

Lumasiran: Adis Evaluation

Clinical Considerations

- First-in-class synthetic, double-stranded, ribonucleic acid interference molecule targeting glycolate oxidase
- Approved for the treatment of primary hyperoxaluria type 1 in patients of any age
- Reduces urinary oxalate excretion and plasma oxalate levels; effect is sustained in longer-term analyses
- Acceptable tolerability profile; injection-site reactions are the most common adverse event

Plain Language Summary

Background and rationale

- Primary hyperoxaluria type 1 (PH1) is a rare genetic disorder that leads to excess oxalate in urine or plasma requiring removal by the kidneys. This overproduction is damaging and can lead to kidney failure. Management of PH1 is typically not curative, eventually ending in kidney and/or liver transplantation
- Lumasiran (Oxlumo®) is the first medicine to be approved in several countries for use in patients with PH1, regardless of their age or level of kidney function. It reduces liver oxalate production, lessening damage to the kidneys and potentially reducing the need for organ transplantation.

Clinical findings

- In clinical trials, lumasiran was effective in reducing oxalate levels (in urine and/or plasma) in patients of all ages with PH1, and irrespective of whether they were receiving dialysis or not.
- Lumasiran either improved or stabilized the severity of calcium deposition in the kidneys, number of kidney stone events and kidney function.
- Lumasiran had an acceptable tolerability profile; the most common side effects were mild injection-site reactions that resolved quickly.

Conclusion

Lumasiran is an effective treatment option, with an acceptable tolerability profile, in patients with PH1.

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