Endari

Description

Endari (L-glutamine oral powder)

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).
Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Endari may be considered medically necessary in patients that are 5 years of age and older with sickle cell disease (SCD) and if the conditions indicated below are met.

Endari is considered investigational in patients less than 5 years of age and for all other indications.

Prior-Approval Requirements

Age 5 years of age or older

Diagnosis

Patient must have the following:

Sickle Cell Disease (SCD)

AND the following:

1. Inadequate treatment response, intolerance, or contraindication (i.e. renal, cardiovascular, GI) to a 3 month trial of generic hydroxyurea

Prior – Approval Renewal Requirements

Age 5 years of age or older

Diagnosis

Patient must have the following:

Sickle Cell Disease (SCD)
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AND the following:
1. Reduction in the number of acute complications (i.e. blood transfusions, sickle cell crisis's, hospitalizations) of sickle cell disease since initiating therapy

### Policy Guidelines

#### Pre - PA Allowance

None

#### Prior - Approval Limits

**Duration** 12 months

#### Prior – Approval *Renewal* Limits

**Duration** 24 months

### Rationale

**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.

**References**

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Policy History

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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 13, 2019 and is effective on October 1, 2019.