NHS ENGLAND SPECIALISED SERVICES

CLINICAL PANEL REPORT

Date: 19th April 2023

Intervention: Obinutuzumab

Indication: prevention of immune Thrombotic Thrombocytopaenic Purpura (TTP) relapse in patients who are refractory or intolerant to rituximab (adults)

URN: 2255

Gateway: 2, Round 1

Programme: Blood and Infection

CRG: Specialised Blood Disorder

Information provided to the Panel

Cover letter

Policy Proposition

Clinical Priorities Advisory Group Summary Report

Equalities and Health Inequalities Assessment (EHIA)

Patient Impact Assessment (PIA) Report

Evidence Reviews x2 by NICE – acute and prevention

Evidence to Decision Making Summary

Blueteq™ Forms – adult initial funding, adult re-treatment, Medicines for Children initial and Medicines for Children re-treatment

Policy Working Group Appendix

This Policy Proposition recommends the routine commissioning of obinutuzumab as treatment option for elective therapy to prevent acute immune thrombotic thrombocytopenic purpura (TTP) relapse. Immune TTP is a rare, potentially life-threatening condition that involves blood clots in the small blood vessels in the body, leading to end organ damage and mortality in >90% of acute episodes. Immune TTP is caused by a lack of the enzyme ADAMTS13. Obinutuzumab is a humanised type II monoclonal antibody that targets the CD20 molecule, improving ADAMTS13 levels. This policy proposition is restricted to adults, with access for post-pubescent children via the medicines for children policy.

Clinical Panel was presented with an overview of the treatment and the evidence review supporting the proposition. The evidence review consisted of one retrospective non-comparative case series of 15 people. Eight participants received obinutuzumab and seven received ofatumumab. Of the eight receiving obinutuzumab, six had ADAMTS13 relapse and therefore fitted the evidence review parameters. The evidence was of very low certainty; however, this was acknowledged this would be the case as this is a rare disease with a very small eligible population. Most outcomes reported in the study were for a mixed population in terms of treatment received and clinical condition presented. The results however did

demonstrate a complete or partial remission. The median time to complete remission as 15 days. Median relapse-free survival was 15.4 months. Very few adverse events reported.

No evidence was identified for cost effectiveness, quality of life or subgroups.

The Panel members heard that ofinutuzumab formulation is not available for use.

The proposition was considered, and a few amendments required.

EHIA – no amendments recommended.

PIA – no amendments recommended.

Recommendation

Clinical Panel recommends this proceeds as a routine commissioning policy proposition.

Why the panel made these recommendations

Clinical Panel members considered that the evidence base, albeit of very low certainty in a very small population, supports the proposition.

Documentation amendments required

Policy Proposition:

- Page 6 – reference to the multidisciplinary team (MDT) needs enhancing and MDT needs adding into the diagram on page 7.
- Stopping criteria: need to state that treatment will be stopped in the event of adverse incidents.
- Monitoring is stated as every 3 – 6 months. This needs to be clarified if this is whilst still on treatment.

Blueteq™ Forms:

- Footnotes in forms relating to adults are missing references to the Medicines for Children Policy and these need adding.
- The re-treatment form needs to refer to off label use of the medicine.

Declarations of Interest of Panel Members: none.

Panel Chair: James Palmer, National Medical Director, Specialised Services

Actioned Amendments:

Policy Proposition:

- Page 6 – reference to the multidisciplinary team (MDT) needs enhancing and MDT needs adding into the diagram on page 7.

  Actioned – MDT box and 'not suitable' route added to flow diagram.

- Stopping criteria: need to state that treatment will be stopped in the event of adverse incidents.

  Actioned – wording amended.

- Monitoring is stated as every 3 – 6 months. This needs to be clarified if this is whilst still on treatment.

  Actioned – wording amended.

Blueteq™ Forms:

- Footnotes in Medicines for Children Blueteq™ inclusion criteria are missing and these need adding to align with the Adult Blueteq™.

  Actioned

- The re-treatment form needs to refer to off label use of the medicine.

  Actioned