# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Final draft guidance

# Eplontersen for treating hereditary transthyretin-related amyloidosis

## 1 Recommendations

1.1 Eplontersen is recommended, within its marketing authorisation, as an option for treating hereditary transthyretin-related amyloidosis in adults with stage 1 or stage 2 polyneuropathy. It is only recommended if the company provides eplontersen according to the commercial arrangement (see section 2).

1.2 Use the least expensive option of the available treatments (including eplontersen and vutrisiran). Take account of administration costs, dosages, price per dose and commercial arrangements. If the least expensive option is unsuitable, people with the condition and their healthcare professional should discuss the advantages and disadvantages of other treatments.

### Why these recommendations were made

Usual treatment for hereditary transthyretin-related amyloidosis with stage 1 or 2 polyneuropathy includes vutrisiran. Eplontersen works in a similar way to vutrisiran but people can inject it themselves at home. Eplontersen is used monthly, whereas vutrisiran is used every 3 months.

Clinical trial evidence suggests that eplontersen is more effective than placebo at lowering levels of transthyretin in the blood, and increases how long people have before their polyneuropathy gets worse. There are no clinical trials directly comparing eplontersen with vutrisiran. An indirect comparison suggests that eplontersen works as well as vutrisiran.



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A cost comparison suggests that eplontersen is cost saving compared with vutrisiran. So it is recommended.

For all evidence, see the committee papers. For more information on NICE's evaluation of vutrisiran, see NICE's technology appraisal guidance on vutrisiran for treating hereditary transthyretin-related amyloidosis.

# 2 Information about eplontersen

## Marketing authorisation indication

2.1 Eplontersen (Wainzua) is indicated for 'treatment of hereditary transthyretin-mediated amyloidosis (ATTRv amyloidosis) in adult patients with stage 1 and 2 polyneuropathy'.

## Dosage in the marketing authorisation

2.2 The dosage schedule will be available in the summary of product characteristics for eplontersen.

## Price

2.3 The list price of a 45 mg vial of eplontersen is confidential and cannot be reported here.

2.4 The company has a commercial arrangement (simple discount patient access scheme). This makes eplontersen available to the NHS with a discount. The size of the discount is commercial in confidence.

# 3 Implementation

3.1 Section 7 of the National Institute for Health and Care Excellence (Constitution and Functions) and the Health and Social Care Information Centre (Functions) Regulations 2013 requires integrated care boards, NHS England and, with respect to their public health functions, local authorities to comply with the recommendations in this evaluation within 3 months of its date of publication. Because eplontersen has been recommended through the cost-comparison process, NHS England and

integrated care boards have agreed to provide funding to implement this guidance 30 days after publication.

3.2 The Welsh ministers have issued directions to the NHS in Wales on implementing NICE technology appraisal guidance. When a NICE technology appraisal guidance recommends the use of a drug or treatment, or other technology, the NHS in Wales must usually provide funding and resources for it within 2 months of the first publication of the final draft guidance.

3.3 When NICE recommends a treatment 'as an option', the NHS must make sure it is available within the period set out in the paragraphs above. This means that, if a patient has hereditary transthyretin-related amyloidosis with stage 1 or stage 2 polyneuropathy and the healthcare professional responsible for their care thinks that eplontersen is the right treatment, it should be available for use, in line with NICE's recommendations.

# 4 Evaluation committee members and NICE project team

## Evaluation committee members

The highly specialised technologies evaluation committee is a standing advisory committee of NICE. This topic was considered by the chair and vice chair of this committee.

Committee members are asked to declare any interests in the technology being evaluated. If it is considered there is a conflict of interest, the member is excluded from participating further in that evaluation.

## NICE project team

Each evaluation is assigned to a team consisting of 1 or more health technology analysts (who act as technical leads for the evaluation), a technical adviser and a project manager.

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