NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Efanesoctocog alfa for treating and preventing bleeding episodes in people of any age with previously treated Haemophilia A

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of efanesoctocog alfa within its marketing authorisation for treating and preventing bleeding episodes in people of any age with previously treated Haemophilia A

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 normally an inherited condition found in males. Females who carry the haemophilia gene may have mild or, rarely, severe symptoms of bleeding.

The main symptom of haemophilia is prolonged bleeding but 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 to 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.

The prevalence of haemophilia A is estimated at around 20 per 100,000 male births.[1] Registry data suggests that in 2020/2021 there were 8,740 people with haemophilia A, including 5,745 people with mild haemophilia A and 816 with moderate haemophilia A 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 and on-demand treatment. About 5-7% of people with haemophilia A develop antibodies to replacement clotting factor, called inhibitors, which makes treatment with clotting factor replacement less effective.[1] NHS England has clinical commissioning policies for emicizumab as a further prophylactic treatment option in people with haemophilia A with inhibitors and in people with severe haemophilia A without inhibitors.

The technology

Efanesoctocog alfa (brand name unknown, Swedish Orphan Biovitrum Ltd). Efanesoctocog alfa does not currently have a marketing authorisation in the UK for treating or preventing bleeding episodes in haemophilia A. It has been studied in clinical trials in adults and children with previously treated severe haemophilia A.

Intervention(s)	Efanesoctocog Alfa
Population(s)	People with previously treated (prophylaxis or on demand) haemophilia A
Comparators	• Established clinical management (including prophylaxis, on-demand treatment and treatment for developed inhibitors) • Emicizumab
Outcomes	The outcome measures to be considered include: • Annualised bleeding rate (ABR) • need for further treatment with factor VIII injections • durability of response to treatment • Complications of the disease e.g., joint problems or surgeries to treat joint problems) • 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 availability and cost of biosimilar and generic products should be taken into account.
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 appraisals in development:** 'Concizumab for preventing bleeding episodes in haemophilia A or haemophilia B' NICE technology appraisal guidance [ID5099]. Awaiting development [GID-TA10972]. Publication date to be confirmed.

	'Valoctocogene roxaparvovec for treating severe haemophilia A' Proposed NICE technology appraisal [ID 3806] [GID-TA10682]. Publication date to be confirmed. **Related highly specialised technology appraisals in development:** None **Related NICE guidelines:** None **Related NICE guidelines in development:** None **Related interventional procedures:** None **Related quality standards:** None
Related National Policy	NHS England. Clinical Commissioning Policy. Human coagulation factor X for hereditary factor X deficiency (all ages). 200208P. February 2020. 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. NHS England. 2013/14 NHS Standard Contract for Haemophilia A (all ages). B05/S/a 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.

Questions for consultation

Are the comparators defined appropriately? Which treatments are considered to be established clinical practice in the NHS for treating or preventing bleeding episodes in people with haemophilia A?

Are there any relevant subgroups to consider?

Where do you consider efanesoctocog alfa will fit into the existing care pathway for the disease?

How might disease severity and clinical presentation be considered to affect the patient population for which efanesoctocog alfa will be licensed?

Would disease severity be considered to affect response to treatment or prophylaxis with efanesoctocog alfa?

Would efanesoctocog alfa be a candidate for managed access?

Do you consider that the use of efanesoctocog alfa 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.

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 Efanesoctocog alfa 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-tehnology-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 12th April 2023]

2. United Kingdom Haemophilia Centres Doctors' Association (2022). Available form: UKHCDO-Annual-Report-2022-2021-22-Data.pdf [Accessed April 2023]