Consultation Report

Topic details

Title of policy or policy statement:	Emicizumab as prophylaxis in people with severe congenital haemophilia A without factor VIII inhibitors (all ages)
Programme of Care:	Blood & Infection
Clinical Reference Group:	Specialised Blood Disorders (F02)
URN:	1819

1. Summary

This report summarises the outcome of a public consultation that was undertaken to test the policy proposition.

2. Background

The Draft Policy Proposition for 'Clinical Commissioning Policy: Emicizumab as prophylaxis in people with severe congenital haemophilia A without factor VIII inhibitors (all ages) (Reference: NHS England 1819 / NICE ID014)' and associated documents were published for public consultation for 30 days from the 12th April.

3. Publication of consultation

The policy and supporting documents were published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 12th April to 12th May 2019. Consultation comments have then been shared with the Policy Working Group to enable full consideration of feedback and to support a decision on whether any changes to the policy might be recommended.

Respondents were asked the following consultation questions:

- Has all the relevant evidence been taken into account?
- Does the impact assessment fairly reflect the likely activity, budget and service impact? If not, what is inaccurate?
- Does the policy proposition accurately describe the current patient pathway that patients experience? If not, what is different?
- Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?
- Are there any changes or additions you think need to be made to this document, and why?

4. Results of consultation

Forty responses were received with summary statistics as described in table 1. The numbering indicates the positive/negative answers i.e. 7(yes)/0 (no)

Table 1	Responders and Responses (Yes / No, or, Response / Null)				
Question	Clinician	Provider	Non-Profit Professional	Patient or Representative	Pharmaceutical Company
Has all the relevant evidence been taken into account?	7 / 0	3 / 0	1 / 0	25 / 0	1 / 3
Does the impact assessment fairly reflect the likely activity, budget and service impact?	7 / 0	3 / 0	1 / 0	24 / 1	1 / 3
Does the policy proposition accurately describe the current patient pathway that patients experience?	7 / 0	3 / 0	1 / 0	23 / 2	2 / 2
Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?	5 / 2	1 / 2	0 / 1	8 / 17	2 / 2
Are there any changes or additions you think need to be made to this document, and why?	4 / 3	0 / 3	0 / 1	3 / 22	4 / 0

Narrative review of responses

The clinician and provider responses may be best considered as a single group as some who self-declared as 'Clinician' subsequently indicated that they were responding on behalf of their organisation. Two of the three providers did not identify which provider. The general theme from this group relates to the potential emergence of variations in clinical practice leading to inequalities in access and outcomes. Less common themes related to safety, especially in very young patients, the impact on local and national services, and postulations on the characteristics of priority patients.

The non-profit professional responder did not add any further information as to their interest in haemophilia and this individual did not provide any text responses.

The consultation elicited a large number of responses from 'patients' with many respondents declaring they were a close relative of a patient. The overwhelming majority of patient responses were positive and supportive of the policy proposition. The nature of most of these responses was such that a specific response would be inappropriate (e.g. comments such as stating an individual case for treatment, or speculating on the potential benefits of treatment, or posing questions relating to access). There were some specific, albeit isolated, comments relating to particular patient groups such as moderate haemophilia A and older patients.

The consultation elicited four responses from pharmaceutical companies, one of which was from the manufacturer of emicizumab. There was a marked difference in the nature of responses from the manufacturer of emicizumab compared with other pharmaceutical companies, all being manufacturers of recombinant factor VIII (rFVIII) which is the current standard of care. In general, the pharmaceutical companies provided detailed responses to the consultation questions compared with other respondents. The general themes of the pharmaceutical company responses relating to the policy included the interpretation of the evidence base and highlighting the emerging safety profile of emicizumab. Other issues related to specific components of the budget impact model, the monitoring of clotting parameters in the presence of emicizumab and drawing comparisons between the policy parameters and constraints which may be in place relating to the current standard of care.

5. How have consultation responses been considered?

Responses have been carefully considered and noted in line with the following categories:

- Level 1: Incorporated into draft document immediately to improve accuracy or clarity
- Level 2: Issue has already been considered by the CRG in its development and therefore draft document requires no further change
- Level 3: Could result in a more substantial change, requiring further consideration by the CRG in its work programme and as part of the next iteration of the document
- Level 4: Falls outside of the scope of the specification and NHS England's direct commissioning responsibility

Table 2: Summary of number and nature of PWG responses

	Clinician / Provider	Non-Profit Professional	Patient or Representative	Pharmaceutical Company	Total
Level 1	0	0	0	4	4
Level 2	9	0	1	32	42
Level 3	0	0	1	1	2
Level 4	2	0	2	11	15

Total	11	0	4	48	63

A number of consultation responses were considered carefully and did not warrant a specific response from the Policy Working Group (PWG) and have not been categorised as above. For example, responses which posed questions relating to access, or which were wholly supportive of the policy without raising any specific issues, or which implored the NHS to commission the treatment, or which described patient experiences etc. Most responses of this nature came from the 'patient' group.

6. Has anything been changed in the policy as a result of the consultation?

Yes. Many consultation responses considered did not require a specific response or were out of scope of the policy process. A small number of consultation responses (n = 4) have resulted in minor amendments and points of clarification to the policy proposition. Many of the responses, especially those from commercial parties, essentially came down to a difference of opinion in the presentation or interpretation of the clinical evidence..

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

None. The PWG, and by extension the related Clinical Reference Group, is already aware of the potential disruptive nature of emicizumab in the management of haemophilia A and will work with all parties to ensure that any transition to a new standard of care is efficient with minimal patient impact.