An Open-label, Expanded Access Protocol for Pediatric Patients With Sickle Cell Disease Who Have No Alternative Treatment Options

Overview

- Global Blood Therapeutics (GBT) is a biopharmaceutical company dedicated to the discovery, development, and delivery of treatments that provide hope to underserved patient communities. The company has introduced Oxbryta® (voxelotor) tablets, which was approved by the FDA in November 2019. **OXBRYTA is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older. It is not known if OXBRYTA is safe and effective in children below 12 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).**

- As part of GBT's continued commitment to the sickle cell community, we have developed an Expanded Access Program (EAP) to provide patient access to Oxbryta in the United States for eligible pediatric patients ages 4 to 11 years with SCD.

- EAPs allow access to investigational treatments to patients who, in their doctor's viewpoint, have an urgent need, are unable to participate in clinical studies, and don't have other treatment options. Access to EAPs is based on meeting specific inclusion/eligibility criteria based on the patient's disease state.

- Numerous clinical sites across the United States will be participating in the EAP. As those sites complete their internal approval processes, they will enroll eligible patients. To find the latest information about this EAP, go to clinicaltrials.gov at https://clinicaltrials.gov/ct2/show/NCT04724421.

FAQs

Q1. Why is GBT conducting an EAP for pediatric SCD patients in the United States?

As part of our commitment to the sickle cell community, GBT has started an EAP, which is intended to help eligible pediatric patients ages 4 to 11 years who need treatment and do not qualify for GBT's existing clinical studies. Patients can get

access to GBT's medicine voxelotor prior to potential FDA approval of this indication.

Q2. How old do patients need to be to be considered for the EAP?
The EAP is open to patients with SCD who are ages 4 to 11 years.

Q3. How will voxelotor be administered to patients enrolled in the EAP?
Pediatric patients who are eligible for participation in this EAP will be treated with voxelotor, dispersible tablets or powder for oral suspension administered orally once daily at a dose that is based on the patient's weight.

Q4. Will patients who enroll in the EAP for voxelotor need to pay for it?
There is no cost associated with voxelotor for patients enrolled in the EAP.

Q5. Is this program exclusive to U.S. patients, or can patients outside of the U.S. apply for treatment through the EAP?
This pediatric EAP is only open to U.S. patients.

Q6. Where are the sites located that are currently actively enrolling patients in the EAP?
To find sites that are currently enrolling patients in the EAP, you can go to ClinicalTrials.gov [Identifier: NCT04724421] or if you still have further questions you can call 1-833-GBT-4YOU (1-833-428-4968) or go to the GBT Medical Information web portal: https://gbtmedinfo.com/.

Q7. What if there isn't an EAP site near a patient?
Patients who meet the EAP inclusion criteria and don't live near an EAP site can have their doctor contact GBT on behalf of the patient at 1-833-GBT-4YOU (1-833-428-4968) or via the GBT Medical Information web portal: https://gbtmedinfo.com/.

Q8. Where can physicians find more information to enroll patients in the EAP?
Information and requests for participation in the EAP must be submitted by the treating physician to the participating sites in the program. To find the latest information about this EAP, go to clinicaltrials.gov at https://clinicaltrials.gov/ct2/show/NCT04724421.

Q9. Can patients participate in the EAP instead of participating in a GBT clinical study?
The EAP is open to pediatric patients age 4 to 11 years who do not qualify for any ongoing GBT studies of voxelotor and who exhibit medical necessity as determined by EAP study inclusion criteria.

Q10. What specific enrollment criteria must patients meet to qualify for the EAP?
Eligibility for the EAP is based on meeting specific inclusion/eligibility criteria based on the patient's disease state. Details about the EAP enrollment criteria

are on ClinicalTrials.gov and can be found at https://clinicaltrials.gov/ct2/show/NCT04724421.

INDICATION AND IMPORTANT SAFETY INFORMATION[1]

What is OXBRYTA (voxelotor) tablets?

OXBRYTA is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older.

It is not known if OXBRYTA is safe and effective in children below 12 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).

Do not take OXBRYTA if you 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.

If you are receiving exchange transfusions, talk to your healthcare provider about possible difficulties with the interpretation of certain blood tests when taking OXBRYTA.

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

- 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 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.

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
- swelling of the face

The most common side effects of OXBRYTA (voxelotor) tablets include:

- headache
- diarrhea
- stomach (abdominal) pain
- nausea
- tiredness
- rash
- fever

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.

1. Patient Information Leaflet 11 2019
2. USPI 11 2019