

Endari™ (L-glutamine) – New orphan drug approval

- On July 7, 2017, the [FDA announced](#) the approval of Emmaus Medical's [Endari \(L-glutamine\)](#), to reduce the acute complications of sickle cell disease (SCD) in adult and pediatric patients ≥ 5 years of age.
- SCD is an inherited blood disorder in which red blood cells (RBCs) are abnormally shaped as a crescent or “sickle”. This restricts the flow in blood vessels, limits oxygen delivery to tissues, and may lead to severe pain and organ damage.
 - An estimated 100,000 Americans have SCD.
 - The average life expectancy for patients with SCD in the U.S. is approximately 40 to 60 years.
- Endari contains L-glutamine, an amino acid. While the mechanism of L-glutamine in treating SCD is not fully understood, it may increase the availability of reduced glutathione and lower the oxidative damage to RBCs.
- The safety and efficacy of Endari were based on a placebo-controlled trial in 230 patients 5 to 58 years of age with SCD who had two or more painful crises within the 12 months prior to trial enrollment. Efficacy was determined by a reduction in the number of sickle cell crises, defined as a visit to the emergency room/medical facility for SCD-related pain which was treated with a parenterally administered narcotic or parenterally administered [ketorolac](#). In addition, occurrence of chest syndrome, priapism, and splenic sequestration were considered sickle cell crises.
 - Patients treated with Endari vs. those treated with placebo experienced fewer sickle cell crises (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).
 - Endari-treated patients also had fewer occurrences of acute chest syndrome vs. placebo-treated patients (8.6% vs. 23.1%).
- The most common adverse reactions (> 10%) with Endari use were constipation, nausea, headache, abdominal pain, cough, pain in extremity, back pain, and chest pain.
- The recommended dosage of Endari is administered orally twice daily based on body weight. See table below.

Weight (kg)	Weight (lbs)	Per dose	Per day	Packets/dose	Packets/day
< 30	< 66	5 gm	10 gm	1	2
30 – 65	66 – 143	10 gm	20 gm	2	4
> 65	> 143	15 gm	30 gm	3	6

- Each dose of Endari should be mixed before ingestion in 8 oz. (240 mL) of cold or room temperature beverage such as water, milk, or apple juice, or 4 oz. to 6 oz. of food such as applesauce or yogurt.
- Complete dissolution is not required prior to administration.
- Emmaus Medical's launch plans for Endari are pending. Endari will be available as an oral powder containing 5 gm of L-glutamine in paper-foil-plastic laminate packets.