Oxbryta® (voxelotor) – Expanded indication, new formulation approval

- On December 17, 2021, Global Blood Therapeutics announced the FDA approval of Oxbryta (voxelotor), for the treatment of sickle cell disease (SCD) in adults and pediatric patients 4 years of age and older.
    - Oxbryta was previously approved for this indication in patients 12 years of age and older.
    - 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).
- In addition to the expanded indication, the FDA approved a new 300 mg tablet for oral suspension formulation of Oxbryta.
    - Oxbryta was previously approved as a 500 mg tablet.
- The approval of Oxbryta for the expanded indication was based on an open-label study in 45 patients 4 to < 12 years of age with SCD. Patients received Oxbryta tablets for oral suspension based on body weight at baseline. Eligible patients on stable doses of hydroxyurea for at least 90 days were allowed to continue hydroxyurea therapy throughout the study. Efficacy was based on Hb response rate, which is defined as a Hb increase of > 1 g/dL from baseline to week 24.
    - Hb response rate in patients aged 4 to < 12 years who took at least one dose of Oxbryta was 36% (95% CI: 21.6, 49.5).
- The most common adverse reactions (> 10%) with Oxbryta use in pediatric patients 4 to < 12 years of age were pyrexia, vomiting, rash, abdominal pain, diarrhea, and headache.
- For pediatric patients 4 years and older, the appropriate product (Oxbryta tablets or Oxbryta tablets for oral suspension) should be selected based on patient's ability to swallow tablets and patient weight. The recommended dose (once daily) based on weight is provided below:
    - 40 kg or greater: 1,500 mg once daily
    - 20 kg to less than 40 kg: 900 mg once daily
    - 10 kg to less than 20 kg: 600 mg once daily
- Refer to the Oxbryta drug label for dosing information in adults and pediatric patients 12 years and older.