SPECIALISED COMMISSIONING - CLINICAL EVIDENCE EVALUATION CRITERIA FOR A PROPOSITION FOR A CLINICAL COMMISSIONING POLICY

URN: 1716
TITLE: Human coagulation factor X for the treatment and prophylaxis of bleeding episodes and perioperative management in people with hereditary factor X deficiency

CRG: Specialised Bleeding Disorders
NPOC: Blood & Infection
[redacted]

Date: 18/11/17

The panel were presented a policy proposal for routine commissioning.

Question	Conclusion of the panel
<u>Advice</u> The Panel should provide advice on matters relating to the evidence base and policy development and prioritisation. Advice may cover: • Uncertainty in the evidence base • Challenges in the clinical interpretation and applicability of policy in clinical practice • Challenges in ensuring policy is applied appropriately • Issues with regard to value for money • Likely changes in the pathway of care and therapeutic advances that may result in the need for policy review.	The Panel noted that the policy should be returned to the PWG for further work, as follows: • The main concern raised by the panel was that the policy proposal recommends human coagulation factor x in all clinical circumstances. The policy needs to focus use where there is evidence of significant clinical benefit compared to the existing standard treatments. • For example, it is unclear why patients well managed on current treatments would not continue to receive these treatments. • The flow diagram needs to include the place of Prothrombin complex concentrate (PCC) in the pathway of care. The evidence base is insufficient to show whether human coagulation factor X is superior to PCC but the panel accepted that it is likely to be non-inferior to PCC. The pathway is not clear of the place of this treatment with regards to PPC. This is important as at prioritisation (if this product is more costly than current treatments) there needs to be a clear case for what is likely to be a sub group of patients who may have a particular ability to benefit from human coagulation factor X compared to PCC. • The relative harms from giving PCC in comparison to human coagulation factor X need to be defined in the CPAG Summary

	Report and based on evidence. The current CPAG report could be interpreted to say that human coagulation factor X is effective but there is little to indicate what benefit there is over existing treatment and this will need to be clear for CPAG prioritisation (assuming there is an additional cost). • Panel are anxious to find out whether there were any subgroups who are likely to gain significant benefit in comparison to receiving existing interventions.. • The studies included were not controlled and did not compare outcomes with PCC, fresh plasma or other treatments. The Panel were not able to identify from the research evidence presented whether there was a significant benefit of human coagulation factor X to other treatments (largely PCC). It was not possible to estimate benefit. The evidence suggested that the treatment was effective but did not give an indication how effective compared with comparators and this is important when prioritisation decisions need to be made. The evidence included a theoretical advantage in terms of reduced thrombogenicity and practical advantages in terms of volume of infusion, thus providing better convenience for patients who may otherwise require more than one trip to receive IV infusion. The PWG may wish to identify particular patient groups where one or other of these disbenefits of existing interventions compared with human coagulation factor X could be significant and thus sub-groups where human coagulation factor X could be important. The degree of benefit needs to be clear in order to support any prioritisation decision. The PWG will need to explain more clearly net benefits compared with other treatments. • The PWG should focus either on patient groups with additional benefit or be clearer as to the relative benefit over existing treatments for all or most patients. • Prophylactic treatment is described but this is not supported by the evidence presented. Panel considered that prophylaxis is not part of common practice at present. The PWG may provide further explanation to describe this; the clinical benefits compared with use

	'as required' as this will need to clear if there are additional costs compared with current treatment regimens. The benefits of prophylactic use of human coagulation factor X compared with current patterns of use of PCC will need to be clear. The policy was not accepted and the amended policy should be returned to Panel.

Overall conclusions of the panel

The amended policy should return to another Clinical Panel meeting.

Report approved by:
David Black

Clinical Panel Co-Chair

28/11/17