NHS ENGLAND SPECIALISED SERVICES

CLINICAL PANEL REPORT

Date: 19 June 2019

Intervention: Ustekinumab

Indication: children and adolescents with paediatric Crohn's disease (3-18 years)

ID: 1861

Gateway: 2 (Round 1)

Programme: Women and Children

CRG: Paediatric Medicine

Information provided to the panel

Cover Note from the Programme of Care

Policy Proposition

Evidence Review undertaken by NICE Medicines and Technologies Programme

CPAG Summary Report

Key elements discussed

This is a not for routine commissioning policy proposition for active Crohn's disease and not responding to current treatment. Ustekinumab is a biological medicine that is licensed in 4th line treatment for moderate to severe active Crohn's disease in adults. This would be an off-label use (covered by a NICE STA for adults).

The evidence review only consisted of one retrospective cohort study which included 44 children with refractory Crohn's disease, a median age of 16 years (13-17). The study included a reference to a US study with four patients. Panel considered there was no evidence base for pre-pubertal children. The study reported 36% remission at 3 months although it wasn't clear regarding longer term remission in remission. 9% children had surgery although the study does not report what this was and if related to the disease in focus. Height unchanged, weight and BMI increased slightly. The study did suggest some evidence of clinical remission in 1/3 of the patients and improved symptoms in ½.

One third of patients had to have treatment stopped due to adverse events (serious in two children) so the Panel questioned the safety and tolerability of patients.

Study results should be considered with caution as not controlled, small volume, did not use standardised treatment regimens.

Dose regimen in the evidence base is unclear and children were treated as an individual rather than there being standard treatment regimens and monitoring. The induction regime in study was different than in the licensed indication and did not evidence intravenous delivery benefit.

NICE have a positive Technology Appraisal covering adults with this condition which has a stronger evidence base which could translate to post pubertal children. Discussion took place regarding the proposition title as the Programme of Care recommended changing the title to pre-pubescent children so post pubescent children would be able to access through the NHS England Medicines for Children policy.

A randomised controlled trial (RCT) is currently recruiting (population of 2-17 years) and will be due to report in 2023. Panel recommend a review of the evidence base once this RCT reports.

Recommendation

Clinical Panel recommend progressing as a not for routine policy proposition, as proposed.

Why the panel made these recommendations

The evidence base is sparse in pre-pubertal children with no standard treatment pathways or monitoring. Await RCT report in 2023 and then review the evidence base.

Documentation amendments required

Change the policy proposition title to pre-pubertal children rather than children and young people.

Declarations of Interest of Panel Members: None

Panel Chair: James Palmer, Medical Director

Post Panel Note:

Policy proposition name changed as requested by Clinical Panel.