NICE National Institute for Health and Care Excellence

NHS England

# Engagement Report for Specialised Commissioning Policies

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| **Unique Reference Number and NICE ID** | 170095P |
| **Policy Title** | Metreleptin Congenital Leptin Deficiency |
| **Accountable Commissioner** | [redacted] |
| **Clinical Lead** | [redacted] |
| **Clinical Reference Group** | Specialised Endocrinology |
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| **Which stakeholders were contacted to be involved in policy development?** | Specialised Endocrinology CRG and registered stakeholders<br>Paediatric Medicine CRG and registered stakeholders<br>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** | N/a |
| **Which stakeholders have actually been involved?** | Internal Medicine CRG and registered stakeholders |
| **Explain reason if there is any difference from previous question** | Stakeholder 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?** | N/a |

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| **How have stakeholders been involved? What engagement methods have been used?** | Policy working group meeting and subsequent contact for policy development.<br>Stakeholder engagement process. 14 day email engagement exercise with registered stakeholders. |
| **What has happened or changed as a result of their input?** | Comments have been reviewed by policy working group and amendments made to documents where appropriate following consideration by the Policy Working Group.<br>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.<br>Two other submissions were made to the stakeholder consultation.<br><br>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.<br><br>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.<br><br>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. |

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| | 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.<br>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:<br><br>1 - changes that could reasonably be expected to be broadly supported by stakeholders - up to 6 week consultation |