NICE National Institute for Health and Care Excellence

Resource impact summary report

Resource impact
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This summary report is based on the NICE assumptions used in the resource impact template. Users can amend the 'Inputs and eligible population' and 'Unit costs' worksheets in the template to reflect local data and assumptions.

Recommendation

NICE has recommended eplontersen 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 it according to the commercial arrangement (see section 2 of the guidance).

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.

Eligible population for eplontersen

Table 1 shows the population who are eligible for eplontersen and the number of people who are expected to have eplontersen in each of the next 5 years, including population growth.

Table 1 Population expected to be eligible for and have eplontersen in England (NICE estimates)

Eligible population and uptake	Current practice	2024-25	2025-26	2026-27	2027-28	2028-29
People eligible for eplontersen (prevalent population) **(a)**	161	163	164	166	167	169
Of whom: People diagnosed each year	23	23	23	24	24	24

Eligible population and uptake	Current practice	2024-25	2025-26	2026-27	2027-28	2028-29
Proportion of people having eplontersen each year (includes those who start treatment and those who are continuing treatment) **%(b)**	0	7	15	22	29	36
Number of people treated (a) x (b)	0	12	24	36	48	60
***Note:** For simplicity, the uptake percentage above reflects both people starting and continuing treatment with eplontersen each year						

The following assumptions have been used to calculate the eligible population:

- Annual incidence of adults eligible for treatment is constant per year at 23 people. There is a small increase for population growth over a 5-year period.
- The number of people who stop being eligible for treatment each year is expected to be the same as the annual incidence.
- Prevalence is based on data from National Amyloidosis Centre (NAC); this can be adjusted for local population variation.
- Clinical haematology expert opinion suggests uptake within people diagnosed each year is 50% (or around 12 people each year). For simplicity, because people are anticipated to continue treatment in future years, the cumulative uptake is used to calculate the proportion of people having eplontersen each year within the prevalent population. This includes people who start treatment in year and people who continue treatment from a previous year.

Treatment options for the eligible population

The main comparator treatment for the eligible population is vutrisiran (subcutaneous injection administered by a health care professional [HCP] as NHS commissioned homecare). Other options are patisiran (intravenous [IV] administration by HCP – NHS commissioned homecare) and inotersen (subcutaneous injection, self-administered).

Use of inotersen is associated with significant toxicity and is rarely prescribed according to clinical expert opinion from the NAC. Treatment with inotersen requires regular monitoring for numerous side effects. Patisiran requires time-consuming IV administration, a premedication regimen of IV corticosteroid, H1 blocker, H2 blocker, and oral

paracetamol, and due to the risk of infusion-related reactions (IRRs), constant monitoring during infusion by HCPs.

For more information about the treatments, such as dose and average treatment duration, see the resource impact template.

The company has a commercial arrangement. This makes eplontersen available to the NHS with a discount.

Users can input the confidential price of eplontersen and amend other variables in the resource impact template.

The payment mechanism for the technology is determined by the responsible commissioner and depends on the technology being classified as high cost.

For further analysis or to calculate the financial impact of cash items, see the resource impact template.

Capacity impact

Eplontersen is self-administered by subcutaneous injection and has capacity benefits because it does not require home care administration by a HCP or frequent monitoring visits. As with other options treatment is initiated at the National Amyloidosis Centre.

Adverse events are not included in the resource impact assessment due the compatibility between adverse events for eplontersen and vutrisiran. This is shown in sections B.3.9.6–3.9.7 of Document B of the company submission.

Table 2 shows the main impacts on capacity activity in each of the next 5 years. This is based on the market share estimates from clinical haematology expert opinion (see table 1 above).

Table 2 Capacity impact (activity) in England – based on NICE estimates

Capacity impact	2024-25	2025-26	2026-27	2027-28	2028-29
NHSE commissioned homecare nurse administrations	(50)	(90)	(130)	(170)	(200)
Monitoring attendances	(80)	(80)	(80)	(80)	(80)

Capacity impact	2024-25	2025-26	2026-27	2027-28	2028-29
Pharmacy support (number of treatment preparations)	(40)	(40)	(40)	(40)	(40)

For further analysis or to calculate the financial capacity impact from a commissioner (national) and provider (local) perspective, see the resource impact template.

Key information

Table 3 Key information

Time from publication to routine commissioning funding	30 days
Programme budgeting category	4B Endocrine, Nutritional and Metabolic Problems - Endocrine
Commissioner	NHS England
Provider	National Amyloidosis Centre
Pathway position	Adults with stage 1 or 2 polyneuropathy

About this resource impact summary report

This resource impact summary report accompanies the NICE guidance on eplontersen for treating hereditary transthyretin-related amyloidosis and should be read with it. See terms and conditions on the NICE website.

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