NICE National Institute for Health and Care Excellence

Resource impact summary report

Resource impact
Published: 24 June 2025

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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

Haemophilia B

NICE has recommended marstacimab within its marketing authorisation, as an option for preventing bleeding episodes caused by severe (factor IX [9] activity less than 1%) haemophilia B (congenital factor 9 deficiency) in people 12 years and over who:

- weigh at least 35 kg and
- do not have factor 9 inhibitors (anti-factor antibodies).

Marstacimab is only recommended if the company provides it according to the commercial arrangement.

Haemophilia A

Marstacimab is not recommended, within its marketing authorisation, for preventing bleeding episodes caused by severe (factor VIII [8] activity less than 1%) haemophilia A (congenital factor 8 deficiency) in people 12 years and over who:

- weigh at least 35 kg and
- do not have factor 8 inhibitors.

This recommendation is not intended to affect treatment with marstacimab that was started in the NHS before this guidance was published. People having treatment outside this 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 or young people, this decision should be made jointly by the healthcare professional, the child or young person, and their parents or carers.

Eligible population for marstacimab

Table 1 shows the population who are eligible for marstacimab and the number of people who are expected to have marstacimab in each of the next 5 years. No population growth is assumed because the number of people becoming eligible aged 12 and over is likely to be decreasing. This is because the birth rate has been declining since 2010.

Table 1 Population expected to be eligible for and have marstacimab in England

Eligible population and market share	People eligible for marstacimab	Market share for marstacimab (%)	People starting treatment each year	People continuing treatment from previous year(s)	People having marstacimab each year
Current practice without marstacimab	208	0	0	0	0
Year 1	200	13	26	0	26
Year 2	193	18.5	10	26	36
Year 3	185	24	9	36	44
Year 4	177	29	7	44	51
Year 5	166	33	4	51	55

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

- Based on haematologist expert opinion, treatment is assumed to be life-long
- A decreasing eligible population is assumed because of uptake of etranacogene dezaparvovec which is a single treatment gene therapy (see NICE's technology appraisal guidance on etranacogene dezaparvovec).

The uptake for marstacimab is a mid-point between the company and NHSE estimates in the first 3 years. Years 4 and 5 are a NICE estimate using the trend from previous years.

Treatment options for the eligible population

The comparator treatments for the eligible population are Factor IX replacement products (see resource impact template).

Marstacimab is given once weekly and delivered by subcutaneous injection using a pre-filled pen. There are currently no subcutaneous treatment options available for people with haemophilia B.

For severe haemophilia B, standard and extended half-life factor replacement therapies are available and extra on-demand factor replacement therapy can also be used for treating bleeds. These are generally delivered via slow intravenous push (bolus injection). Frequent injections for factor replacement therapy can damage veins, resulting in pain on administration and increasing the chance of a vein collapsing. Factor replacement therapies have varying dosing regimens from multiple injections per week to once weekly. Some people with haemophilia develop antibodies to replacement clotting factor, called inhibitors, which makes treatment with clotting factor replacement less effective.

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 marstacimab available to the NHS with a discount.

Users can input the confidential price of marstacimab 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.

We expect that the resource impact of implementing the recommendations in England will be less than £5 million per year (or about £8,800 per 100,000 population, based on a population for England of 57.16 million people).

This is because the population size is small.

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

Capacity impact

The Phase 3 BASIS trial showed that people having marstacimab prophylaxis had fewer bleed events than people having routine factor prophylaxis as measured by the annualised bleeding rate of treated bleeds. The annualised bleeding rate for treated bleeds was 7.85 for routine prophylaxis compared with 5.08 for marstacimab prophylaxis, equating to a 35.2% reduction. Note that these rates are based on trial data in a non-UK population and the rates seen in NHS clinical practice are expected to be lower than this.

Haematology expert opinion is that most bleeds are managed at home without the need for a hospital visit, and that very few bleeds now require hospital admission or inpatient treatment. The resource impact template includes an assumption that 20% of the bleed events will be treated in hospital for both marstacimab and standard Factor IX treatment. This is consistent with the economic modelling. This capacity benefit is shown in the estimates in table 2.

A reduction in the number of treated bleeds also has a cash benefit from avoiding additional use of factor replacement therapy, however this would be challenging to estimate because of the variable nature of bleed events.

There is expected to be additional appointments in the year that people commence treatment with marstacimab (for counselling, initial administrations, dose changes and reviews). This capacity impact is shown in table 2. Appointments decrease overall in future years as people take up treatment with etranacogene dezaparvovec gene therapy (see NICE's technology appraisal guidance on etranacogene dezaparvovec).

Table 2 Capacity impact (activity) in England

Capacity impact	Year 1	Year 2	Year 3	Year 4	Year 5
Number of bleed events needing hospital treatment avoided	(27)	(43)	(60)	(77)	(96)
Change in appointments	113	20	(3)	(28)	(65)

Source: Matino et al. 2023.

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(s)	NHS England
Provider	Haemophilia Comprehensive Care Centres
Pathway position	First line in people without anti-factor antibodies

About this resource impact summary report

This resource impact summary report accompanies the NICE technology appraisal guidance on marstacimab for treating severe haemophilia A or B in people 12 years and over without anti-factor antibodies' and should be read with it.

ISBN: 978-1-4731-7090-2