NICE National Institute for Health and Care Excellence

NICE guidance

Eplontersen for treating hereditary transthyretin-related amyloidosis

Technology appraisal guidance
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www.nice.org.uk/guidance/ta1020

Your responsibility

The recommendations in this guidance represent the view of NICE, arrived at after careful consideration of the evidence available. When exercising their judgement, health professionals are expected to take this guidance fully into account, alongside the individual needs, preferences and values of their patients. The application of the recommendations in this guidance is at the discretion of health professionals and their individual patients and do not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian.

All problems (adverse events) related to a medicine or medical device used for treatment or in a procedure should be reported to the Medicines and Healthcare products Regulatory Agency using the Yellow Card Scheme.

Commissioners and/or providers have a responsibility to provide the funding required to enable the guidance to be applied when individual health professionals and their patients wish to use it, in accordance with the NHS Constitution. They should do so in light of their duties to have due regard to the need to eliminate unlawful discrimination, to advance equality of opportunity and to reduce health inequalities.

Commissioners and providers have a responsibility to promote an environmentally sustainable health and care system and should assess and reduce the environmental impact of implementing NICE recommendations wherever possible.

Contents

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.

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.

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, AstraZeneca) 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 is available in the summary of product characteristics for eplontersen (PDF only).

Price

2.3 The list price is £31,954.12 for a 45-mg vial (excluding VAT; company submission). The annual cost is £383,449.44 based on monthly injections.

2.4 The company has a commercial arrangement. 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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