FDA News Release

FDA approves new treatment for sickle cell disease

First approval for this rare blood disorder in nearly 20 years

For Immediate Release

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Release

The U.S. Food and Drug Administration today approved Endari (L-glutamine oral powder) for patients age five years and older with sickle cell disease to reduce severe complications associated with the blood disorder.

"Endari is the first treatment approved for patients with sickle cell disease in almost 20 years," said Richard Pazdur, M.D., acting director of the Office of Hematology and Oncology Products in the FDA's Center for Drug Evaluation and Research and director of the FDA's Oncology Center of Excellence. "Until now, only one other drug was approved for patients living with this serious, debilitating condition."

Sickle cell disease is an inherited blood disorder in which the red blood cells are abnormally shaped (in a crescent, or "sickle," shape). This restricts the flow in blood vessels and limits oxygen delivery to the body's tissues, leading to severe pain and organ damage. According to the National Institutes of Health, approximately 100,000 people in the United States have sickle cell disease. The disease occurs most often in African-Americans, Latinos and other minority groups. The average life expectancy for patients with sickle cell disease in the United States is approximately 40 to 60 years.

The safety and efficacy of Endari were studied in a randomized trial of patients ages five to 58 years old with sickle cell disease who had two or more painful crises within the 12 months prior to enrollment in the trial. Patients were assigned randomly to treatment with Endari or placebo, and the effect of treatment was evaluated over 48 weeks. Patients who were treated with Endari experienced fewer hospital visits for pain treated with a parenterally administered narcotic or ketorolac (sickle cell crises), on average, compared to patients who received a placebo (median 3 vs. median 4), fewer hospitalizations for sickle cell pain (median 2 vs. median 3), and fewer days in the hospital (median 6.5 days vs. median 11 days). Patients who received Endari also had fewer occurrences of acute chest syndrome (a life-threatening complication of sickle cell disease) compared with patients who received a placebo (8.6 percent vs. 23.1 percent).

Common side effects of Endari include constipation, nausea, headache, abdominal pain, cough, pain in the extremities, back pain and chest pain.

Endari received **Orphan Drug designation (/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm)** for this use, which provides incentives to assist and encourage the development of drugs for rare diseases. In addition, development of this drug was in part supported by the FDA Orphan Products Grants Program, which provides grants for clinical studies on safety and/or effectiveness of products for use in rare diseases or conditions.

The FDA granted the approval of Endari to Emmaus Medical Inc.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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Related Information

- **FDA: Office of Hematology and Oncology Products (/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm091745.htm)**
- **FDA: Approved Drugs: Questions and Answers (/Drugs/ResourcesForYou/Consumers/ucm054420.htm)**
- **NIH: Sickle Cell Disease (https://www.nhlbi.nih.gov/health/health-topics/topics/sca)**

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