

## Oxbryta<sup>™</sup> (voxelotor) – New orphan drug approval

- On November 25, 2019, the <u>FDA announced</u> the approval of <u>Global Blood Therapeutics</u>' <u>Oxbryta</u> (<u>voxelotor</u>), for the treatment of sickle cell disease (SCD) in adults and pediatric patients 12 years of age and older.
  - This indication is approved under accelerated approval based on increase in hemoglobin.
    Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).
- SCD is an inherited blood disorder that impacts hemoglobin. Due to a genetic mutation, people with SCD form abnormal hemoglobin known as sickle hemoglobin (hemoglobin S). Through a process called hemoglobin polymerization, red blood cells become sickled – deoxygenated, crescent-shaped and rigid. The sickling process causes hemolytic anemia and blockages in capillaries and small blood vessels, which impede the flow of blood and oxygen throughout the body.
  - SCD affects an estimated 100,000 people in the U.S.
- Oxbryta is a novel hemoglobin S polymerization inhibitor. By increasing the affinity of hemoglobin for oxygen, Oxbryta demonstrates dose-dependent inhibition of hemoglobin S polymerization.
   Nonclinical studies suggest that Oxbryta may inhibit red blood cell sickling, improve red blood cell deformability, and reduce whole blood viscosity.
- The efficacy of Oxbryta was established in the HOPE trial, a randomized, double-blind, placebo-controlled study in 274 patients with SCD. Patients received daily Oxbryta 1,500 mg, Oxbryta 900 mg, or placebo. Efficacy was based on hemoglobin response rate defined as a hemoglobin increase of > 1 g/dL from baseline to week 24 in patients treated with Oxbryta 1,500 mg vs. placebo.
  - The response rate for Oxbryta 1,500 mg was 51.1% (46/90) vs. 6.5% (6/92) in the placebo group (p < 0.001).</li>
- Warnings and precautions for Oxbryta include hypersensitivity reactions and laboratory test interference.
- The most common adverse reactions (> 10%) with Oxbryta use were headache, diarrhea, abdominal pain, nausea, fatigue, rash, and pyrexia.
- The recommended dose of Oxbryta is 1,500 mg taken orally once daily with or without food.
- Global Blood Therapeutics plans to launch Oxbryta within two weeks. Oxbryta will be available as a 500 mg tablet.



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