Draft New Policy: Do Not Implement

Pegzilarginase-nbln (Loargys)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Loargys is indicated for the treatment of hyperargininemia in adult and pediatric patients two years of age and older with Arginase 1 Deficiency (ARG1-D), in conjunction with dietary protein restriction.

This indication is approved under accelerated approval based on reduction of plasma arginine. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
Initial Requests

- Lab results documenting elevated plasma arginine levels (i.e., greater than or equal to 250 micromol/L).
- Chart notes, medical records, or lab results documenting one of the following:
  - Pathogenic (or likely pathogenic) variant in the ARG1 gene.
  - Enzyme assay demonstrating a deficiency of arginase enzyme activity (i.e., <1% of normal) in erythrocytes.
- Clinical documentation establishing baseline functional status, including measurements or qualitative descriptions of spasticity, mobility or ambulation

Continuation Requests

- Lab results documenting pre-dose plasma arginine levels.
- Clinical documentation of follow-up functional assessment scores, including but not limited to results from the 2-Minute Walk Test (2MWT), Gross Motor Function Measure (GMFM-E and/or GMFM-D) evaluations, etc.

PRESCRIBER SPECIALITIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of enzyme or metabolic disorders.

COVERAGE CRITERIA

Arginase 1 Deficiency (ARG1-D)

Authorization of 12 months may be granted for treatment of ARG1-D when all of the following criteria are met:

- The member is 2 to less than 32 years of age.
- The member has elevated plasma arginine levels (i.e., greater than or equal to 250 micromol/L) prior to initiating therapy with the requested medication.
- The diagnosis of ARG1-D is confirmed by one of the following:
  - Pathogenic (or likely pathogenic) variant in the ARG1 gene.
  - Enzyme assay demonstrating a deficiency of arginase enzyme activity (i.e., <1% of normal) in erythrocytes.
- The requested medication will be used in conjunction with dietary protein restriction.
- The member has not had active infection requiring anti-infective therapy within 3 weeks of initiating treatment with the requested medication.
- The member does not have known, active infection with human immunodeficiency virus (HIV), hepatitis B, or hepatitis C.
- The member does not have a history of hypersensitivity to polyethylene glycol that, in the opinion of the provider, puts the member at unacceptable risk for adverse events.
- The member has not received previous liver or hematopoietic transplant procedure.
- Baseline and subsequent pre-dose plasma arginine levels will be collected and monitored as outlined in the manufacturer's prescribing information.
- The dose of the requested medication will be adjusted as outlined in the manufacturer's prescribing information if the member's pre-dose plasma arginine levels fall outside of the therapeutic range (i.e., 50 micromol/L to 150 micromol/L).
- Initial and subsequent doses of the requested medication will not exceed 0.2 mg/kg once weekly.
- Baseline functional status was assessed

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section when all of the following criteria are met:

- There is no evidence of unacceptable toxicity or disease progression while on the current regimen.
- The member has not received liver or hematopoietic transplant procedure.
- Either of the following criteria apply:
  - The member has achieved a pre-dose plasma arginine level between 50 micromol/L and 150 micromol/L.
  - The member has not achieved a pre-dose plasma arginine level between 50 micromol/L and 150 micromol/L, and the dose of the requested medication will be adjusted as outlined in the manufacturer's prescribing information.
- The member shows stability or improvement in net motor function or developmental milestones, including but not limited to:
  - Improvement or maintenance on the 2-Minute Walk Test (2MWT)
  - Improvement or maintenance in Gross Motor Function Measure (GMFM-E and/or GMFM-D) scores
  - Clinical documentation of stabilization or improvement in spasticity, mobility or ambulation relative to baseline
- The requested dose does not exceed 0.2 mg/kg once weekly.

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

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EFFECTIVE DATE

ID_BT_2026