Consultation Report

Topic details

Title of policy or policy statement:	Vonicog alfa for the treatment and prevention of bleeding in adults with von Willebrand disease
Programme of Care:	Blood and Infection
Clinical Reference Group:	Specialised Blood Disorders
URN:	1709

1. Summary

The public consultation for the NHS England Clinical Commissioning Policy proposition, Vonicog alfa for the treatment and prevention of bleeding in adults with von Willebrand disease (ref 1709), was open for 30 days from 19th August to the 17th September 2019.

Vonicog alfa is licensed for the management of von Willebrand disease in adults when desmopressin treatment alone is ineffective or not indicated for the treatment of haemorrhage and surgical bleeding, and for the prevention of surgical bleeding. It is not yet licensed in children or for routine (non-surgical) prophylaxis. This vonicog alfa policy proposition only relates to the current licensed indication.

2. Background

Von Willebrand disease (VWD) causes patients to have absent or low levels of a blood protein called von Willebrand factor (VWF), or they may have sufficient VWF but it does not work. This means that people with VWD have difficulty forming a blood clot and they bleed more after events such as injury, childbirth, menstruation, or during surgery including dental procedures. Symptoms can range from mild and barely noticeable to frequent and severe, and can include nosebleeds, bleeding from the gums, easy bruising, and heavy menstrual bleeding. VWD has 3 main types (known as VWD types 1, 2, and 3), each associated with a different phenotype and, in general, with a different degree of severity.

The current standard of care for von Willebrand disease is with the use of plasma-derived products, often with additional factor VIII (8) which is not always required.

Vonicog alfa is a recombinant (synthetic) form of human von Willebrand factor. It works in the body in the same way as von Willebrand factor made by the body itself, by replacing the protein needed to stop bleeding that is missing or not working. It has been artificially made rather than taking it from plasma. Recombinant (synthetic) blood products are generally preferred to the same products obtained from plasma. In addition, unlike many plasma-derived von Willebrand factor products, vonicog alfa does not contain any factor 8 so that co-dosing does not need to be accounted for and the risk of excess factor 8 building up in the body can be mitigated.

3. Publication of consultation

The policy proposition was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 19th August to 17th September 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 proposition 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

Nine separate submissions were received.

- Three submissions were from pharmaceutical companies including the manufacturer of vonicog alfa plus two other manufacturers of competitor products.
- One submission was from a patient representative organisation.
- Four submissions were from clinicians, one of whom declared they were responding on behalf of their organisation which is a hospital trust itself contracted with NHS England for haemophilia/specialised blood disorders. The other clinician submissions were presumably made in a personal professional capacity. Three clinician responses answered 'yes' to each of the first three questions and did not provide any comments to the last two questions other than one question being posed. These three submissions will not be considered further.
- One submission was from a service provider although stated not to be responding on behalf of an organisation. The responses provided were 'no' to each of the first three questions and no comments were provided to either of the last two questions. This submission will not be considered further.

Therefore, all comments and issues which the Policy Working Group considered originated from the three pharmaceutical companies plus the patient organisation plus one of the clinician responses. A summary of all responses is provided in table 1.

Table 1.
Summary table of consultation submissions in respect of the vonicog alfa policy proposition (ref 1709)

	Has all the relevant evidence been taken into account?	Does the impact assessment fairly reflect the likely activity, budget and service impact?	Does the policy proposition accurately describe the current patient pathway?	Provide any comments about the potential impact on equality and health inequalities	Are there any changes or additions you think need to be made to the policy proposition?
Pharmaceutical company 1	**No**	**No**	**Yes**	Null	Comments provided
Pharmaceutical company 2	**No**	**No**	Yes	Null	Comments provided
Pharmaceutical company 3	**No**	**No**	**No**	Null	Comments provided
Patient organisation	**No**	**No**	Yes	Comments provided	Null
Clinician 1	Yes	Yes	Yes	Null	Null
Clinician 2	Yes	Yes	Yes	Null	Null
Clinician 3	Yes	**No**	Yes	Comments provided	Comments provided
Clinician 4	Yes	Yes	Yes	Question posed	Null
Service provider	No	No	No	Null	Null

Bold = additional comments provided

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

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

Minor points of clarification and other edits of no material consequence to the proposed criteria for commissioning have been made to the policy proposition. All of these changes were of level 1. A number of points made, or issues raised, were of level 2 and did not result in any change to the policy proposition. None of the submissions received as part of the consultation received a level 3 response. A few responses related to the budget impact model which was not itself part of the consultation and consequently these responses have been categorised as level 4.

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

The PWG and several of the consultation submissions expressed concern that the Impact Assessment, and in turn the associated Budget Impact Model, did not accurately reflect the likely impact of vonicog alfa. Specifically concerns were that the modelled use of vonicog alfa had been overestimated due to use of unrepresentative trial results, and data gaps from the National Haemophilia Database leading to an underestimate of current use for the specific patient group. As a result, it was agreed further work on the impact assessment would be undertaken.

The impact assessment and financial model has been refreshed using comprehensive and up-to-date sales figures from the Commercial Medicines Unit. The model assumes vonicog is used on 1:1 basis when compared with current treatments. The revised and simpler modelling has been signed off by the Specialised Commissioning Finance team. The net effect was a modest reduction in the expected budget impact.

Further amendment to Budget Impact Model 2020:
The manufacturer submitted evidence from independently published sources to indicate that the mean number of vonicog alfa units used to control bleeding episodes in patients with VWD would be lower than assumed in the earlier model. This yields an expected use ratio of about 3:1 of current products to vonicog alfa (in VWF units). This change reduces the net budget impact.

END.