# SmartPA Criteria Proposal

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|---|---|
| **Drug/Drug Class:** | Oxlumo Clinical Edit |
| **First Implementation Date:** | TBD |
| **Proposed Date:** | March 18, 2021 |
| **Prepared for:** | MO HealthNet |
| **Prepared by:** | MO HealthNet/Conduent |
| **Criteria Status:** | ☐Existing Criteria<br>☐Revision of Existing Criteria<br>☒New Criteria |

## Executive Summary

**Purpose:** Ensure appropriate utilization and control of Oxlumo™ (lumasiran)

**Why Issue Selected:** Oxlumo™ (lumasiran) was FDA approved on November 23, 2020 and is the first FDA approved therapy for primary hyperoxaluria type 1 (PH1). An estimated 1 to 3 people per million in North America and Europe are affected by PH1. PH1 is caused by a mutation in the alanine-glyoxylate aminotransferase (AGXT) gene, which encodes the alanine glyoxylate aminotransferase (AGT) enzyme. This mutation prevents the breakdown of glyoxylate, causing it to convert to oxalate and accumulate in the kidney and urinary tract, where it can then combine with calcium to form kidney and urinary stones. Patients typically develop recurrent kidney stones with progressive nephrocalcinosis and end stage renal disease by 20 - 30 years of age. Oxlumo reduces levels of the glycolate oxidase (GO) enzyme by targeting the hydroxyacid oxidase 1 mRNA in hepatocytes through RNA interference. Decreased GO enzyme levels reduce the amount of available glyoxylate, decreasing conversion to oxalate. The goal of therapy is to slow the progression of disease and preserve kidney function as long as possible. Oxlumo is not expected to be effective in other forms of primary hyperoxaluria , such as PH2 or PH3, because its mechanism of action does not affect the metabolic pathways causing hyperoxaluria in those subtypes. PH1 is the most common and severe type of PH and accounts for approximately 80% of all PH cases. Due to the high cost and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of Oxlumo.

**Program-Specific Information:**

| Drug | Cost per vial | Cost per year (maintenance dosing based on a 70 kg patient) |
|---|---|---|
| OXLUMO 94.5 MG/0.5 ML VIAL | $55,000 WAC | $660,000 WAC |

**Type of Criteria:**
☐ **Increased risk of ADE**
☒ **Appropriate Indications**
☐ **Preferred Drug List**
☒ **Clinical Edit**

**Data Sources:**
☐ **Only Administrative Databases**
☒ **Databases + Prescriber-Supplied**

## Setting & Population

- Drug class for review: Oxlumo™ (lumasiran)
- Age range: All appropriate MO HealthNet participants

## Approval Criteria

Initial Therapy:

- Prescribed by or in consultation with a nephrologist, urologist, or other specialist in the treated disease state **AND**
- Documented diagnosis of primary hyperoxaluria type 1 (PH1) **AND**
- Diagnosis confirmed by:
  - Genetic testing confirming a pathogenic variant of the AGXT gene **OR**
  - Presence of characteristic disease symptoms such as:
    - chronic kidney stone formation (with 95% or more of calcium oxalate monohydrate)
    - hyperoxaluria
    - liver biopsy showing low levels of AGT enzyme activity **AND**
- Documented previous or concurrent therapy with pyridoxine for at least 90 days **AND**
- Documentation of baseline urinary oxalate excretion

Continuation of Therapy:

- Initial approval is for 1 year, renewal of prior authorization may be given following documentation of improved urinary oxalate excretion from baseline

## Denial Criteria

- Therapy will be denied if all approval criteria are not met
- Documented history of liver transplant
- Participant demonstrates clinical evidence of systemic oxalosis (i.e., oxalate deposits in the heart)
- Participant has an eGFR < 30 ml/min/1.73 m2

## Required Documentation

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|---|---|---|---|
| Laboratory Results: | X | Progress Notes: | |
| MedWatch Form: | | Other: | X |

## Disposition of Edit

Denial: Exception code “0682” (Clinical Edit)
Rule Type: CE

## Default Approval Period

1 year

## References


- OXLUMO (lumasiran) [package insert]. Cambridge, MA: Alnylam Pharmaceuticals; November 2020.

- National Organization for Rare Disorders. Primary Hyperoxaluria. https://rarediseases.org/rare-diseases/primary-hyperoxaluria/. Accessed October 16, 2020.
- Hoyer-Kuhn, H., Kohbrok, S., Volland, R., Franklin, J., Hero, B., Beck, B.B., Hope, B. Vitamin B6 in Primary Hyperoxaluria I: First Prospective Trial after 40 Years of Practice. CJASN; March 2014; 9(3):468-477.
- Allena Pharmaceuticals. Hyperoxaluria. https://www.allenapharma.com/hyperoxaluria. Accessed November 27, 2020.
- Milliner, D.S., Eickholt, J.T., Bergstralh, E.J., Wilson, D.M., Smith, L.H. Results of Long-term Treatment with Orthophosphate and Pyridoxine in Patients with Primary Hyperoxaluria. N Engl J Med 1994; 331:1553-1558.
- Medscape. Hypercalciuria Treatment & Management. https://emedicine.medscape.com/article/2182757-treatment#d9. Accessed November 27, 2020.
- IPD Analytics. New Drug Review: Oxlumo (lumasiran). December 2020.