Reference number(s)
4397-A

Standard Medicare Part B Management
Oxlumo

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Oxlumo	lumasiran

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[1]

Oxlumo is indicated for the treatment of primary hyperoxaluria type 1 (PH1) to lower urinary and plasma oxalate levels in pediatric and adult patients.

All other indications will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

Initial Requests:

© 2025 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.

- Molecular genetic test results demonstrating a pathogenic variant in the alanine:glyoxylate aminotransferase (AGXT) gene or liver enzyme analysis results demonstrating absent or significantly reduced alanine:glyoxylate aminotransferase (AGT) activity.

Continuation Requests:

- Chart notes or medical records demonstrating a benefit from therapy.

Coverage Criteria

Primary Hyperoxaluria Type 1[1-4]

Authorization of 12 months may be granted for the treatment of primary hyperoxaluria type 1 (PH1) when the diagnosis is confirmed by either of the following:

- Molecular genetic test results demonstrating a pathogenic variant in the alanine:glyoxylate aminotransferase (AGXT) gene.
- Liver enzyme analysis results demonstrating absent or significantly reduced alanine:glyoxylate aminotransferase (AGT) activity.

Continuation of Therapy

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

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

- The member is currently receiving therapy with Oxlumo.
- Oxlumo is being used to treat an indication in the coverage criteria section.
- The member is receiving benefit from therapy (e.g., decrease or normalization of urinary and/or plasma oxalate levels, improvement in kidney function).

Summary of Evidence

The contents of this document were created after examining the following resources:

- The prescribing information for Oxlumo.
- The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service- Drug Information (AHFS-DI)
    - Lexi-Drugs

© 2025 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.

    - Clinical Pharmacology
- The primary hyperoxalurias: an algorithm for diagnosis

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Oxlumo are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for the diagnostic criteria for primary hyperoxaluria type 1 can be found in a review article by Cochat and Rumsby. A definitive diagnosis of primary hyperoxaluria in a patient with clinical signs and symptoms requires genetic testing to detect a pathogenic variant in the alanine:glyoxylate aminotransferase (AGXT) gene. In some cases, the phenotype is typical of primary hyperoxaluria, but no pathogenic variant is detected, either because the pathogenic variant lies in a promoter or other regulatory sequence or because some other, as yet undefined, metabolic defect is present (i.e., "uncategorized" primary hyperoxaluria). In such cases, a liver biopsy can be performed to test for levels of AGT and GRHPR activity; if the results are negative, primary hyperoxaluria types 1 and 2 can be ruled out.

References

1. Oxlumo [package insert]. Cambridge, MA: Alnylam Pharmaceuticals, Inc; April 2025.
2. Niaudet, P. Primary hyperoxaluria: Clinical features, diagnosis, and management. Waltham, MA. UpToDate. Last Modified September 30, 2025. https://www.uptodate.com/contents/primary-hyperoxaluria-clinical-features-diagnosis-and-management. Accessed October 20, 2025.
3. Milliner DS. The primary hyperoxalurias: an algorithm for diagnosis. Am J Nephrol 2005; 25:154.
4. Cochat P, Rumsby G. Primary hyperoxaluria [published correction appears in N Engl J Med. 2013 Nov 28;369(22):2168]. *N Engl J Med*. 2013;369(7):649-658. doi:10.1056/NEJMra1301564

© 2025 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.