

## FDA approves novel treatment for sickle cell disease abnormalities

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The U.S. Food and Drug Administration granted accelerated approval to Oxbryta (voxelotor) for the treatment of sickle cell disease (SCD) in adults and pediatric patients 12 years of age and older.

"Today's approval provides additional hope to the 100,000 people in the U.S., and the more than 20 million globally, who live with this debilitating blood disorder," said Acting FDA Commissioner Adm. Brett P. Giroir, M.D. "Our scientific investments have brought us to a point where we have many more tools available in the battle against sickle cell disease, which presents daily challenges for those living with it. We remain committed to raising the profile of this disease as a public health priority and to approving new therapies that are proven to be safe and effective. Together with improved provider education, patient empowerment, and improved care delivery systems, these newly approved drugs have the potential to immediately impact people living with SCD."

Nonclinical studies have demonstrated that Oxbryta inhibits red blood cell sickling, improves red blood cell deformability (ability of a red blood cell to change shape) and improves the blood's ability to flow.

"Oxbryta is an inhibitor of deoxygenated sickle hemoglobin polymerization, which is the central abnormality in sickle cell disease," said Richard Pazdur, M.D., director of the FDA's Oncology Center of Excellence and acting director of the Office of Oncologic Diseases in the FDA's Center for Drug Evaluation and Research. "With Oxbryta, sickle cells are less likely to bind together and form the sickle shape, which can cause low hemoglobin levels due to red blood cell destruction. This therapy provides a new treatment option for patients with this serious and life-threatening condition."

Oxbryta's approval was based on the results of a clinical trial with 274 patients with sickle cell disease. In the study, 90 patients received 1500 mg of Oxbryta, 92 patients received 900 mg of Oxbryta and 92 patients received a placebo. Effectiveness was based on an increase in hemoglobin response rate in patients who received 1500 mg of Oxbryta, which was 51.1% for these patients compared to 6.5% in the placebo group.

Oxbryta was granted Accelerated Approval, which enables the FDA to approve drugs for serious conditions to fill an unmet medical need based on a result that is reasonably likely to predict a clinical benefit to patients. Further clinical trials are required to verify and describe Oxbryta's clinical benefit.

The FDA granted this application Fast Track designation. Oxbryta also received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases. The FDA granted the approval of Oxbryta to Global Blood Therapeutics.