NHS England

Consultation Report

Topic details

Title of policy or policy statement:	Human coagulation factor X for hereditary factor X deficiency (all ages)
Programme of Care:	Blood & Infection
Clinical Reference Group:	Specialised Blood Disorders
URN:	170105P

1. Summary

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

2. Background

Hereditary factor X (ten) deficiency is a very rare inherited bleeding disorder caused by a lack of a protein called factor X, which is needed for blood to clot properly and therefore to prevent bleeding. It is passed on when both parents of an individual have a defective copy of the factor X gene, and therefore affects both sexes equally. Hereditary factor X deficiency varies in severity depending on the levels of factor X in the blood. Milder forms of the disease typically present as nosebleeds, easy bruising, prolonged menstrual bleeding, musculoskeletal bleeds, excessive bleeding following surgery, and bleeding of the gums. The most severe forms of the condition can result in bleeding in the brain or gastrointestinal tract, which can be life-threatening. Diagnosis of the condition can occur at any age, although more severe forms are often diagnosed within the first month of life. Hereditary factor X deficiency requires life-long treatment which includes preventing or stopping bleeding events.

Treatments for hereditary factor X deficiency often involve the replacement of factor X. Currently, this is done using a treatment called prothrombin complex concentrate (PCC) which contains factor X as well as additional blood clotting factors that patients with factor X deficiency are not deficient in, including factor II (two), VII (seven), and IX (nine). Also, less commonly fresh frozen plasma (a blood product made from the liquid portion of whole blood) is used, however, this also contains other proteins and clotting factors apart from factor X.

Human coagulation factor X (Coagadex®) is a concentrate of the protein called factor X derived from human plasma (the colourless liquid part of blood). It has a marketing authorisation in the UK for the treatment and prophylaxis of bleeding episodes and for perioperative management in patients with hereditary factor X deficiency. It is one of the proteins which is needed for blood to clot. Human coagulation factor X temporarily replaces the missing factor X in people with hereditary factor X deficiency.

3. Publication of consultation

The policy was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 23rd Feb 2018 to 25th March 2018. 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 made to this document, and why?

4. Results of consultation

Two individuals, with one representing one organisation, responded to the public consultation. The organisation advocates for and supports patients with bleeding disorders such as factor X deficiency.

Most of the comments were supportive of the policy proposition and the PWG was grateful to receive these comments.

One respondent raised two issues which merited a response, one regarding wider use of the treatment for example for short term use to manage acute bleeds or specific bleeding episodes, and the other stating that restricting or denying access to the treatment could create inequalities relative to the management of other bleeding disorders.

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

In both cases where a response was merited these were of a level 2 nature.

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

The PWG did not opt to make any amendments to the policy proposition.

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

The PWG does not consider that any concerns remain outstanding following the public consultation.