CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Loargys (pegzilarginase-nbln)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Loargys, approved by the FDA in 2026, is an arginine specific enzyme indicated for the treatment of hyperargininemia in adult and pediatric patients 2 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 in the phase 3 PEACE trial. Loargys also demonstrated clinically relevant functional mobility improvements as secondary outcomes.

The urea cycle clears nitrogen waste from the body as urea. Urea cycle disorders (UCDs) are rare inherited deficiencies in any of the enzymes involved in the urea cycle. The enzyme deficiency makes ureagenesis defective and causes ammonia to accumulate.

ARG1-D, caused by mutations of the *ARG1* gene, is one of the rarest UCDs and affects the last step of the pathway. Deficient ARG1 enzyme leads to elevated plasma arginine, resulting in neurologic manifestations including the hallmark feature of lower limb spasticity. Standard of care is dietary protein restriction. Nitrogen scavenger drugs do not lower arginine levels. Loargys provides an exogenous source of ARG1 to reduce plasma arginine.

Loargys (pegzilarginase-nbln) will be considered for coverage when the following criteria are met:

Arginase 1 Deficiency (ARG1-D)

For **initial** authorization:

1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with a metabolic specialist, geneticist, or neurologist; AND
3. Member has a diagnosis of ARG1-D confirmed by at least one of the following:
   a) Genetic test results that show biallelic pathogenic variants in *ARG1*
   b) Enzyme assay results that show reduced RBC arginase activity (<1% of normal); AND
4. Chart notes must document baseline plasma arginine (pArg) concentration of ≥250 µmol/L despite adherent dietary protein restriction; AND
5. Medication is prescribed in conjunction with continued dietary protein restriction.
6. **Dosage allowed/Quantity limit:**
   Starting dose: 0.1 mg/kg by intravenous infusion once weekly
   Max dose: 0.2 mg/kg once weekly
   Titrate and maintain per plasma arginine level and prescribing information.
   May switch to subcutaneous dosing after 8 weeks.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show a clinically significant reduction of plasma arginine levels from baseline. (Normal range is 40 to 115 µmol/L; goal is <200 µmol/L).

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Loargys (pegzilarginase-nbln) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
03/17/2026	New policy created for Loargys.

References:

1. Loargys [prescribing information]. Immedica Pharma AB; 2026.
2. Russo RS, Gasperini S, Bubb G, et al. Efficacy and safety of pegzilarginase in arginase 1 deficiency (PEACE): a phase 3, randomized, double-blind, placebo-controlled, multi-centre trial. *EClinicalMedicine*. 2024;68:102405. Published 2024 Jan 12. doi:10.1016/j.eclinm.2023.102405
3. Häberle J, Boddaert N, Burlina A, et al. Suggested guidelines for the diagnosis and management of urea cycle disorders. *Orphanet J Rare Dis*. 2012;7:32. Published 2012 May 29. doi:10.1186/1750-1172-7-32
4. Häberle J, Burlina A, Chakrapani A, et al. Suggested guidelines for the diagnosis and management of urea cycle disorders: First revision. *J Inherit Metab Dis*. 2019;42(6):1192-1230. doi:10.1002/jimd.12100 9.
5. Sun A, Crombez EA, Wong D. Arginase Deficiency. 2004 Oct 21 [Updated 2020 May 28]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1159/

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