision to stay in the study. There is a small risk for the loss of confidentiality. However, the study personnel will make every effort to minimize these risks.

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**Potential Benefits**

The benefits of participating in this study may be: improvement to your ITP, and increasing the knowledge and understanding of ITP and treatment. However, you may receive no benefit from participating.

**Alternatives**

You may choose to not participate in this study.

**Subject Withdrawal from a Study**

Taking part in research is always a choice. If you decide to take part in this study, you can change your mind at any time. Please tell the Study Doctor or study staff if you decide to temporarily or permanently stop taking your study medication. You will be asked to return to the study site as soon as possible for a check-up.

If you decide to participate in the optional banking sub-study and decide later that you would no longer like to participate in this study, we will destroy any leftover samples. We will not be able to withdraw your samples from studies that have already begun since we cannot get the samples back once they have been shared with other researchers. If you change your mind, and would like to withdraw from the study, we ask that you inform the research team using the contact information provided above

**Investigator Withdrawal of Subject from a Study**

The investigator or sponsor may decide to stop you from taking part in this study at any time. You could be removed from the study for reasons related only to you (for example, if you move to another city, if you do not take your study medication, or if you have a serious reaction to your study medication) or because the entire study is stopped. The sponsor, investigator, drug supplier, Food and Drug Administration, or Institutional Review Board may stop the study at any time.

**Subject Costs and Payments**

Most procedures used in this study will be part of standard medical care, and therefore you/your insurance company will be responsible for these costs.

If you are randomized to one of the standard of care therapies, you or your insurance will be responsible for the cost of the medication. If you are randomized to receive eltrombopag, Novartis will cover the cost of the study medication as it is considered "investigational" in this study.

You will not be paid for taking part in this study.

**Research Related Injury**

Please contact your study doctor, if you feel you have been injured as a result of taking part in this study.

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Research personnel will try to reduce, control, and treat any complications from this research. If you are injured because of this study, you will receive medical care that you or your insurance will have to pay for just like any other medical care.

Novartis (drug supplier) will not pay any money to you or your medical bills.

**Subject's Rights**

Your signature on this consent form means that you have received the information about this study and that you agree to volunteer for this research study.

You will be given a copy of this signed form to keep. You are not giving up any of your rights by signing this form. Even after you have signed this form, you may change your mind at any time. Please contact the study staff if you decide to stop taking part in this study.

If you choose not to take part in the research or if you decide to stop taking part later, your benefits and services will stay the same as before this study was discussed with you. You will not lose these benefits, services, or rights.

The investigator, JENNY DESPOTOVIC, and/or someone he/she appoints in his/her place will try to answer all of your questions. If you have questions or concerns at any time, or if you need to report an injury related to the research, you may speak with a member of the study staff: JENNY MCDADE DESPOTOVIC at 832-822-4362 during the day and after hours.

Members of the Institutional Review Board for Baylor College of Medicine and Affiliated Hospitals (IRB) can also answer your questions and concerns about your rights as a research subject. The IRB office number is (713) 798-6970. Call the IRB office if you would like to speak to a person independent of the investigator and research staff for complaints about the research, if you cannot reach the research staff, or if you wish to talk to someone other than the research staff.

**CLINICALTRIALS.GOV**

A description of this clinical trial will be available on <http://www.ClinicalTrials.gov>. This website will not include information that can identify you. At most, the website will include a summary of the results. You can search this website at any time.

**CHILD ASSENT CLAUSE**

If your child is the one invited to take part in this study you are signing to give your permission. Each child may agree to take part in a study at his or her own level of understanding. When you sign this you also note that your child understands and agrees to take part in this study according to his or her understanding.

Please print your child's name here \_\_\_\_\_

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Signing this consent form indicates that you have read this consent form (or have had it read to you), that your questions have been answered to your satisfaction, and that you voluntarily agree to participate in this research study. You will receive a copy of this signed consent form.

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 Subject

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 Date

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 Legally Authorized Representative  
 Parent or Guardian

---

 Date

---

 Investigator or Designee Obtaining Consent

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 Date

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 Witness (if applicable)

---

 Date

---

 Translator (if applicable)

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 Date

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