NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Denecimig (Mim8) for preventing bleeding episodes in haemophilia A in people 1 year and over

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of denecimig (Mim8) within its marketing authorisation for preventing bleeding episodes in haemophilia A in people 1 year and over.

Background

Haemophilia is a rare, lifelong genetic condition that affects the ability of blood to clot. This is caused by the inability or reduced ability of the body to produce substances called clotting factors which are needed for clotting. In haemophilia A the factor affected is called factor VIII (eight). Haemophilia A is an inherited condition predominantly found in males. Females who carry the haemophilia gene may have mild or, rarely, moderate to severe symptoms of bleeding.

The main symptom of haemophilia is prolonged bleeding. Other complications can include bleeding into joints and muscles without having had an injury. Severity of haemophilia is classed according to how much clotting factor is missing compared with normal expected levels of clotting factor. Mild haemophilia is classed as having over 5% of normal clotting factor. Moderate haemophilia is classed as having between 1% and 5% of normal clotting factor. Severe haemophilia is classed as having less than 1% of normal clotting factor. About 5 to 7% of people with haemophilia A develop antibodies to replacement clotting factor, called inhibitors, which makes treatment with clotting factor replacement less effective.[1]

The prevalence of haemophilia A is estimated at around 20 per 100,000 male births.[1] Registry data suggests that in 2023/2024 there were 9,662 people with haemophilia A, including 6424 people with mild, 794 with moderate and 2,348 with severe disease in the UK.[2]

Current clinical management of haemophilia A involves prophylactic treatment to prevent bleeding and long-term damage caused by bleeding, and on-demand treatment in response to bleeding episodes. Replacement of the missing clotting factor VIII in the blood through an intravenous infusion of clotting factor concentrate is used as a prophylactic (involving multiple injections per week) and on-demand treatment. For people aged 2 years and over with severe haemophilia A, NICE technology appraisal guidance 1051, recommends efanesoctocog alfa. NHS England has a clinical commissioning policy for emicizumab as a further prophylactic treatment option in people with haemophilia A with inhibitors and in people with severe haemophilia A without inhibitors.[3]

The technology

Denecimig (Mim8; brand name unknown, Novo Nordisk) does not currently have a marketing authorisation in the UK. It has been studied in clinical trials in adults and children with haemophilia A with or without inhibitors.

Intervention(s)	Denecimig (Mim8)
Population(s)	People 1 year and over with haemophilia A
Subgroups	If the evidence allows the following subgroups will be considered: • severity of haemophilia • presence or development of inhibitors • previous treatment status
Comparators	• Prophylaxis and on-demand treatment with factor VIII replacement therapy • Emicizumab (in accordance with NHS England's clinical commissioning policy) • Efanesoctocog alfa (for people 2 years and over with severe haemophilia A [factor VIII activity level of less than 1%])
Outcomes	The outcome measures to be considered include: • annualised bleeding rate • change in factor VIII levels • need for further treatment with factor VIII injections • duration of response to treatment • complications of the disease (for example joint problems and joint surgeries) • mortality • adverse effects of treatment • health-related quality of life.

Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The use of denecimig (Mim8) may be conditional on the presence of anti-factor antibodies. If so, the economic modelling should include the costs associated with diagnostic testing for anti-factor antibodies in people with haemophilia A who would not otherwise have been tested. A sensitivity analysis should be provided without the cost of the diagnostic test. See section 4.8 of the guidance development manual (available here: https://www.nice.org.uk/process/pmg36/chapter/introduction-to-health-technology-evaluation).
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related technology appraisals:** Marstacimab for treating severe haemophilia A or B in people 12 years and over without anti-factor antibodies. (2025) NICE Technology appraisal guidance 1073. Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A in people 2 years and over (2025) NICE Technology appraisal guidance 1051. **Related technology appraisals in development:** Valoctocogene roxaparvovec for treating severe haemophilia A. NICE technology appraisal guidance [ID3806] Publication date to be confirmed. Concizumab for treating severe haemophilia A or moderate to severe haemophilia B in people 12 years and over without inhibitors. NICE technology appraisal guidance [ID5099] Publication date to be confirmed. Concizumab for treating haemophilia A or B in people 12 years and over with inhibitors. NICE technology appraisal guidance [ID6665] Publication date to be confirmed.

	Marstacimab for treating severe haemophilia A or B in people 12 years and over with inhibitors to factor-replacement therapy. NICE technology appraisal guidance [TSID12143] Publication date to be confirmed
Related National Policy	NHS England (2013) 2013/14 NHS standard contract for haemophilia (all ages) section B part 1 - service specifications The NHS Long Term Plan, 2019. NHS Long Term Plan NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019). Chapter 132. NHS England. 2013/14 NHS Standard Contract for haemophilia A (all ages). B05/S/a NHS England. Clinical Commissioning Policy: Emicizumab as prophylaxis in people with severe congenital haemophilia A without factor VIII inhibitors (all ages). 170134P. August 2019. NHS England. Clinical Commissioning Policy: Emicizumab as prophylaxis in people with congenital haemophilia A with factor VIII inhibitors (all ages). 170067/P. July 2018.

Questions for consultation

Where do you consider denecimig (Mim8) will fit into the existing care pathway for preventing bleeding episodes in haemophilia A?

Please select from the following, will denecimig (Mim8) be:
A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):
For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would denecimig (Mim8) be a candidate for managed access?

Do you consider that the use of denecimig (Mim8) can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

Please indicate if any of the treatments in the scope are used in NHS practice differently than advised in their Summary of Product Characteristics. For example, if the dose or dosing schedule for a treatment is different in clinical practice. If so, please indicate the reasons for different usage of the treatment(s) in NHS practice. If stakeholders consider this a relevant issue, please provide references for data on the

efficacy of any treatments in the pathway used differently than advised in the Summary of Product Characteristics.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which denecimig (Mim8) will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1 National Organisation for Rare Disorders. Hemophilia A. (2022). Available from: https://rarediseases.org/rare-diseases/hemophilia-a/ [Accessed November 2025]

2 United Kingdom Haemophilia Centres Doctors' Association (2024). Available from: Microsoft Word - UKHCDO Annual report 2024.docx [Accessed November 2025]

3 NHS England. Emicizumab as prophylaxis in people with severe congenital haemophilia A without factor VIII inhibitors (all ages). Clinical Commissioning Policy 170134P. August 2019. [Accessed November 2025]