NHS ENGLAND SPECIALISED SERVICES

CLINICAL PANEL REPORT

Date: June 2021

Intervention: Canakinumab

Indication: for patients with Still's disease refractory to anakinra and tocilizumab (adults and children 2 years and over)

URN: 2002

Gateway: 2, Round 1

Programme: Blood & Infection

CRG: Immunology and Allergy

Information provided to the Panel

Policy Proposition

Evidence review completed by Solutions for Public Health x 2

Equality and Health Inequalities Assessment (EHIA) Report

Clinical Priorities Advisory Group (CPAG) Summary Report

Patient Impact Form

Policy Working Group Appendix

Blueteq® Form

Key elements discussed

This policy proposition recommends the routine commissioning of canakinumab as a 4th line treatment option for patients 2 years and over with Still's disease. Still's disease includes systemic-onset juvenile idiopathic arthritis (SJIA) in children and adult-onset Still's disease (AOSD) in adults. Still's disease presents heterogeneously but may include joint pain, rash, weight loss and muscle ache. Still's disease is currently managed by 1st line treatments with non-steroidal anti-inflammatory drugs and corticosteroids, 2nd line treatments of disease-modifying antirheumatic drugs and 3rd line treatment with anakinra and tocilizumab. Canakinumab is proposed as an off- label 4th line treatment. 22 patients a year would be likely to meet the proposed access criteria.

Two evidence reviews were presented. One for SJIA and one for AOSD. The evidence review on SIJA comprised of 3 papers, one retrospective study and 2 prospective studies. 49 patients within the relevant group participated in the studies. There was low or no evidence for critical outcomes and no data on quality of life. Symptomology was assessed using varied scoring systems but focused on remission. The 3 studies reported remission between 11.5%-100%. There were low levels of evidence on safety but the data presented no concerns. There was no data on cost effectiveness data or further subgroups. Overall, there was weak evidence on symptom control and steroid use reduction. The AOSD evidence review comprised of one multicentre retrospective case series. 4 patients were in the relevant patient group. The majority

of patient were found to experience remission of symptoms. There was no strong safety data, no cost effectiveness data and no subgroups identified.

Panel discussed whether intravenous immunoglobulin (IVIg) not being routinely commissioned for SJIA would impact on patient accessing this treatment. It was not expected to have an impact as IVIg had been superseded by other treatment options. Panel also queried whether the canakinumab was off-label for this indication as it was licensed for 3rd line treatment. It was agreed this should be clarified.

Clinical Panel considered the policy proposition to be well written with a clearly defined patient group and consider that it provided a treatment option for a patient group where symptom management is challenging. It was noted the efficacy criteria required a less significant drop in DAS28 score than for other conditions.

Panel noted grammatical errors within the inclusion criteria. The reassessment check time point for adults was missing. It was suggested the stopping criteria should be revised to state '*the treatment is not effective as outlined in the reassessment criteria'* or that there is limited response to the efficacy criteria. The Policy Working Group should consider if the stopping criteria should state no response or limited response. It was agreed the exclusion criteria of infection should be defined.

Blueteq® form – no additional comments received.

EHIA – no additional comments received.

Patient Impact Form – no additional comments received.

Recommendation

Clinical Panel recommends that this proposition progresses as a routine commissioning proposition.

Why the panel made these recommendations

The Panel debated the evidence base and considered it was reflected by the policy proposition.

Documentation amendments required

Policy Proposition:

- Pharmacy Lead to clarify with MHRA if this is an off-label treatment. Amend proposition if necessary.
- Amend 'has' to have' in inclusion criteria.
- Add time point for adult reassessment
- Revise stopping criteria to state the treatment is not effective as outlined in the reassessment criteria' or that there is limited response to the efficacy criteria. Amend according the PWG advise on 'no response' or 'limited response'.
- Define infection within exclusion criteria.
- Remove '*Provider organisations must register all patients…*' statement from starting criteria.
- Flowchart:
    - Add 'no' as an outcome for the first diagnosis/evaluation box.
    - amend treatment option box to 'withdraw' instead of 'consider withdrawing'

Declarations of Interest of Panel Members: None received.

Panel Chair: James Palmer, Medical Director Specialised Services

Post-panel amendments

The policy working group has made the following amendments following the advice from Clinical Panel:

- MHRA has confirmed that this is considered to be an off-label treatment. No changes to the policy proposition have been made.
- The inclusion criteria have been amended to the correct tense (from ‘has’ to ‘have’).
- The ‘Reassessment’ section and ‘Stopping criteria’ section have been amended for clarity around the difference between ‘no response’ and ‘limited response’ and the differences.
- A note has been made to refer to the Summary of Product Characteristics in relation to defining infections within the exclusion criteria.
- The Prior Approval paragraph has been removed from the ‘Starting criteria’ section.
- The Patient Pathway diagram has been amended as suggested.