NHS ENGLAND SPECIALISED SERVICES

CLINICAL PANEL REPORT

Date: June 2020

Intervention: Abatacept

Indication: severe treatment-resistant morphoea (localised scleroderma)

URN: 1921

Gateway: 2, Round 1

Programme: Internal Medicine

CRG: Specialised Dermatology

Information provided to the Panel

Clinical Panel Preliminary Policy Proposition Report

Policy Statement Proposition

Evidence Review completed by Public Health England

Equalities and Health Inequalities Assessment (EHIA) Report

Clinical Priorities Advisory Group (CPAG) Summary Report

Blueteq forms – initiation and continuation

Key elements discussed

This policy statement proposition has been developed as a for routine commissioning proposition. Morphoea (or localised scleroderma) is an autoimmune disorder of the connective tissues that causes an initial inflammatory reaction and then sclerotic changes. It is considered there is a high level of unmet need. Abatacept is currently licenced (and approved by NICE) for the treatment of rheumatoid and psoriatic arthritis in adults and juvenile idiopathic arthritis in children. It is not currently licenced for use in morphoea but is a recognised treatment option in a subset of patients with severe treatment resistant disease.

The evidence review was presented which includes three studies. Panel heard that the evidence is limited. Sample sizes in the studies were very small, the studies were unblinded, and it was unclear in some patients whether there were comorbidities and what other treatments were being used. There were no comparative studies included. Therefore, there was potential for bias.

The majority of patients received intravenous (IV) doses although a few patients did transfer to subcutaneous (SC) injections. Panel questioned whether efficacy was the same for either delivery method. It was considered it was.

Panel members were unclear how severe morphea is being defined. The proposition referred to a different number of subtypes in different sections and this led to some confusion. There needs

to be consistency through the proposition with a clear section defining severity. Panel considered that a lot of the information on specific subtypes was not needed. They requested that the Clinical Reference Group be asked to help define severity in these patients.
Panel discussed whether there was evidence regarding other treatment options. However it was considered that these were not valid treatment options used in England. The proposition could include a sentence on this.

The criteria were discussed. Some reordering of the criteria is required to make it clear and flow better. Both implementation criteria and starting criteria were not considered to be needed. The criteria also need to consistent with the criteria included in the Blueteq forms as currently there is some inconsistency.

Recommendation

Clinical Panel recommends that this proposition progress as a for routine commissioning policy statement proposition once the amendments requested have been made. A revised version of the proposition needs to be reviewed by the Clinical Director for Internal Medicine and the National Programme of Care Clinical Lead for Blood and Infection.

Why the panel made these recommendations

The Panel considered that the proposition was reflective of the evidence base presented, although acknowledged that the evidence base was very limited.

Documentation amendments required

Policy Proposition:

- The introduction needs to state that this treatment can be administered either by IV or SC injection.
- Page 1 – 2nd paragraph after the condition needs to have less information on subtypes and just state that it covers varied clinical manifestations. Seek CRG advice on defining what severity is. Then this needs to be consistent through the proposition.
- Include a statement on other treatment options not valid options within UK.
- Page 2 – the last sentence in the section titles 'Current Treatment' -put in a comma after hydroxychloroquine and cross out the 'the' after the word increasing.
- Page 4 - criteria for implementation and starting – don't need both. Begin with the section as is current up to the last bullet point ending with 'deep structures'. Then insert the section that defines severity, then continue as written.
- The starting criteria in the proposition and the Blueteq form need to be amended to be consistent. Specifically that the first criterion re definition of severe disease is identical in Blueteq form and policy, and that the criterion referencing psychological deterioration is added to the Blueteq form.
- 'Page 5 – add comment 'as per Summary of Product Characteristics' to section re Induction dose of 500-1000mg (weight dependent) at baseline.'
- Page 7 – schematic – say 'meet criteria' rather than 'agreement with criteria'.
- Clarity of severe in criteria is required.
- Page 5 and 6 – monitoring - should include the criteria e.g. Rodnan skin score. Stopping criteria – state when you are monitoring x, y and z then you should stop.
- The proposition includes that a review is required at 6 months. However, it also states that a review is undertaken at 12 months when any treatment decisions would be made (between the graph and the text). Clarity is required whether the 6 month review is required is no treatment decision is being made at that point.

- The proposition needs to be clear whether data collection is mandatory or optional.

Declarations of Interest of Panel Members: None.

Panel Chair: James Palmer, Medical Director Specialised Services.

Post-Panel Note

PWG response to requested amendments

Policy Proposition:

1. The introduction needs to state that this treatment can be administered either by IV or SC injection.
   Response: This was added.
2. Page 1 – 2nd paragraph after the condition needs to have less information on subtypes and just state that it covers varied clinical manifestations. Seek CRG advice on defining what severity is. Then this needs to be consistent through the proposition. Background has been amended to give further information on severity.
   Response: There is no single definition of severity. Scoring systems used to assess response to treatment are not reflective of the severity of the disease in many patients, hence why there has been a focus on impact on mobility and function.
3. Include a statement on other treatment options not valid options within England.
   Response: An email written by the clinical lead to be provided separately to clinical panel details why these treatment options are not considered viable.
4. Page 2 – the last sentence in the section titles 'Current Treatment' -put in a comma after hydroxychloroquine and cross out the 'the' after the word increasing.
   Response: Added.
5. Page 4 - criteria for implementation and starting – don't need both. Begin with the section as is current up to the last bullet point ending with "deep structures". Then insert the section that defines severity, then continue as written.
   Response: Amended as advised. Section re-structured to be clear on what combination of criteria is required.
6. The starting criteria in the proposition and the Blueteq form need to be amended to be consistent. Specifically, that the first criterion re definition of severe disease is identical in Blueteq form and policy, and that the criterion referencing psychological deterioration is added to the Blueteq form.
   Response: Amended.
7. Page 5 – add comment 'as per Summary of Product Characteristics' to section re Induction dose of 500-1000mg (weight dependent) at baseline.
   Response: Added.
8. Page 7 – schematic – say 'meet criteria' rather than 'agreement with criteria'.
   Response: Amended.
9. Clarity of severe in criteria is required.
   Response: As in point two, PWG have advised that the scores alone do not define severe disease well, and impact on patient including quality of life and mobility are more accurate in identifying severe cases. They recognise the importance of objective measures of disease severity, but these are not reliable for this small group of patients.
10. Page 5 and 6 – monitoring - should include the criteria e.g. Rodnan skin score. Stopping criteria – state when you are monitoring x, y and z then you should stop.
    Response: Added.
11. The proposition includes that a review is required at 6 months. However, it also states that a review is undertaken at 12 months when any treatment decisions would be made

(between the graph and the text). Clarity is required whether the 6 month review is required is no treatment decision is being made at that point.
Response: The PWG have requested the requirement for 6 month review remain, in order to improve the evidence base for the efficacy and safety of abatacept in these patients. They have advised that the decision to cease treatment should not be taken until 12 months (unless on safety grounds) as this is the earliest timepoint clinicians could confidently assess treatment to be of no benefit.

12. The proposition needs to be clear whether data collection is mandatory or optional.
Response: PWG have decided data collection should be mandatory and have identified a pre-existing system (SMART for localised systemic scleroderma) that can be utilised to create a national audit.