NHS ENGLAND SPECIALISED SERVICES

CLINICAL PANEL REPORT

Date: 17 April 2024

Intervention: emicizumab

Indication: prophylaxis of bleeding episodes in people with moderate haemophilia A without factor VIII inhibitors (all ages)

Gateway: 2, Round 1

Programme: Blood and Infection

CRG: Specialised Blood Disorder

Information provided to the Panel

Policy Proposition

Title Change report

Evidence Review completed by NICE

Clinical Priorities Advisory Group (CPAG) Summary Report

Evidence to Decision (EtD) Summary

Equalities and Health Inequalities (EHIA) Assessment

Patient Impact Assessment

Blueteq™ Form

Policy Working Group (PWG) Appendix

This Policy Proposition recommends the use of emicizumab in people of all ages as prophylaxis of bleeding episodes in people with moderate haemophilia A without factor VIII inhibitors. Haemophilia A (HA) is an inherited genetic condition characterized by a deficiency of coagulation factor VIII (FVIII) which causes increased bleeding. HAusually affects males, and patients experience abnormal bleeding. Bleeding can be life threatening, and bleeding into joints causes acute pain and over time irreversible arthropathy which impacts mobility. There is no cure for HA. Current and lifelong treatment is with replacement factor VIIII which is given intravenously. Emicizumab is a drug used to prevent bleeding or reduce the number of bleeds in people with HA, administered via subcutaneous injection. Emicizumab is licensed for use in the population covered by this policy proposition. It was previously considered by NICE but was not selected for appraisal at topic selection.

The proposition and the supporting documentation were considered by Clinical Panel members. The evidence review consisted of one open-label, single arm, multi-centre, non-comparative study, which include 72 people with moderate (n=51) or mild haemophilia (n=21) without inhibitors. Outcomes that were considered critical to decision making included: rate of treated bleeding events; rate of all bleeding events, and joint health. Important outcomes included: health related Quality of Life (HRQoL), patient treatment preference, rate of joint bleeding events, and activities of daily living (ADLs). The study provided very low certainty evidence of clinical benefit.

Clinical Panel members debated the evidence and asked questions for clarification. A reduction in bleeding rates were reported compared to what would have been expected without treatment. This was a model-based benefit. Evidence was shown that emicizumab resolved target joints in (20/21) 95% people who had target joints at baseline and were studied for 52 weeks. A trend to improvement in treatment burden was reported and people reported a preference for treatment with emicizumab compared to previous treatment received.

This treatment would address significant resource implications by not needing to administer Factor VIII.

EHIA – the benefit in children and young people was clear regarding ease of administration compared to other treatments.
PIA – no amendments requested.

Recommendation

Clinical Panel recommends that this proposition progresses as a routine commissioning policy proposition. The Panel members, though, asked for assurance from the Policy Working Group (PWG) that the clinical outcomes described were sufficient, before this progresses.

Why the panel made these recommendations

Panel members agreed that the evidence base supported the proposition but required further assurance from the PWG regarding outcomes. The amendments requested could be considered via Chair's action.

Documentation amendments required

Policy Proposition:

- Links and updates to other policies – page 2 – only one published policy is currently referred to and so needs amending.
- Reference to iu/dl needs to be written consistently throughout the proposition.
- Inclusion criteria – reference to more than 3 bleeds in 12 months. Clarification is needed as to whether this is any bleed or only those needed treatment.
- Dosing – a link to the UKHCDO dosing algorithm would provide clarity.

Blueteq™ Form:

- The Blueteq form does not currently pick up the specific phenotypes that are detailed in the flow diagram within the proposition and so needs amending to align.

Declarations of Interest of Panel Members: None received.

Panel Chair: Anthony Kessel, Deputy Medical Director, Specialised Services

Post Panel Amendments

Policy Proposition		
Panel Comment	**Amendment**	**Page number (if applicable)**
Links and updates to other policies – page 2 – only one published policy is currently referred to and so needs amending.	Link updated to include other relevant policy	P2
Reference to iu/dl needs to be written consistently throughout the proposition.	Changed to IU/dL throughout all documentation	N/A
Inclusion criteria – reference to more than 3 bleeds in 12 months. Clarification is needed as to whether this is any bleed or only those needed treatment	Changed to '3 treated bleeds in 12 months' to clarify this	P4
Dosing – a link to the UKHCDO dosing algorithm would provide clarity.	Link added	P5
Blueteq™ Form		
Panel Comment	**Amendment**	**Page number (if applicable)**
The Blueteq form does not currently pick up the specific phenotypes that are detailed in the flow diagram within the proposition and so needs amending to align.	Amended to reflect inclusion criteria of moderate with phenotype and combined with pre-existing severe haemophilia Blueteq	P1