NHS ENGLAND SPECIALISED SERVICES

CLINICAL PANEL REPORT

Date: February 2022

Intervention: Desferrioxamine (DFO) / deferasirox (DFX) and deferiprone (DFP) / deferasirox (DFX) combination therapies

Indication: Treatment of iron overload for transfused and non-transfused patients with chronic inherited anaemias (all ages)

URN: 2109

Gateway: 2, Round 1

Programme: Blood and infection

CRG: Haemoglobinopathies

Information provided to the Panel

Evidence Review x2 completed by Solutions for Public Health – DFO/DFX and DFP/DFX

Policy Proposition

Evidence to Decision Making Summary

Patient Impact Report

Equality and Health Inequalities Assessment (EHIA) Report

Clinical Priorities Advisory Group (CPAG) Summary report

Policy Working Group Appendix

This draft policy proposition recommends DFO/DFX and DFP/DFX combination therapies to be available as a routine commissioning treatment option for iron overload in transfused and non-transfused patients with chronic inherited anaemias not adequately controlled on monotherapy or DFP/DFO combination (all ages).

Clinical Panel members were presented with the evidence base supporting this proposition which consisted of two evidence reviews, six studies.

In the DFO/DFX review, six papers were identified for inclusion, two randomised controlled trials (RCTs), one retrospective cohort study and three prospective case series. The studies included in this review provide moderate to very low certainty evidence that treatment with DFO/DFX may improve measures of iron overload in patients with chronic inherited anaemias and iron overload, and moderate certainty evidence that these improvements may be better in patients receiving DFO/DFX compared with DFX only. They also provide very low certainty evidence that treatment with DFO/DFX may improve some measures of disease response, but there was no comparative evidence for these outcomes. No evidence was identified for quality of life, psychological outcomes, mortality, activities of daily living or cost effectiveness.

In the DFP/DFX review, three papers were identified for inclusion, one RCT and two prospective cohort studies. Limitations found across the studies regarding design. Moderate certainty evidence of a statistically significant improvement in quality of life from baseline to 12 months was found for both DFP/DFX and DFO/DFP combination therapies. No difference was found between the two combination therapy regimens. A statistically significant improvement in serum

ferritin (lower) from baseline to 12 months was demonstrated in both DFP/DFX and DFO/DFP combination therapies.

Panel members discussed what treatment was licenced and what was proposed to be used off-label. They considered this needs to be clearer in the proposition.

Patient Impact Report – no additional comments received.
EHIA – No additional comments received.

Recommendation

Clinical Panel recommends that this proposition progresses as proposed, with the amendments requested.

Why the panel made these recommendations

Clinical Panel members considered that the proposition was written reflective of the evidence base, and concluded that there is sufficient evidence to support a proposition for the routine commissioning of this treatment for the indication.

Documentation amendments required

- Clearly identify which treatments are to be used within their marketing authorisation and which are proposed as off label use.
- Governance section – check the wording reading the Drugs and Therapeutics Committee that it aligns with the standard statement usually used.

Declarations of Interest of Panel Members: None

Panel Chair: James Palmer, National Director, Specialised Services

Post Panel Note

POC Note: The amendments as above have been undertaken and have been reflected in the documentation.

10 June 2022

FINAL