

PATIENT INFORMATION

ZYNTEGLO® (pronounced *zin-TEH-glo*) (betibeglogene autotemcel)

What is the most important information I should know about ZYNTEGLO?

ZYNTEGLO may cause the following side effects:

- On the day of treatment with ZYNTEGLO
 - Increased heart rate
 - Abdominal pain
- Following treatment for up to 6 months
 - Low level of platelets, which may reduce the ability of blood to clot and may cause bleeding
 - Low level of white blood cells, which may make you more susceptible to infection
 - Pain in arms or legs

Call your healthcare provider right away if you have new or unusual bleeding which may include any of these signs or symptoms:

- Severe headache
- Abnormal bruising
- Nose bleed
- Blood in your urine, stool, or vomit
- Coughing up blood
- Unusual stomach or back pain

You may experience side effects associated with other medicines administered as part of the ZYNTEGLO treatment regimen. Talk to your physician regarding those possible side effects. Your healthcare providers may give you other medicines to treat your side effects.

It is important for you to be monitored at least yearly for at least 15 years for any changes to your blood. There is a potential risk of blood cancer associated with this treatment; however, no cases have been seen in studies of ZYNTEGLO. If you are diagnosed with a cancer, have your treating physician contact Genetix Biotherapeutics at 1-833-999-6378.

What is ZYNTEGLO?

ZYNTEGLO is a one-time gene therapy to treat beta-thalassemia (also known as beta-thalassemia major or Cooley's Anemia) in patients who require regular transfusions. Beta-thalassemia is caused by a change in the beta-globin gene, which causes the body to produce reduced or no beta-globin. ZYNTEGLO is made specifically for each patient, using the patient's own blood stem cells and adds functional copies of the beta-globin gene to your cells. This may allow you to produce sufficient hemoglobin to stop receiving regular transfusions.

How will I get ZYNTEGLO?

Your healthcare providers will give you other medicines, including chemotherapy medicine, as part of your treatment with ZYNTEGLO. It's important to talk to your healthcare provider about the risks and benefits of all medicines involved in your treatment.

After receiving the chemotherapy, it may not be possible for you to become pregnant or father a child. You should consider discussing options for fertility preservation with your doctor before treatment.

STEP 1: ZYNTEGLO is made specifically for you from your own blood stem cells. Your healthcare provider will collect your blood stem cells through a process called mobilization and apheresis (*A-feh-REE-sis*). This process takes approximately one week and may need to be repeated.

'Back-up' stem cells (or 'rescue cells') are also collected and stored at the hospital. This is a precaution in case there is a problem in the treatment process. If this happens, your back-up stem cells will be given back to you. If you receive back-up cells, you will have no benefit from ZYNTEGLO.

STEP 2: Your blood stem cells will be sent to a manufacturing site where they are used to make ZYNTEGLO. It takes approximately 70-90 days from the time your cells are collected to manufacture and test ZYNTEGLO before it is shipped to your healthcare provider, but the time may vary.

STEP 3: Before you receive ZYNTEGLO, your healthcare provider will give you chemotherapy for a few days to make room in the bone marrow. You will be admitted to the hospital for this step and remain in the hospital until after ZYNTEGLO infusion.

STEP 4: ZYNTEGLO is given by an intravenous infusion (into your vein). You may receive more than one bag of ZYNTEGLO. Each bag is infused in 30 minutes or less.

After ZYNTEGLO infusion, you will stay in the hospital for approximately 3-6 weeks so that your healthcare team can closely monitor your recovery. Your healthcare provider will determine when you can go home.

What should I avoid after receiving ZYNTEGLO?

- Do not donate blood, organs, tissues or cells.

What are the possible or reasonably likely side effects of ZYNTEGLO?

The most common side effects of ZYNTEGLO are:

- On the day of treatment with ZYNTEGLO
 - Increased heart rate
 - Abdominal pain
- Following treatment for up to 6 months
 - Low level of platelets, which may reduce the ability of blood to clot and may cause bleeding

- Low level of white blood cells, which may make you more susceptible to infection
- Pain in arms or legs

ZYNTÉGLO will not give you an HIV infection. Treatment with Zynteglo may cause a false-positive human immunodeficiency virus (HIV) test result by some commercial tests. If you need to have an HIV test, talk with your healthcare provider about the appropriate test to use.

These are not all the possible side effects of ZYNTÉGLO. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.

General information about the safe and effective use of ZYNTÉGLO.

It is important that you have regular check-ups with your healthcare provider, including at least annual blood tests, to detect any adverse effects and to confirm that ZYNTÉGLO is still working.

Talk to your healthcare provider about any concerns. You can ask your healthcare provider for information about ZYNTÉGLO that is written for healthcare professionals.

For more information, go to ZYNTÉGLO.com or call 1-833-666-2583 for Patient Services.

Manufactured for: **Genetix Biotherapeutics Inc., Somerville, Massachusetts 02145**

This Patient Information has been approved by the U.S. Food and Drug Administration.

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