

Crizanlizumab

TREATMENT FOR SICKLE CELL DISEASE

Crizanlizumab (pronounced “criz-an-liz-u-mab”), or **ADAKVEO**[®] is a treatment for sickle cell disease (SCD). It may help people ages 16 years and older who live with all types of SCD (If you don’t know the type of SCD you or the person receiving care has, ask your doctor). Share the information in this fact sheet with your health care team to decide whether this drug might be a good choice for you or the person receiving care. You may be able to take this drug even if you are currently taking hydroxyurea, but consult with your health care team. Crizanlizumab has not been studied in women who are pregnant or breastfeeding.



How crizanlizumab works

- For individuals living with SCD, red blood cells are distressed, which makes them stiff, sticky, and sickled-shaped. This stickiness makes blood cells clump and block blood flow in a process called vaso-occlusion, which causes severe SCD pain crises.
- Crizanlizumab works by slowing or stopping red blood cells and other blood cells, like white blood cells and platelets, from sticking to blood vessel walls.
- Crizanlizumab acts like an oil slick, spreading over and between cells, which helps the blood flow and decreases the number of pain crises.



How crizanlizumab is given

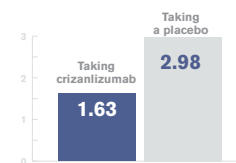
- Crizanlizumab may be administered monthly and is given intravenously (IV), or into the vein, over a period of 30 minutes every four weeks. Talk to your doctor if you have a history of problems with IV treatment.
- This drug may be administered in many settings depending on your health care team and your insurance provider’s policies. You can take this drug at your doctor’s office or an infusion center, or an outpatient day hospital.
- Crizanlizumab is for long-term use. Talk to your health care team about how long will be right for you or the person receiving care.



Research study of crizanlizumab

- During a clinical research study known as the SUSTAIN study, participants ages 16 and over living with SCD who took crizanlizumab reported fewer pain crises than those on placebo (a harmless substance that looks like the treatment but is specifically designed to do nothing).
- Over the course of a year, the participants taking crizanlizumab reported 45% fewer pain crises: participants on crizanlizumab had an average 1.63 pain crises while those on the placebo averaged 2.98 crises.
- Some of the side effects of crizanlizumab (occurring in at least 1 out of 10 people) include reactions to the IV, nausea, fever, back pain, and joint pain. These reactions also occurred in people taking the placebo but in only half as many people.

Average number of crises events after 48 weeks



People experiencing side effects on crizanlizumab



People experiencing side effects taking a placebo



Talk to your health care team about...

- The possible benefits and risks of crizanlizumab for you or the person receiving care
- Whether other health conditions or taking other medications might limit taking this drug
- Past reactions to drugs that may give clues about whether you or the person receiving care could have an allergic reaction to crizanlizumab
- Other treatment options
- How long you or the person receiving care should take crizanlizumab

About This Fact Sheet: This fact sheet has been prepared by the Sickle Cell Disease Coalition and provides general information about crizanlizumab, which is one of the few FDA-approved therapies to treat sickle cell disease. This fact sheet is purely an informational resource and is intended to facilitate discussion with your physician. It does not provide medical advice and is not intended to substitute for consultation with a medical professional. People with sickle cell disease should talk to their doctor before making any changes to their treatment.

For more information about SCD go to www.scdcoalition.org



L-Glutamine

TREATMENT FOR SICKLE CELL DISEASE

L-glutamine (pronounced “L-gloo-ta-meem,”), or **ENDARI**[®] is a treatment for sickle cell disease (SCD) that may help patients age five years and older (If you don’t know the type of SCD you or the person receiving care has, ask your doctor). Share the information in this fact sheet with your health care team to decide whether this drug might be a good choice for you or the person receiving care. You may be able to take this drug even if you are currently taking hydroxyurea, but consult with your health care team. L-glutamine has not been studied in women who are pregnant or breastfeeding.

How L-glutamine works

- L-glutamine is an amino acid. The body naturally produces amino acids to help prevent and fight against infection, injury, and stress. However, people with SCD might need additional L-glutamine because they experience more pain and infection than what the body can handle on its own.
- For individuals living with SCD, red blood cells are distressed, which makes them stiff, sticky, and sickled-shaped. This causes damage that blocks blood flow in a process called vaso-occlusion, which can cause pain crises. L-glutamine helps stop or slow down this damage and works to lower the number of pain crises.

