Oxlumo (lumasiran)

Override(s)	Approval Duration
Prior Authorization	Initial Approval: 6 months Continuation Approval: 1 year

Medications	Body Weight	Loading Dose	Maintenance Dose (starting one month after the last loading dose)
Oxlumo (lumasiran) 94.5 mg/0.5 mL vial	Less than 10 kg	6 mg/kg once monthly for 3 doses	3 mg/kg once monthly
	10 kg to less than 20 kg	6 mg/kg once monthly for 3 doses	6 mg/kg once every 3 months
	20 kg and above	3 mg/kg once monthly for 3 doses	3 mg/kg once every 3 months

APPROVAL CRITERIA

Initial requests for Oxlumo (lumasiran) may be approved if the following criteria are met:

I. Individual has a diagnosis of primary hyperoxaluria type 1 (PH1); **AND**
II. Documentation is provided that diagnosis has been verified by (Cochat 2012; Milliner 2022):
    A. Genetic testing demonstrating mutation in the alanine:glyoxylate aminotransferase (*AGXT)* gene; **OR**
    B. Liver biopsy demonstrating significantly decreased or absent alanine:glyoxylate aminotransferase (AGT) enzyme activity; **AND**
III. Documentation is provided that individual has elevated urinary oxalate levels or plasma oxalate levels; **AND**
IV. Individual is using in combination with pyridoxine (unless individual is a pyridoxine non-responder) Cochat 2012; Milliner 2022).

Continuation requests for Oxlumo (lumasiran) may be approved if the following criteria are met:

I. Documentation is provided that there is clinically significant reduction in urinary oxalate excretion, spot urinary oxalate:creatinine ratio or plasma oxalate levels with Oxlumo therapy; **AND**
II. Individual is using in combination with pyridoxine (unless individual is a pyridoxine non-responder) (Cochat 2012; Milliner 2022).

Oxlumo (lumasiran) may not be approved for the following:

I. Individual with primary hyperoxaluria type 2 or type 3; **OR**
II. Individual with a history of or planned kidney or liver transplant (NCT 03681184, NCT 03905694, NCT 04152200); **OR**
III. Use in combination with Rivfloza (nedosiran); **OR**
IV. May not be approved when the above criteria are not met and for all other indications.

Key References:

1. Alnylam Pharmaceuticals. A study of lumasiran in infants and young children with primary hyperoxaluria type 1 (ILLUMINATE-B). NLM Identifier: NCT 03905694. Last updated: November 18, 2023. Available at: https://www.clinicaltrials.gov/ct2/show/NCT03905694?term=NCT03905694&draw=2&rank=1. Accessed: December 5, 2023.
2. Alnylam Pharmaceuticals. A study to evaluate lumasiran in children and adults in primary hyperoxaluria type 1 (ILLUMINATE-A). NLM Identifier: NCT 03681184. Last updated: November 18, 2023. Available at: https://www.clinicaltrials.gov/ct2/show/NCT03681184?term=03681184&draw=2&rank=1. Accessed: December 5, 2023.
3. Alynylam Pharmaceuticals. A study to evaluate lumasiran in patients with advanced primary hyperoxaluria type 1 (ILLUMINATE-C). NLM Identifier: NCT 04152200. Last updated: November 19, 2023. Available at: https://clinicaltrials.gov/ct2/show/NCT04152200. Accessed: December 6, 2023.
4. Cochat P, Hulton SA, Acquaviva C, et. al. Primary hyperoxaluria Type 1: indications for screening and guidance for diagnosis and treatment. *Nephrol Dial Transplant*. 2012 May;27(5):1729-36.
5. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: December 4, 2023.
6. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
7. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
8. Milliner DS, Harris PC, Sas DJ, et al. Primary Hyperoxaluria Type 1. 2002 Jun 19 [Updated 2022 Feb 10]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2022. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1283/.
9. Niaudet P. Primary hyperoxaluria. Last updated: December 1, 2023. In: UpToDate, Post TW (Ed), UpToDate, Waltham, MA. Accessed: December 3, 2023.