NICE National Institute for Health and Care Excellence

Resource impact summary report

Resource impact
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Resource impact summary report

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

Efanesoctocog alfa is recommended as an option for treating and preventing bleeding episodes in people 2 years and over with haemophilia A (congenital factor VIII deficiency), only if:

- they have a factor VIII activity level of less than 1% (severe haemophilia A)
- the company provides it according to the commercial arrangement.

This recommendation is not intended to affect treatment with efanesoctocog alfa that was started in the NHS before this guidance was published. People having treatment outside this recommendation may continue without change to the funding arrangements in place for them before this guidance was published, until they and their NHS healthcare professional consider it appropriate to stop. For children and young people, this decision should be made jointly by them, their healthcare professional, and their parents or carers.

Eligible population for efanesoctocog alfa

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

Table 1 Population expected to be eligible for and have efanesoctocog alfa (IV injection) in England

Eligible population and market share	Current practice (without efanesoctocog alfa)	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029	2029 to 2030
People eligible for efanesoctocog alfa who have had previous treatment with factor VIII replacement therapy – population 1	720	730	730	740	740	750

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Eligible population and market share	Current practice (without efanesoctocog alfa)	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029	2029 to 2030
Market share of efanesoctocog alfa (%)	0	37	53	66	66	66
People having the treatment – population 1	0	270	390	490	490	490
People eligible for efanesoctocog alfa who have had previous treatment with emicizumab – population 2	1,140	1,150	1,160	1,170	1,180	1,180
Market share of efanesoctocog alfa (%)	0	12	18	20	20	20
People having the treatment – population 2	0	140	210	230	240	240
Total people having efanesoctocog alfa each year	0	410	600	720	730	730

Notes: The population is split into 2 groups according to what previous treatment they have had. This is because the market share is anticipated to be different depending on whether people are currently having factor VIII replacement therapy or emicizumab. The figures above remove any double counting from people having both factor VIII replacement therapy and emicizumab. The incident population is not added because treatment is assumed to be ongoing, and incidence is small compared with prevalence. So, the prevalence is assumed to be steady.

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

- Data from the National Haemophilia Database from April 2022-March 2023 shows that:
  - prevalence of haemophilia A in England is around 0.013%
  - people who have severe haemophilia A (Factor VIII activity level of less than 1%) is 24.6%
  - around 720 people with severe haemophilia A currently have factor VIII replacement therapy (see table 1)
  - around 1,140 people with severe haemophilia A currently have emicizumab (see table 1)
  - monitoring costs and adverse events costs are not materially different to comparators.
- The resource impact assessment has only been made based on prophylactic use. This is because it is not possible to quantify on-demand treatment and treatment of

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bleeding episodes because this is dependent on the severity of the bleeding episode and the disease severity.

- The uptake for efanesoctocog alfa is based on clinical haematology expert opinion.

Treatment options for the eligible population

The comparator treatment options for severe haemophilia A include factor VIII replacement therapies (including standard half-life and extended half-life therapies) or emicizumab to prevent bleeding (ongoing treatment). On demand factor VIII replacement therapies are used to treat bleeding.

Factor VIII replacement therapy needs frequent intravenous injections. For half-life products this varies and could range from every 2 days to every 4 days, which can be self-administered through slow intravenous push injections over 30 minutes. A central venous access device may need to be surgically fitted in young children. Frequent factor VIII replacement injections can damage veins, resulting in pain on administration and increasing the chance of 'vein collapse'.

Emicizumab is a subcutaneous injection. People need to administer 3 mg/kg once weekly for the first 4 weeks, followed by a maintenance dose from week 5 of 1.5 mg/kg once weekly, 3 mg/kg every 2 weeks, or 6 mg/kg every 4 weeks. All doses are administered as a subcutaneous injection (emicizumab summary of product characteristics).

Efanesoctocog alfa is delivered weekly as an intravenous injection directly into the bloodstream and may be self-administered after proper training. It comes in single-dose vials that must be reconstituted, or diluted, in 3 ml of sterile water within 3 hours before using. Prefilled syringes of sterile water are provided with the treatment.

As with other options, treatment is started in the acute setting and needs input from a clinical haematologist. Postadministration nursing time for observation is needed in case of adverse reactions. An additional follow-up appointment with a specialist may be needed. Efanesoctocog alfa has a longer half-life than other factor VIII replacement therapies and can be used for both on-demand treatment and prophylaxis. The guidance committee concluded that a new treatment option with effective bleeding control and a less frequent dosing schedule would be welcomed by people with haemophilia A.

For more information about the treatments, such as dose and average treatment duration,

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see the resource impact template.

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

Users can input the confidential price of efanesoctocog alfa 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

Table 2 shows the main impact on capacity activity in each of the next 5 years.

Table 2 Capacity impact (activity) in England

Capacity impact	Current practice (without efanesoctocog alfa)	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029	2029 to 2030
Number of specialist appointments – treatment follow up	0	440	670	810	840	860

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	90 days
Programme budgeting category	3 disorders of blood
Commissioner	NHS England
Provider	NHS hospital trusts

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Pathway position	First-line option

About this resource impact summary report

This resource impact summary report accompanies NICE's technology appraisal guidance on efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A for people 2 years and over and should be read with it. See the terms and conditions on the NICE website.

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