NICE National Institute for Health and Care Excellence

NHS England

Engagement Report for Specialised Commissioning Policies

Unique Reference Number and NICE ID	1810 ID011
Policy Title	Idebenone for treating Leber hereditary optic neuropathy
Accountable Commissioner	Nicola Symes
Clinical Lead	Fion Bremner
Clinical Reference Group	Specialised Ear and Ophthalmology Clinical Reference Group
Which stakeholders were contacted to be involved in policy development?	A policy working group was established in line with NHS England's standard methods. The draft policy proposition was sent to the following groups for comment: • Specialised Ear and Ophthalmology Clinical Reference Group registered stakeholders
Identify the relevant Royal College or Professional Society to the policy and indicate how they have been involved	All of the relevant Royal Colleges and professional societies were invited to take part in stakeholder testing.
Which stakeholders have actually been involved?	7 responses were received from stakeholders, including 1 individual clinician. Santhera LHON Society Fight for Sight The Lily Foundation Royal College of Ophthalmologists Royal National Institute of Blind People (RNIB)

Explain reason if there is any difference from previous question	Not all organisations commented on the documents.
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?	None, the main patient and carer representative organisations were involved throughout the development of the draft policy proposition
How have stakeholders been involved? What engagement methods have been used?	Policy working group meeting and subsequent contact for policy development The draft policy proposition was distributed to stakeholders via email for a period of two weeks of stakeholder testing, in preparation for public consultation. Stakeholders were asked to submit their responses via email, using a standard response and in line with NHS England's standard processes for developing clinical commissioning policies.
What has happened or changed as a result of their input?	During the development of the policy, the policy working group expressed concern at the decision made by Clinical Panel to change the policy to not for routine commissioning. The PWG sent a letter of concern to NHS England. Comments were submitted by 7 stakeholders and these have been reviewed by the policy working group. Comments included: • A statement that using the pivotal trial as the main evidence base for the policy was not appropriate, because the trial was flawed. No change will be made because this was the main study for the drug (the European public assessment report notes that the RHODOS trial was considered the "main support for efficacy" for idebenone in this indication), plus data from all appropriate sources was fully explored. • A suggestion that the decision was based on economic reasons not clinical. No change will be made as at this stage decision making is based on clinical evidence only

	• A request for a minor amendment to text as follows, which we have agreed: Statistical significance was also reached in patients with a disease duration ≥1 year, but there was no significance between-treatment difference for disease duration <1 year A managed access agreement was suggested by one of the respondents and the following response has been provided: Managed Access Agreements (MAA) are being used for treatments that NICE appraises and there is not enough evidence to support a positive recommendation by NICE but the treatment is expected to be very promising. Managed Access Agreements are complex to set up and are expensive for the NHS to operate due to the administration and infrastructure around them to support patient follow up and data collection. MAAs are being used within the context of a NICE Highly Specialised Technology appraisals where the number of patients and centres offering treatment are small. They can also be offered within the context of the Cancer Drugs Fund where the infrastructure and data systems are already set up to support data collection only for cancer treatments. MAAs also require the agreement of the company to offer the treatment at a cost-effective price for the duration of the MAA and post the MAA reappraisal.
How are stakeholders being kept informed of progress with policy development as a result of their input?	All stakeholders (including CRG members and registered stakeholders) will be notified when the draft policy proposition goes out to public consultation and will be kept informed of the policy's progress through NHS England's consultation portal website
What level of wider public consultation is recommended by the CRG for the NPOC Board to agree as a result of stakeholder involvement?	It is proposed that highly specialised products will go for period of public consultation for four weeks