Engagement Report

Topic details

Title of policy or policy statement:	Baricitinib for use in monogenic interferonopathies (adults and children 2 years and over)
Programme of Care:	Blood and Infection
Clinical Reference Group:	Immunology and Allergy
URN:	1930

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. There were ten respondents, with nine supporting the policy proposition progressing. One respondent did not support the policy proposition due to it excluding children under 2 years.

2. Background

Monogenic interferonopathies are a group of conditions where too many interferons are produced because of a problem with a single gene. Interferons are proteins that are produced by the immune system, usually in response to viruses. There are a wide range of potential symptoms such as severe skin rashes, gangrene, arthritis, breathing difficulty and abnormal blood counts. These conditions can lead to poor quality of life, the requirement for frequent hospital admissions and can eventually be fatal.

The current treatment for monogenic interferonopathies is to treat the symptoms, which is often with corticosteroids. There are no treatments that are currently used which stop the progression of the disease. Baricitinib is a tablet that can help to treat monogenic interferonopathies and can slow down and stop the progression of the disease.

NHS England has carefully reviewed the evidence to treat monogenic interferonopathies with baricitinib. We have concluded that there is enough evidence to make the treatment available for adults and children 2 years and over at this time. This policy proposition has been developed by a Policy Working Group made up of specialist clinicians, patient carers, a Specialist Pharmacist, a Public Health Consultant and a commissioner.

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 was sent for stakeholder testing for 2 weeks from 9th December to 23rd December 2020. 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.

Respondents were asked the following questions:

- Do you support the proposal for baricitinib to be available for monogenic interferonopathies through routine commissioning based on the evidence review and within the criteria set out in this document?
- Do you believe that there is any additional information that we should have considered in the evidence review? If so, please give brief details.
- Do you believe that there are any potential positive and/or negative impacts on patient care as a result of making this treatment option available? If so, please give details.
- Do you have any further comments on the proposition? If Yes, please describe below, in no more than 500 words, any further comments on the proposed changes to the document as part of this initial 'sense check'.
- 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.

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.

Engagement results

There were ten respondents to the stakeholder testing request. Respondents by profession are in Figure 1.

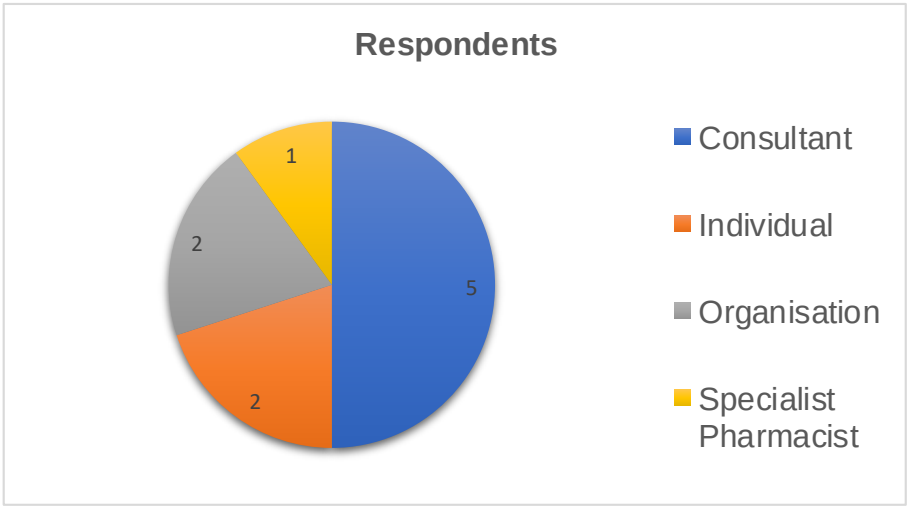

Figure 1: respondents by profession (if given)

Nine of the ten respondents supported the policy progressing with some comments. The general themes of these comments are described in further detail in section 5 of this document. One respondent did not support the policy proposition due to it excluding children under 2 years.

In line with the 13Q assessment it was deemed that further public consultation was not required.

4. How has feedback been considered?

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

Keys themes in feedback	NHS England Response
Relevant Evidence	
Children under 2 years with a monogenic interferonopathy would benefit from treatment with baricitinib.	There is limited efficacy data for children under 2 years, coupled with very limited safety data available. A Paediatric Investigation Plan (PIP) for baricitinib in children from 2 to less than 18 years with moderate to severe atopic dermatitis has been accepted by the EMA. Until further safety data has been published for the use of baricitinib in children under 2 years the inclusion of this age group is not possible.
Current Patient Pathway	
Interferon score should not be a requirement, as it is not widely available.	As the direct or indirect measurement of interferon is not widely available, the policy has been amended to encourage the use of one of these tests to aid diagnosis if the clinical phenotype is unclear but is no longer a requirement.
Outcomes should be tracked to improve available evidence of efficacy.	It is recognised that it would be useful to track the outcomes of patients that receive baricitinib as part of this policy; however, outcomes cannot be mandated as part of the Prior Approval System. There are also no known national registries for this cohort of patients.
Potential impact on equality and health inequalities	
Exclusion of children under 2 years will negatively affect this age group.	There is limited efficacy data for children under 2 years, coupled with very limited safety data available. A Paediatric Investigation Plan (PIP) for baricitinib in children from 2 to less than 18 years with moderate to severe atopic dermatitis has been accepted by the EMA. Until further safety data has been published for the use of baricitinib in children under 2 years the inclusion of this age group is not possible.

	The EHIA has been updated to reflect that this policy may have a negative impact on children under 2 years.

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

The following changes based on the engagement responses have been made to the policy proposition:

- Minor change to the inclusion criteria, acknowledging that direct or indirect measurement of interferon may not be possible throughout the country
- Minor changes to the plain language summary to improve clarity
- Minor changes to the condition information to reflect potential severity of disease
- Minor changes to the dosing schedule

The following changes based on the engagement responses have been made to the EHIA:

- Updated to reflect that the policy may have a negative impact on children under 2 years.

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

There are no remaining concerns outstanding following the stakeholder engagement.