Engagement Report

Topic details

Title of policy or policy statement:	**Abatacept for autoimmune complications of primary immunodeficiencies caused by CTLA-4 or LRBA genetic mutation in those aged 2 years and older**
Programme of Care:	**Blood and Infection**
Clinical Reference Group:	**Specialised Immunology and Allergy**
URN:	**2309**

1. Summary

This report summarises the feedback NHS England received from engagement during the development of this policy proposition, and how this feedback has been considered.

2. Background

Primary immunodeficiencies (PID) are rare heritable conditions where the body's immune system does not work properly and in some cases attacks itself. The illness can affect one or many parts of the immune system. One of the presentations of PID is of chronic immune dysregulation which may cause autoimmune disease including chronic inflammation. This may be caused by dysfunction of the regulatory T (T_{Reg}) cell, an immune cell that is essential for preventing autoimmunity. Single gene mutation of the lipopolysaccharide (LPS)-responsive and beige-like anchor protein (LRBA) or cytotoxic T-lymphocyte associated protein 4 (CTLA-4) genes have been identified to lead to dysregulation of the T_{Reg} cell.

In severe cases, the autoimmune complications may be life-threatening and the life expectancy of patients with PID who suffer with autoimmune complications is considerably shortened. Patients are also vulnerable to infection as they are unable to make effective antibodies. As a result, most patients will be established on immunoglobulin (Ig) replacement therapy.

Currently there is no nationally agreed first line treatment for the autoimmune complications of PID and each complication tends to be managed in isolation by immunology specialists in tertiary centres. This is usually with steroids or off label options such as sirolimus and non-specific immune suppressant agents, such as azathioprine or mycophenolate mofetil.

Abatacept is a biological drug that specifically targets T_{Reg} cells. It is licensed for rheumatoid and psoriatic arthritis in adults and for polyarticular juvenile idiopathic arthritis in children aged two years and older. Abatacept can be given intravenously or subcutaneously. Abatacept is proposed to be used as first line long-term treatment for the autoimmune or inflammatory complications that arise due to T_{Reg} cell dysfunction.

Abatacept is used to maintain remission in this condition. This proposed use of abatacept is off label.

3. Engagement

NHS England has a duty under Section 13Q of the NHS Act 2006 (as amended) to 'make arrangements' to involve the public in commissioning. Full guidance is available in the Statement of Arrangements and Guidance on Patient and Public Participation in Commissioning. In addition, NHS England has a legal duty to promote equality under the Equality Act (2010) and reduce health inequalities under the Health and Social Care Act (2012).

The policy proposition underwent a two-week stakeholder testing between the 20th of February and 5th March 2024 with registered stakeholders from the following Clinical Reference Groups:

- Specialised Paediatric Allergy, Immunology and Infectious Disease
- Specialised Immunology and Allergy Services

Respondents were asked the following consultation questions:

- Do you believe that there is any additional information that we should have considered in the evidence review?
- Do you support the inclusion criteria set out in the policy proposition?
- Do you support the exclusion criteria set out in the policy proposition?
- Do you have any further comments on the proposal?
- Do you support the Equality and Health Inequalities Impact Assessment?
- Does the Patient Impact Summary present a true reflection of the patient and carers lived experience of this condition?
- Please declare any conflict of interests relating to this document or service area.

The comments have then been shared with the Policy Working Group to enable full consideration of feedback and to support a decision on whether any changes to the proposition might be recommended.

A 13Q assessment has been completed following stakeholder testing.

The Programme of Care has decided that the proposition offers a clear and positive impact on patient treatment, by potentially making a new treatment available which widens the range of treatment options without disrupting current care or limiting patient choice, and therefore further public consultation was not required. This decision has been assured by the Patient Public Voice Advisory Group.

4. Engagement Results

Five stakeholders responded:

- Two patient charity
- Two clinicians
- One patient

5. How has feedback been considered?

Responses to engagement have been reviewed by the Policy Working Group and the Blood and Infection PoC. The following themes were raised during engagement:

Keys themes in feedback	NHS England Response
Relevant Evidence	
One additional paper was identified which was published a week after the original search was run for the independent evidence review: *Taghizade, N. et al. (2023) 'Therapeutic modalities and clinical outcomes in a large cohort with LRBA deficiency and CTLA4 insufficiency', Journal of Allergy and Clinical Immunology, 152(6), pp. 1634–1645. doi:10.1016/j.jaci.2023.08.004.*	Noted. The assessment of the evidence is written up in the public health evidence report.
Policy proposition	
All stakeholders supported the policy proposition and agreed with the inclusion and exclusion criteria.	Noted.
Potential impact on equality and health inequalities	
All stakeholders agreed with the equalities and health inequalities impact assessment.	Noted.
Patient impact assessment	
Some stakeholders felt that the patient impact assessment did not accurately reflect the severity of the condition.	Noted.
Changes/addition to policy	
It was highlighted during stakeholder testing that the 50mg and 87.5mg subcutaneous formulation of abatacept are not marketed in the United Kingdom and thus not available as treatment options.	Noted. Subcutaneous abatacept is available via Clinigen on a named patient basis, at the agreed Patient Access Scheme (PAS) Price.

6. Has anything been changed in the policy proposition as a result of the stakeholder testing and consultation?

The following change(s) based on the engagement responses has (have) been made to the policy proposition:

- Patient impact assessment updated to highlight severity of the disease

7. Are there any remaining concerns outstanding following the consultation that have not been resolved in the final policy proposition?

No.