

Consultation Report

Title of policy or policy statement:	Canakinumab for Periodic Fevers
Programme of Care:	Blood and Infection
Clinical Reference Group:	Immunology and Allergy
URN:	1813

1. Summary

This report summarises the outcome of a public consultation that was undertaken to test the policy proposition.

2. Background

2.1. Periodic fever and autoinflammatory diseases are a group of very rare genetic conditions that occur in children and adults. In this policy proposition, the diseases included are:

- a) Familial Mediterranean Fever (FMF)
- b) Hyperimmunoglobulin D syndrome (HIDS) also known as Mevalonate Kinase Deficiency (MKD)
- c) Tumour necrosis factor receptor-associated periodic syndrome (TRAPS)

2.2 These diseases affect adults and children; symptoms include recurrent attacks of fever, pain, enlarged neck glands, abdominal pain with vomiting and diarrhoea as well as more general symptoms such as aching limbs, large joint arthritis, headache, rash and mouth ulcers. People with all these conditions have an abnormal immune system response that results in recurring bouts of inflammation. The symptoms of inflammation include: high temperature (fever); severe fatigue; severe abdominal, chest or joint pains; headaches; rash; and mouth ulcers. Vital organs may be damaged, including the kidneys and brain.

2.3 These diseases can also eventually cause a condition called systemic amyloidosis that affects the kidney and causes kidney failure through build-up of abnormal protein in the tissues.

2.4 As these conditions are very rare, prior to definitive diagnosis, some patients have unnecessary investigative surgery, for example, exploratory laparotomy for peritonitis, arthroscopy to exclude infective arthritis, appendectomy, cholecystectomy, surgery for endometriosis. When the symptoms are so severe, they can reduce quality of life and cause significant disability.

2.5 Canakinumab is effective in 'switching off' the abnormal immune response and patients can resume a normal, pain and symptom-free life in the main.

2.6 Canakinumab is a licensed treatment for these diseases. NHS England has commissioned canakinumab for many years to treat cryopyrin-associated periodic syndrome (CAPS).

2.7. Canakinumab is administered by injection on an eight-twelve weekly basis.

3. Publication of consultation

The policy proposition was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 2nd August to the 1st September 2019. Consultation comments have then been shared with the Policy Working Group (PWG) to enable full consideration of feedback and to support a decision on whether any changes to the policy might be recommended.

Respondents were asked the following consultation questions:

- Has all the relevant evidence been taken into account?
- Does the impact assessment fairly reflect the likely activity, budget and service impact? If not, what is inaccurate?
- Does the policy proposition accurately describe the current patient pathway that patients experience? If not, what is different?
- Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?
- Are there any changes or additions you think need to make to this document, and why?

4. Results of consultation

There were 15 responses to the consultation;

- Five patients were received from patients
- Three responses were received from patient support groups
- One response was received from a not for profit provider
- One clinician responded
- One service provider responded
- One drug company, not the manufacturer, responded
- Three responses were received from carers/parents

5. How have consultation responses been considered?

Responses have been carefully considered and noted in line with the following categories:

- Level 1: Incorporated into draft document immediately to improve accuracy or clarity. There were no level 1 responses
- Level 2: Issue has already been considered by the CRG in its development and therefore draft document requires no further change. There were four level 2 responses
- Level 3: Could result in a more substantial change, requiring further consideration by the CRG in its work programme and as part of the next iteration of the document. There was one level 3 response which referred to diseases that are covered by another NHS England policy for this drug.
- Level 4: Falls outside of the scope of the specification and NHS England's direct commissioning responsibility. There were no level four responses.

All the responses to the policy were positive. The patients and carers and the patient groups commented that the drug made a very major positive impact on their lives. A small number of patients thought the policy should include more detail about the impact of periodic fevers on patients' quality of life, that with this treatment patients spent less time in hospital and so able to work and attend school. The tolerability of this treatment by children in comparison to anakinra, the current unlicensed treatment, was noted,

'Currently our routine varies from bad (on a good day) to terrible - our son is old enough to know the injection is coming but too young to understand why; this places a lot of a mental anguish on him (and also, less importantly, on us) and there is a grey cloud hanging over us all morning and until about 30mins after his injection once he has calmed down. I appreciate this consultation is focusing primarily on the science of the condition, but I truly believe there is something to be said for the mental / psychological relief it would surely allow.'

(Parent of four-year-old child with HIDS).

The PWG think that the impact of the disease on quality of life is appropriately represented in the policy proposition.

A desire to see treatment available locally was raised by some carers and a service provider queried the commissioning arrangement, raising concerns that patients in the Midlands would have to travel. The policy allows for other centres to prescribe following agreement with the one of the centres who are members of the European Reference Network (ERN), an arrangement which would mitigate this concern.

A drug company, which manufactures other drugs in the treatment pathway, queried the need to include canakinumab by name in the treatment pathway in section 9 of the policy proposition. The PWG think it is appropriate to include this in the proposition.

A small number of respondents queried why the policy proposition did not cover other extremely rare conditions and autoinflammatory diseases. The PWG consider that the proposition addresses those diseases for which there is sufficient evidence to support commissioning this treatment.

Two respondents were concerned that treatment was based on genetic diagnosis only, the PWG consider that the proposition recognises that diagnosing these conditions is complex and genetics is one element of this.

6. Has anything been changed in the policy as a result of the consultation?

There have been no changes to the policy proposition following this consultation.

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

The PWG consider that there are no unresolved concerns in the final policy proposition.