Consultation Report

Topic details

Title of policy or policy statement:	Idebenone for the treatment of Leber's Hereditary Optic Neuropathy (in patients over 12 years of age)
Programme of Care:	Trauma
Clinical Reference Group:	Specialised Ophthalmology
URN:	1810

1. Summary

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

2. Background

Leber's hereditary optic neuropathy (LHON) is a rare maternally-inherited genetic disease that causes visual impairment. LHON usually starts with a painless blurring of central vision in one or both eyes, usually followed by progression to blindness. Progression can be very rapid (over a period of months), or it may take longer to progress (over a number of years); but in around 97% of people with LHON, both eyes will be affected within 1 year of diagnosis (Meyerson, 2015).

Current treatment for people with LHON is limited to best supportive care (i.e. it is focused on relieving the symptoms caused by the condition, without actively treating it). This includes regular neuro-ophthalmology outpatient appointments, referral to low-vision services, lifestyle advice and/or genetic counselling. Idebenone is licensed to treat visual impairment in adolescent and adult patients with LHON.

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 23 July to 22 August 2019. Consultation comments have then been shared with the Policy Working Group (PWG) to enable full consideration of feedback and to support a decision as to 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 be made to this document, and if so, why?

4. Results of consultation

A total of 20 responses were received.

Summary statistics in relation to whether the responses were positive/negative are shown in Table 1 (below)

Table 1	**Responses by type of Respondent** (Numbers relate to Yes / No / nil response)			
Question	Clinician	Professional Association / Organisation	Patient or Representative	Other (non-profit professional, Pharma Co)
Did the respondent think that all the relevant evidence had been taken into account?	4 / 0 / 0	3 / 2 / 0	5 / 4 / 0	1 / 1 / 0
Did the respondent think that the impact assessment fairly reflects the likely activity, budget and service impact?	4 / 0 / 0	2 / 3 / 0	3 / 6 / 0	1 / 1 / 0
Did the respondent think that the policy proposition accurately describes the current patient pathway that patients experience?	3 / 1 / 0	2 / 3 / 0	2 / 7 / 0	2 / 0 / 0
Did the respondent provide any comments about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?	1 / 1 / 2	3 / 2 / 0	7 / 0 / 2	1 / 0 / 1
Did the respondent recommend any changes or additions to the policy proposition?	2 / 1 / 1	3 / 2 / 0	1 / 4 / 4	1 / 0 / 1

Common themes in relation to the comments received are listed below:

- Over half of all respondents felt that the proposal to not routinely commission idebenone for the treatment of this condition would result in health inequalities, because of the fact that it is available in other European countries, including Scotland and Ireland. Some made the point that those who could afford to, would be able to pay for treatment. These issues are detailed in the equalities impact assessment.
- Those who recommended changes to the policy asked for the decision to be reversed so that treatment for LHON with idebenone is routinely commissioned by NHS

England. Some stated that as this was the only treatment option for these patients then it should be commissioned as anything that may have benefit would be beneficial to patients.

- Whilst a number of the respondents acknowledged that there was a lack of evidence to demonstrate that idebenone is effective for the treatment of LHON, some felt that this was outweighed by the fact that there is currently no available alternative (other than best supportive care) and that any degree of benefit that might be gained from treatment (however small) would result in improved quality of life for patients.
- Some respondents suggested that more research should be carried out.

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 policy and NHS England's direct commissioning responsibility

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

Following feedback from the public consultation, wording under the section 'about current treatments' has been reviewed and amended. New wording is set out below:

About current treatments

There are no treatments currently available to treat LHON, therefore patients are only able to access supportive care. This is likely to broadly consist of (a) low vision aids, (b) registration as visually impaired, (c) genetic counselling (including reproductive options) and (d) follow-up visits, but the details may vary from centre to centre and from patient to patient.

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

The public consultation highlighted concern in relation to the decision not to routinely commission this treatment in England, when it has been made available elsewhere in Europe including Scotland. Concerns were raised in relation to the fact that this might lead to inequalities amongst those living with the condition, in that patients who could afford to, may seek treatment privately, or try to purchase the treatment via the internet through unregulated routes.

The PoC gave due consideration to the feedback from public consultation and noted the fact that no new evidence had been highlighted as a result of the consultation process. The PoC concluded that the recommendation of Clinical Panel was based on there being insufficient clinical evidence to support routine commissioning of the treatment and is supportive of this position.