



**BlueCross  
BlueShield**

Federal Employee Program.

## **ENDARI (L-glutamine oral powder)**

### **RATIONALE FOR INCLUSION IN PA PROGRAM**

#### **Background**

Endari is used in the treatment for patients with sickle cell disease to reduce severe complications associated with the blood disorder. 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 (1).

#### **Regulatory Status**

FDA-approved indication: Endari is an amino acid indicated to reduce the acute complications of sickle cell disease (SCD) in adult and pediatric patients 5 years of age and older (1).

Two effective disease-modifying therapies for SCD (hydroxyurea and chronic transfusion) are potentially widely available but remain underutilized. These are the only currently proven disease-modifying treatments for people with SCD. Both therapies are used in primary and secondary stroke prevention. Although neither has been shown to prevent all SCD-related organ damage, these treatment modalities can improve the quality of life for individuals with SCD (2).

The safety and effectiveness of Endari have been established in pediatric patients 5 years and older (1).

#### **Summary**

Endari is used in the treatment for patients with sickle cell disease to reduce severe complications associated with the blood disorder. 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. Two effective disease-modifying therapies for SCD (hydroxyurea and chronic transfusion) are potentially widely available but remain underutilized. These are the only currently proven disease-modifying treatments for people with SCD (1).

Prior approval is required to ensure the safe, clinically appropriate and cost-effective use of Endari while maintaining optimal therapeutic outcomes.



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**References**

1. Endari [Package Insert]. Torrance, CA: Emmaus Medical Inc.; October 2020.
2. Gibbons G, Shurin S, et al. Evidence-Based Management of Sickle Cell Disease: Expert Panel Report (EPR), 2014. U.S. Department of Health and Human Services National Institutes of Health.