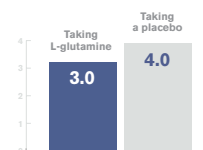
How to take L-glutamine

- L-glutamine is taken in powder form and is intended for long-term use.
- Each dose normally is mixed in eight ounces of cold or room temperature beverage or four to six ounces of food.

Research study of L-glutamine

- In a study looking at the effectiveness of L-glutamine, researchers found that participants assigned to take L-glutamine had fewer pain crises than those on the placebo (a harmless substance that looks like the treatment but is specifically designed to do nothing).
- After 48 weeks, the L-glutamine group had fewer crisis events than the placebo group (median of 3 instead of 4 events).
- People who took L-glutamine went to the hospital less often than the other group (median of 2 instead of 3 hospitalizations).
- L-glutamine may help decrease other sickle cell complications. Please discuss further with your health care team.
- Some of the side effects of L-glutamine (occurring in at least one in 10 people – same as placebo) include constipation, nausea, headache, stomach pain, cough, leg or arm pain, back pain, and chest pain.

Average number of crises events after 48 weeks



People experiencing side effects on L-glutamine or placebo



Talk to your health care team about . . .

- The possible benefits and risks of L-glutamine for you or your child
- Whether other health conditions or taking other medications might limit taking L-glutamine
- Other treatment options
- How long you or the person receiving care should take L-glutamine.

About This Fact Sheet: This fact sheet has been prepared by the Sickle Cell Disease Coalition and provides general information about L-glutamine, which is one of the few FDA-approved therapies to treat sickle cell disease. This fact sheet is purely an informational resource and is intended to facilitate discussion with your physician. It does not provide medical advice and is not intended to substitute for consultation with a medical professional. People with sickle cell disease should talk to their doctor before making any changes to their treatment.

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Voxelotor

TREATMENT FOR SICKLE CELL DISEASE

Voxelotor (pronounced “vox-EL-o-tor”), or **OXBRYTA**[®] is a treatment for sickle cell disease (SCD) that may help people aged 12 years and older (If you don’t know the type of SCD you or the person receiving care has, ask your doctor). Share the information in this fact sheet with your health care team to decide whether this drug might be a good choice for you or the person receiving care. You may be able to take this drug even if you are currently taking hydroxyurea, but consult with your health care team. Voxelotor has not been studied in women who are pregnant or breastfeeding.

How voxelotor works

- Healthy red blood cells contain hemoglobin (Hb), which carries oxygen to all parts of your body.
- For individuals living with SCD, red blood cells lose their round shape and become sickled, or crescent-shaped.
- The sickled red blood cells break apart in a process called hemolysis.
- Voxelotor decreases hemolysis and strengthens hemoglobin’s carrying of oxygen.

How to take voxelotor

- Voxelotor is given as a pill. The typical dose is three pills (1,500 mg) by mouth daily, with or without food but patients should consult with their health care team.
- Tablets should not be crushed.
- Voxelotor is for long-term use. Talk to your health care team about how long will be right for you or the person receiving care.

Research study of voxelotor

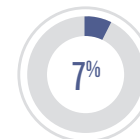
- During a clinical study known as the HOPE study, 50% of participants between the ages of 12 and 64 years were found with increased Hb levels (i.e. in two to four weeks), whereas only 7% of those given the placebo (a harmless substance that looks like the treatment but is specifically designed to do nothing) had increased Hb levels.
- After 24 weeks, the group given voxelotor also showed fewer hemolysis events than the placebo group.
- Side effects of voxelotor (occurring in at least one in 10 people - same as placebo) include headache, diarrhea, stomach pain, nausea, tiredness, rash, or fever.

People with increased HB levels

Taking voxelotor



Taking a placebo



People experiencing side effects on voxelotor or placebo



Talk to your health care team about . . .

- The possible benefits and risks of voxelotor for you or the person receiving care
- Whether other health conditions or taking other medications might affect taking voxelotor
- Past reactions to drugs that may give clues about whether you or your child could have an allergic reaction to voxelotor
- Other treatment options
- How long you or the person receiving care should take voxelotor

About This Fact Sheet: This fact sheet has been prepared by the Sickle Cell Disease Coalition and provides general information about voxelotor, which is one of the few FDA-approved therapies to treat sickle cell disease. This fact sheet is purely an informational resource and is intended to facilitate discussion with your physician. It does not provide medical advice and is not intended to substitute for consultation with a medical professional. People with sickle cell disease should talk to their doctor before making any changes to their treatment.

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