FACT SHEET

Oxbryta® (voxelotor)
500mg tablets • 300mg tablets for oral suspension

Approved by the U.S. Food and Drug Administration (FDA) for the treatment of sickle cell disease (SCD) in patients as young as 4 years of age

What is Oxbryta®?

Oxbryta (voxelotor) is a prescription medicine used for the treatment of sickle cell disease in adults and children 4 years of age and older. It is not known if Oxbryta is safe and effective in children below 4 years of age.

This indication is approved under accelerated approval based on increase in hemoglobin (Hb). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

Oxbryta comes in two dosage forms for patients 4 years and older based on the patient's age, weight, and ability to swallow tablets:

1. Oxbryta 500 mg tablets for ages 12 years and older, as well as patients ages 4 to less than 12 years who weigh more than 88 lbs (40 kg) and can swallow whole tablets.

2. Oxbryta 300 mg tablets for oral suspension for patients ages 4 to less than 12 who weigh between 22 to 88 lbs (10 to 40 kg) and for patients who have difficulty swallowing whole tablets. The dispersible tablet form includes grape flavoring and is intended to be dispersed in room-temperature clear drinks for ease of swallowing.

What is Sickle Cell Disease?

Sickle cell disease (SCD) is an inherited blood disorder that affects millions of people throughout the world, including an estimated 100,000 people in the U.S., approximately 16,000 of which are children ages 4 to 11. SCD most commonly affects those with ancestors from sub-Saharan Africa, and also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.

With SCD, the hemoglobin in the red blood cell releases oxygen, causing it to clump together (polymerize) and change into a sickle, or banana, shape.

How Does Oxbryta Work?

Oxbryta inhibits sickle hemoglobin polymerization, the process that causes red blood cells to deform and become sickle shaped.

How Was Oxbryta Studied?

Oxbryta 500 mg tablets and Oxbryta 300 mg tablets for oral suspension were studied in two different age groups in two clinical trials: HOPE and HOPE-KIDS 1. The trials studied similar target goals and showed similar results.

HOPE Trial: Oxbryta 500 mg tablets were studied in 90 patients who received Oxbryta (daily dose of 1,500 mg) and 92 patients who received a placebo (sugar pill). The trial included patients ages 12 and up.

HOPE-KIDS 1 Trial: Oxbryta 300 mg tablets for oral suspension were studied in 45 patients ages 4 to less than 12 (daily dose based on body weight). The trial also included 11 patients ages 12-17 who received Oxbryta 500 mg tablets (daily dose of 1,500 mg).

Important Safety Information
The Prescribing Information for Oxbryta includes Warnings and Precautions for hypersensitivity reactions.
Please see additional Important Safety Information on the next page and the U.S. Full Prescribing Information for Oxbryta at Oxbryta.com

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IMPORTANT SAFETY INFORMATION

Oxbryta® (voxelotor)
500mg tablets • 300mg tablets for oral suspension

INDICATION

What is OXBRYTA?
OXBRYTA is a prescription medicine used for the treatment of sickle cell disease in adults and children 4 years of age and older.

It is not known if OXBRYTA is safe and effective in children with sickle cell disease below 4 years of age.

This indication is approved under accelerated approval based on increase in hemoglobin (Hb). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

IMPORTANT SAFETY INFORMATION

Do not take OXBRYTA if you or your child have had an allergic reaction to voxelotor or any of the ingredients in OXBRYTA. See the end of the patient leaflet for a list of the ingredients in OXBRYTA.

Before taking OXBRYTA, tell your healthcare provider about all of your medical conditions, including if you or your child:

- have liver problems
- are pregnant or plan to become pregnant. It is not known if OXBRYTA can harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if OXBRYTA can pass into your breastmilk and if it can harm your baby. Do not breastfeed during treatment with OXBRYTA and for at least 2 weeks after the last dose.

Tell your healthcare provider about all the medicines you or your child take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Some medicines may affect how OXBRYTA works. OXBRYTA may also affect how other medicines work and may affect the results of certain blood tests. Keep a list of all your medicines and show it to your healthcare provider.

What should I avoid while taking OXBRYTA?
Do not take St. John's wort during treatment with OXBRYTA.

What are the possible side effects of OXBRYTA?
OXBRYTA can cause serious side effects, including:
Serious allergic reactions. Tell your healthcare provider or get emergency medical help right away if you get:

- rash
- hives
- shortness of breath (difficult breathing)
- swelling of the face

The most common side effects of OXBRYTA include:

- headache
- diarrhea
- stomach-area (abdominal) pain
- nausea
- rash or hives
- fever

The most common side effects of OXBRYTA in children ages 4 to less than 12 years of age include:

- fever
- vomiting
- rash
- stomach-area (abdominal) pain
- diarrhea
- headache

These are not all the possible side effects of OXBRYTA.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.
You may also report side effects to Global Blood Therapeutics at 1-833-428-4968 (1-833-GBT-4YOU).

Keep OXBRYTA and all medicines out of the reach of children.

Please see U.S. Full Prescribing Information on Oxbryta.com.

GBT Media Contact:
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To learn more about Oxbryta, visit Oxbryta.com.
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