Engagement Report

Topic details

Title of policy or policy statement:	Emicizumab for prophylaxis of bleeding episodes in people with moderate haemophilia A without inhibitors (all ages)
Programme of Care:	Blood and infection
Clinical Reference Group:	Specialised Blood Disorders
URN:	2333

1. Summary

This report summarises the feedback NHS England received from engagement during the development of this policy proposition, and how this feedback has been considered.

2. Background

Haemophilia A is a rare condition that affects the blood's ability to clot. Haemophilia A is usually inherited and usually occurs in males. People with haemophilia A do not have enough of a clotting factor called factor VIII (eight) (FVIII) in their blood, or it isn't working properly, so they cannot form strong clots and bleed for longer than usual. Bleeding can be external (for example, from cuts) or internal (for example, into the brain or into joints, including the knee and elbow). Bleeding episodes into joints initially causes pain, bruising and swelling. Over time this leads to irreversible joint damage, reducing the person's ability to move and reducing their quality of life. Bleeding into other areas of the body, such as the brain, may be fatal.

Haemophilia A can be categorised from mild to severe, depending on the level of clotting FVIII activity. Mild deficiency is categorised as 5–40% FVIII activity, moderate deficiency as 1 to 5% FVIII activity and severe deficiency as <1% FVIII activity. Some patients with moderate haemophilia A are described as having a 'severe bleeding phenotype'. This is a term used in clinical practice with no one single definition, but generally in adults the term refers to those with ongoing bleeding events or joint damage or requiring FVIII prophylaxis. In children, the aim is to identify this group before bleeding episodes and joint damage occurs, so the clinical consensus is that a severe bleeding phenotype refers to those with baseline FVIII levels of 1–3 IU/dL in this age group.

There is currently no cure for haemophilia A and lifelong treatment is required. The aim of treatment for haemophilia A is to prevent bleeding episodes from occurring. Currently, bleeds can be prevented by injections of FVIII into the vein (either directly or via a central venous access device for patients who require it), given every 2 to 3 days.

Emicizumab is a drug used to prevent bleeding or reduce the number of bleeds in people with haemophilia A. It is administered as an injection that goes under the skin. Emicizumab works by binding to FX and activated FIX which brings those clotting factors near each other and activates the blood clotting system even if no FVIII is present. This is different to how replacement FVIII works. When a person starts on emicizumab they need to inject it once a week for the first 4 weeks (this is called a loading dose). After this, the person can inject emicizumab either once a week, once every 2 weeks or once every 4 weeks.

Engagement

NHS England has a duty under Section 13Q of the NHS Act 2006 (as amended) to 'make arrangements' to involve the public in commissioning. Full guidance is available in the Statement of Arrangements and Guidance on Patient and Public Participation in Commissioning. In addition, NHS England has a legal duty to promote equality under the Equality Act (2010) and reduce health inequalities under the Health and Social Care Act (2012).

The policy proposition underwent a two-week stakeholder testing between the 8th of July and 22nd July 2024 (one additional response accepted on 1st August 2024) with registered stakeholders from the following Clinical Reference Groups:

- Specialised Blood Disorders

Respondents were asked the following consultation questions:

- Do you support the proposition for emicizumab to be available as a routinely commissioned treatment option for patients with moderate haemophilia A without inhibitors for prophylaxis of bleeding episodes based on the evidence review and within the criteria set out in this document?
- Do you believe that there are any potential positive and/or negative impacts on patient care as a result of making this treatment option available?
- Do you believe that there is any additional information that we should have considered in the evidence review?
- Do you support the Equality and Health Inequalities Impact Assessment (EHIA)?

- Does the Patient Impact Assessment (PIA) present a true reflection of the patient and carers lived experience of this condition?
- Do you have any further comments on the policy proposal?
- Do you have any potential conflict of interest relating to this document or service area?

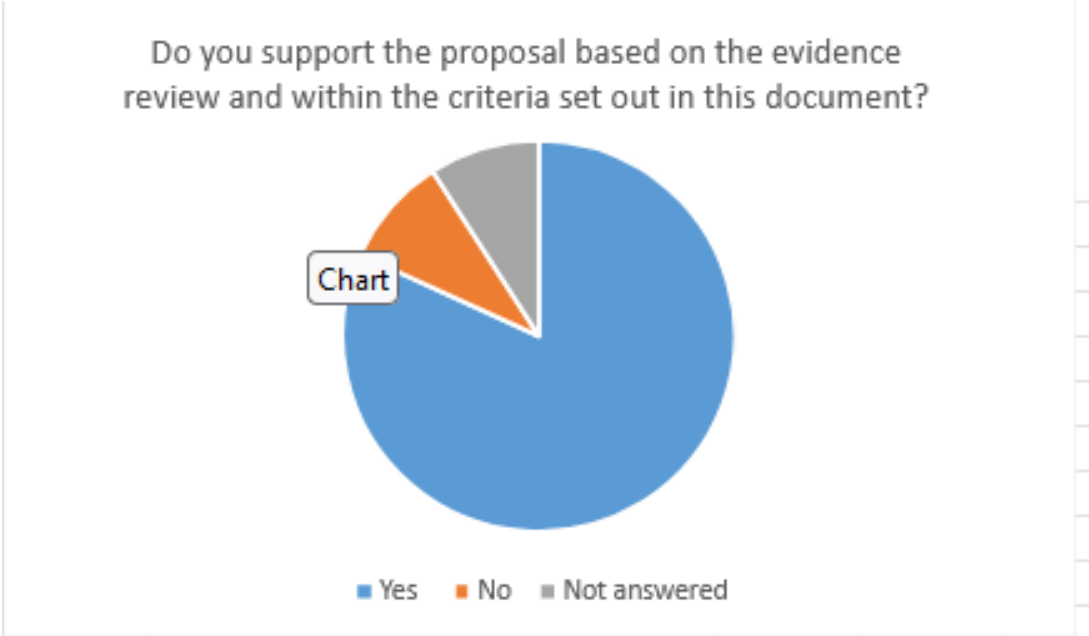
A 13Q assessment has been completed following stakeholder testing.

