NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A

Final scope

Final remit/evaluation objective

To appraise the clinical and cost effectiveness of efanesoctocog alfa within its marketing authorisation for treating and preventing bleeding episodes in 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 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.

The prevalence of haemophilia A is estimated at around 20 per 100,000 male births.[1] Registry data suggests that in 2021/2022 there were 8,959 people with haemophilia A, including 4,048 people with mild, 836 with moderate and 2,178 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. 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 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

Efanesoctocog alfa (brand name unknown, Swedish Orphan Biovitrum) 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 haemophilia A
Subgroup(s)	If evidence allows subgroups will be considered based on: • severity of haemophilia • presence or development of inhibitors • previous treatment status
Comparators	Established clinical management, including: • prophylaxis and on-demand treatment with factor VIII replacement therapy • emicizumab (in accordance with NHS England's clinical commissioning policy)
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) • adverse effects of treatment • mortality • 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 Technology Appraisals:** None. **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 [ID3806] [GID-TA10682]. Publication date to be confirmed. Giroctocogene fitelparvovec for treating moderately severe to severe haemophilia A. Proposed NICE technology appraisal [GID-TA11329]. Publication date to be confirmed.
Related National Policy	NHS England. Emicizumab as prophylaxis in people with severe congenital haemophilia A without factor VIII inhibitors (all ages). Clinical Commissioning Policy 170134P. August 2019. 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.

References

1. National Organisation for Rare Disorders. Hemophilia A. (2022). Available from: https://rarediseases.org/rare-diseases/hemophilia-a/ [Accessed August 2023]

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

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 August 2023]