NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Final draft guidance

Vutrisiran for treating hereditary transthyretin-related amyloidosis

1 Recommendations

1.1 Vutrisiran 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 vutrisiran according to the commercial arrangement (see section 2).

1.2 If people with the condition and their clinicians consider vutrisiran to be 1 of a range of suitable treatments, discuss the advantages and disadvantages of the available treatments. After that discussion, if more than 1 treatment is suitable, choose the least expensive. Take account of administration costs, dosage, price per dose and commercial arrangements.

Why this decision was made

Hereditary transthyretin-related amyloidosis is usually treated with patisiran, which is already recommended in NICE's highly specialised technologies guidance on patisiran. Vutrisiran works in a similar way, but it is given as an injection under the skin instead of into a vein.

Evidence from a clinical trial and an indirect comparison shows that vutrisiran works as well as patisiran.

In the economic model, the company estimated costs for patisiran using pharmacy data showing the number of vials used per person. They provided a scenario using clinical trial evidence. The clinical experts suggested that more vials of patisiran

would be used in clinical practice than in the clinical trial. If more vials of patisiran are used, vutrisiran is more likely to be cost saving.

The cost savings for vutrisiran also depend on how long it takes to administer patisiran and which type of healthcare professional administers it. The clinical experts agreed with the company's estimate of administration time. In the model, when the administration cost for patisiran increases, vutrisiran is more cost saving.

Taking the number of vials used per person in the pharmacy data and administration costs into account, a cost comparison suggests vutrisiran is cost saving compared with patisiran. So vutrisiran is recommended.

For all evidence see the external assessment group report and committee papers. To see what NICE did for patisiran, see the committee discussion in NICE's guidance on patisiran.

2 Information about vutrisiran

Marketing authorisation indication

2.1 Vutrisiran (Amvuttra, Alnylam) is indicated for the 'treatment of hereditary transthyretin-mediated amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy'.

Dosage in the marketing authorisation

2.2 The dosage schedule is available in the summary of product characteristics for vutrisiran.

Price

2.3 £95,862.36 per 0.5 ml solution for injection containing 25 mg of vutrisiran (excluding VAT; company submission). At the recommended dose of 25 mg administered subcutaneously every 3 months, the estimated annual cost per patient is £383,449.44.

2.4 The company has a commercial arrangement (simple discount patient access scheme). This makes vutrisiran available to the NHS with a

discount. The size of the discount is commercial in confidence. It is the company's responsibility to let relevant NHS organisations know details of the discount.

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 appraisal within 3 months of its date of publication. Because vutrisiran has been recommended through the cost-comparison process, NHS England and commissioning groups 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 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 appraisal document.

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 and the doctor responsible for their care thinks that vutrisiran is the right treatment, it should be available for use, in line with NICE's recommendations.

4 Appraisal committee members and NICE project team

Appraisal committee members

The 4 technology appraisal committees are standing advisory committees of NICE. This topic was considered by the chair and vice chair of NICE's highly specialised technologies evaluation committee.

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

NICE project team

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

Anne Murray-Cota
Technical lead

Alexandra Filby
Technical adviser

Celia Mayers
Project manager

ISBN: [to be added at publication]