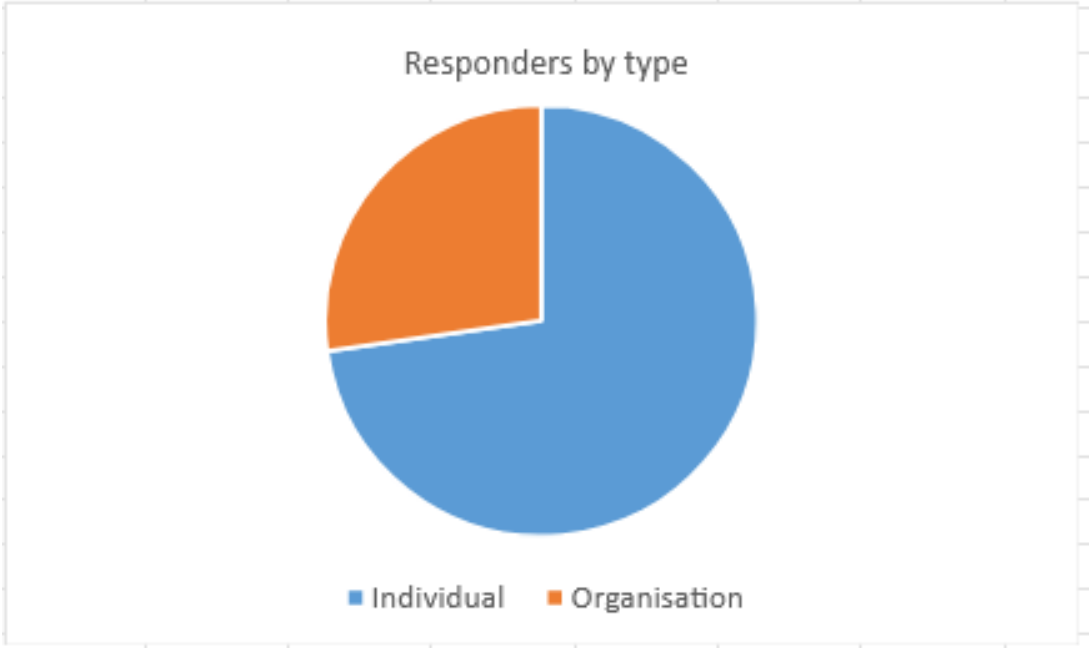
The Programme of Care (PoC) has decided that the proposition offers a clear and positive impact on patient treatment, by potentially making a new treatment available which widens the range of treatment options without disrupting current care or limiting patient choice, and therefore further public consultation was not required. This decision has been assured by the Patient Public Voice Advisory Group.

Engagement Results

11 stakeholders responded:

- Three organisations
- Eight individuals





In line with the 13Q assessment it was deemed that further public consultation was not required.

3. How has feedback been considered?

Responses to engagement have been reviewed by the Policy Working Group (PWG) and the Blood and Infection PoC. The following themes were raised during engagement:

Keys themes in feedback	NHS England Response
Relevant Evidence	
No additional evidence was provided by stakeholders.	Noted.
Impact Assessment	
All stakeholders felt the patient impact assessment presented a true reflection of the patient and carers lived experience of moderate haemophilia A.	Noted.
Inclusion and exclusion criteria	
Stakeholders were broadly supportive of the inclusion criteria. Those who disagreed with the inclusion criteria either felt it should be for all ages with levels of 1-3, not just children, or opened up to all patients with moderate haemophilia A.	Noted. This policy proposition is limited to moderate haemophilia A with severe phenotype. The inclusion criteria reflects a moderate haemophilia with severe phenotype, the population in the policy proposition and in line with emicizumab licence, hence the cut of levels used in children. The PWG believe that adults shouldn't be treated due to levels of factor VIII, instead it should reflect phenotype hence the separate inclusion criteria. Some adults have levels in 1-3 range, but, unlike children, do not have a severe phenotype with this and may not require prophylaxis. Therefore, the inclusion criteria for a severe phenotype in adults is different to reflect this.
Potential impact on equality and health inequalities	
All stakeholders supported the Equality and Health Inequalities Impact Assessment	Noted
Changes/addition to policy	
N/A	

4. Has anything been changed in the policy proposition as a result of the stakeholder testing and consultation?

The following change(s) based on the engagement responses has (have) been made to the policy proposition:

No changes to policy proposition or documents.

5. Are there any remaining concerns outstanding following the consultation that have not been resolved in the final policy proposition?

No