

Oxlumo® (lumasiran) Injection

When requesting Oxlumo® (lumasiran) injection, the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved Indication

- For the treatment of primary hyperoxaluria type 1 (PH1) to lower urinary oxalate levels in pediatric and adult patients

Coverage Guidelines

Primary Hyperoxaluria Type 1

The individual must meet **all** of the following criteria for initial approval:

- Patient has had a genetic test confirming diagnosis of Primary Hyperoxaluria Type 1 via identification of an alanine:glyoxylate aminotransferase gene (AGXT) mutation; AND
- Patient has elevated urine oxalate excretion as demonstrated by ONE of the following:
 - Urinary oxalate excretion greater than or equal to 0.7 mmol/24 hours/1.73 meters²; OR
 - Urinary oxalate:creatinine ratio above the age-specific upper limit of normal; AND
- Patient has not previously received a liver transplant for Primary Hyperoxaluria Type 1; AND
- The medication is prescribed by or in consultation with a nephrologist or urologist.

Approval duration: 6 months

The individual must meet **all** of the following criteria for renewal:

- The patient is continuing to derive benefit from Oxlumo as determined by the most recent (i.e., within the past 6 months) objective measurement
 - NOTE: Objective measurements of a response to Oxlumo therapy are reduced urinary oxalate excretion, decreased urinary oxalate:creatinine ratio, reduced plasma oxalate levels from baseline (i.e., prior to Oxlumo therapy), or improved/stabilized clinical signs/symptoms of Primary Hyperoxaluria Type 1 (e.g., nephrocalcinosis, formation of renal stones, renal impairment)

Approval duration (renewal): 12 months

Dosing Recommendation

Initially, approve up to 6 mg/kg administered subcutaneously not more frequently than once every month for three doses; AND/OR

For maintenance dosing, approve 3 mg/kg administered subcutaneously not more frequently than once every month or 6 mg/kg administered subcutaneously not more frequently than once every 3 months.

References

1. Oxlumo™ injection for subcutaneous use [prescribing information]. Cambridge, MA: Alnylam Pharmaceuticals, Inc.; November 2020.
2. Garrelfs SF, Frishberg Y, Hulton SA, et al. ILLUMINATE-A, a phase III study of lumasiran, an investigational RNAi therapeutic, in children and adults with primary hyperoxaluria type 1 (PH1) [presentation LB002]. Presented at: the European Renal Association-European Dialysis and Transplant Association (ERA-EDTA) International Congress; virtual meeting; June 6-9, 2020.
3. Deschenes G, Cochat P, Magen D, et al. ILLUMINATE-B, a phase 3 open-label study to evaluate lumasiran, an RNAi therapeutic, in young children with primary hyperoxaluria type 1 (PH1) [presentation PO1624]. Presented at: the American Society of Nephrology (ASN) Kidney Week; virtual meeting; October 22-25, 2020.
4. Adam MP, Ardinger HH, Pagon RA, et al., editors. Primary Hyperoxaluria Type 1. Gene Reviews® Available at: <https://www.ncbi.nlm.nih.gov/books/NBK1283/>. Updated November 30, 2017. Accessed on December 2, 2020.
5. Primary Hyperoxaluria: MedlinePlus Genetics. U.S. National Library of Medicine; National Institutes of Health; Department of Health and Human Services. Available at: <https://medlineplus.gov/genetics/condition/primary-hyperoxaluria/#resources>. Accessed on December 2, 2020.

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