NICE National Institute for Health and Care Excellence

NHS England

Engagement Report for Specialised Commissioning Policies

Unique Reference Number and NICE ID	1698
Policy Title	Metreleptin Congenital Leptin Deficiency
Accountable Commissioner	Sarah Watson
Clinical Lead	Professor Sadaf Farooqi
Clinical Reference Group	Specialised Endocrinology
Which stakeholders were contacted to be involved in policy development?	Specialised Endocrinology CRG and registered stakeholders Paediatric Medicine CRG and registered stakeholders Patient Groups – there are very few affected patients and no relevant patient groups.
Identify the relevant Royal College or Professional Society to the policy and indicate how they have been involved	Na
Which stakeholders have actually been involved?	Internal Medicine CRG and registered stakeholders
Explain reason if there is any difference from previous question	Organisations declined the offer to participate in the development of the policy
Identify any particular stakeholder organisations that may be key to the policy development that you have approached that have yet to be engaged. Indicate why?	Na
How have stakeholders been	Policy working group meeting and subsequent contact for

involved? What engagement methods have been used?	policy development. Stakeholder engagement process. 14 day email engagement exercise with registered stakeholders.
`	Comments have been reviewed by policy working group and amendments made to documents where appropriate following consideration by the Policy Working Group. A number of comments were received from the Specialised Endocrinology CRG and the Paediatric Medicine CRG. All were positive and supportive of the routinely commissioned policy for metreleptin for congenital leptin deficiency. Two other submissions were made to the stakeholder consultation. One response was from NICE which asked for the papers to add a note about the ongoing NICE evaluation ID861 on metreleptin for generalised and partial lipodystrophy. This Highly Specialised Technology Appraisal is not for congenital leptin deficiency and so has no impact on the provisional policy proposal presented here. The expected publication date of HST ID861 is the 26 September 2018. Clarification has been added into the policy to confirm that a licensing application has been submitted in the EU by Aegerion for metreleptin to be used to treat complications of leptin deficiency in patients with congenital or acquired generalised lipodystrophy and in a subset of patients with partial lipodystrophy which also results in a lack of leptin. Aegerion Pharmaceuticals commented that the clinical implications of congenital leptin deficiency and therefore the benefits of treatment were understated. Some minor edits have been done in the Plain Language Summary section of the draft policy to more fully describe the impact of the condition on patients. One comment about repeating details of co-morbidities was felt to not necessitate a change to the policy. The last comment was about the longer term safety profile of metreleptin and a note was added into the policy on longer term safety.
How are stakeholders being kept informed of progress with policy development as a result of their input?	Stakeholders will be kept informed of the policy's progress through NHS England's consultation portal website. There has been some direct correspondence with Aegerion UK Ltd.

What level of wider public consultation is recommended by the CRG for the NPOC Board to agree as a result of stakeholder involvement?	Not all stakeholders made a recommendation. Those that did selected: 1 - changes that could reasonably be expected to be broadly supported by stakeholders - up to 6 week consultation

Appendix One – Stakeholder/CRG Feedback

Organisation Responding	Feedback Received	PWG response	Resulting Action
NICE	We think that there may be some added benefit in adding more clarity within the policy proposition to describe how this differs from the evaluation that NICE is currently completing - [ID861] Metreleptin for treating lipodystrophy. This should help to provide clarity to those commenting so that they understand the remit of the NICE evaluation and what the policy intends to cover.	Agreed in part. Clarification on the licensing added to the policy but not specifically noting the NICE work on lipodystrophy	Clarification added at about the new treatment that it is a different condition that also results in leptin deficiency and therefore where metreleptin is effective
Neale Harris Aegerion UK Ltd	Plain Language Summary The section starts off stating that Leptin is a hormone which regulates appetite and body weight. Leptin also plays an important role in glycaemic and lipid regulation, immune control and hormone secretion. The implication of CLD are understated in the plain English summary with a wider range of implications detailed in the main document, it would be good to have these reflected in the plain English section. In the 'About the new treatment' the benefit of treatment is also understated commenting that the patient returns to normal weight, there are a number of significant improvements seen as a result of treatment that are reflected later in the document for example at the end of page 9, inclusion of these would give a fairer understanding of the benefit of the new treatment.	Agreed Agreed Agreed	Amended plain language summary Change made to the Plain English section Added a sentence into the benefit of treatment section

	Proposed Interventions and Clinical Indication At the bottom of page 6 in the document the author talks about; 'Successful treatment leads to significant weight loss (Paz Filho 2011) and reversal of certain comorbidities.' It would be helpful to detail the nature of the comorbidities as detailed later in the document. Evidence Base End of page 9, the author refers to 'Longest follow up is less than five years' and yet at the start of page 8 in the same section the author refers to a follow up period of ten years, and indeed UK experience of patient treatment is over 20 years long.	PWG disagreed and didn't think that there was a need to repeat this information in both sections of the policy Agreed	No action Comment on longer follow up data added