

Eplontersen: Adis Evaluation

Key Points

- A ligand-conjugated antisense oligonucleotide being developed by Ionis Pharmaceuticals and AstraZeneca for the treatment of ATTR
- Received its first approval on 21 December 2023 in the USA
- Approved for use in adults with ATTRv-PN

Summary

Eplontersen (Wainua™) is a ligand-conjugated antisense oligonucleotide directed to *TTR*, which is being developed by Ionis Pharmaceuticals and AstraZeneca for the treatment of TTR-mediated amyloidosis (ATTR).

Eplontersen, which is targeted to the liver by a ligand containing three N-acetyl galactosamine residues, binds to wild-type and variant *TTR* mRNA, thus reducing the levels of circulating TTR protein and amyloid deposition.

Subcutaneous eplontersen reduced serum TTR levels, inhibited neuropathy progression and improved health-related quality of life in patients with polyneuropathy of hereditary ATTR (ATTRv-PN; v for variant) in a phase III trial. Based on these results, eplontersen was approved in the USA for the treatment of ATTRv-PN on 21 December 2023 and is currently undergoing regulatory review for a similar indication in the EU, the UK, Switzerland and Canada.

Eplontersen is also undergoing phase III development for ATTR cardiomyopathy.

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