Consultation Report

Topic details

Title of policy or policy statement:	Metreleptin for congenital leptin deficiency (all ages)
Programme of Care:	Internal Medicine
Clinical Reference Group:	Specialised Endocrinology
URN:	170095P

1. Summary

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

2. Background

Leptin is a hormone which regulates appetite and body weight. Leptin also plays an important role in controlling blood sugar, immune control and hormone secretion. When the fat cells of the body are full, leptin is produced and signals the brain to stop eating. People with the extremely rare condition of congenital leptin deficiency are unable to make leptin and so are in a continual state of extreme hunger. This sensation of extreme hunger is overpowering. Affected individuals develop abnormal behaviour around eating, such as hiding food, secretiveness about eating and fighting over food. The condition is also associated with increased risk of infections due to impaired defence against infection; associated hormone abnormalities leads to absence of puberty. Complications of extreme obesity occur, including diabetes, sleep apnoea and bone problems.

High mortality in childhood and adolescence occurs in untreated individuals with the condition. No treatment is available which modifies the disease. Supportive care can be given but the sensation of hunger overpowers any attempt at dietary control. Without treatment, very few patients survive to adulthood.

Metreleptin is an artificial form of leptin. It replaces the hormone which is missing. Patients who have congenital leptin deficiency return to normal weight when treated with metreleptin. Treatment with metreleptin leads to either improvement or resolution of many of the comorbidities associated with obesity caused by congenital leptin deficiency.

Metreleptin was granted marketing authorisation in July 2018.There are currently seven patients from England being treated with metreleptin. All are on a compassionate use programme at Cambridge University Hospital.

3. Publication of consultation

The policy was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 17th August 2018 to 16th September 2018. Consultation 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 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 made to this document, and why?

4. Results of consultation

There were four responses to the public consultation, two clearly from clinicians, one from the specialised endocrinology Clinical Reference Group (CRG) and one from a non-profit professional. All responses were positive in answering every question in the consultation.

All respondents responded that:

- all the relevant evidence been taken into account in developing the policy proposition,
- that the impact assessment fairly reflect the likely activity, budget and service impact to be expected,
- that the policy proposition accurately describe the current patient pathway that patients experience,
- there was no potential impact on equality and health inequalities which might arise as a result of the proposed policy.

That there was no response from patient groups which was anticipated by the Policy Working Group because there is stigma associated with having an inherited disease. Also respondents were known to be concerned that a response would be identifiable.

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
- Level 2: Issue has already been considered by the CRG in its development and therefore draft document requires no further change
- 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
- Level 4: Falls outside of the scope of the specification and NHS England's direct commissioning responsibility

All responses were noted and none fell into the categories set out.

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

No

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

No