HTA Done Properly - The Scottish Experiment

Prior to 2016 I attended EURORDIS Summer school (2012) EuNetHTA training (2014) EUPATI training (2015-2016) as well as involvement in the steering group for ISPOR's Special Interest Group for Patient Involvement in Research.

So it is fair to say that I had a fair amount of exposure to HTA and, recognising its likely importance to the rare disease community, I read extensively into the subject. With a background in banking and finance, this came relatively easy to me.

BUT, it was all desk based, book (or screen) based learning. I had no direct hands-on experience of an aspect of healthcare that seemed likely to me to be one of the most important, yet one of the most neglected.

Early November 2016 I attended the ISPOR* patient roundtable, at which a leading HTA practitioner was asked if, in his experience, he had ever encountered a patient testimony that made a difference to the outcome of an assessment. He replied emphatically "No!"

**International Society for Pharmaco-economics and Outcomes Research.*

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By sheer coincidence our society received the following email just a few weeks later:

I am writing to let you know that **idebenone (Raxone)** will shortly be assessed by the Scottish Medicines Consortium (SMC) for use in NHS Scotland, and to invite you to make a Patient Group Submission for the medicine. The submission deadline is **6 February 2017**.

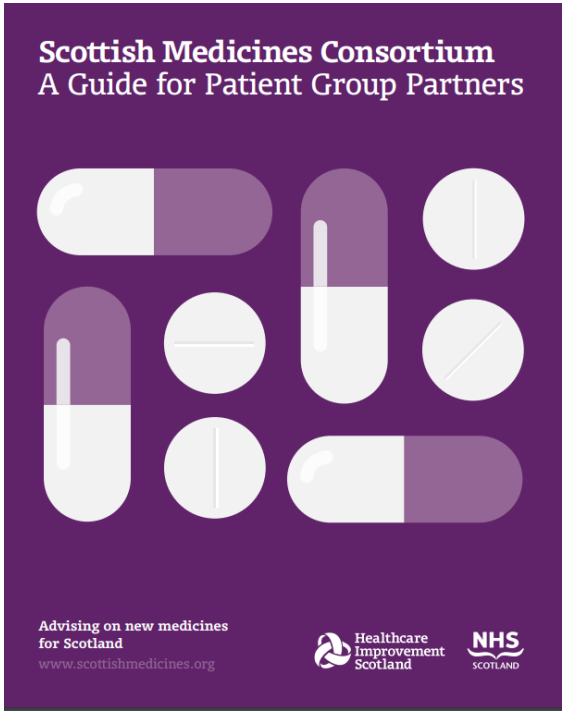
The pharmaceutical company, Santhera Pharmaceuticals, has also provided the attached Summary of Information for Patients document which contains additional information about the medicine. While it may help patient groups with their own patient group submission, **the information should not be copied and pasted from this document into patient group submissions.**

It is very important that we capture the impact of this condition and the potential benefit this medicine has to both patients and carers. I would like to invite The LHON Society to make a Patient Group Submission for the medicine, as the wealth of knowledge and experience your organisation could provide would help our SMC Committee members to make a fully informed decision on whether they can make this medicine available on the NHS to patients in Scotland.

The process for patient groups making a submission was developed by SMC in partnership with patient groups. We hope this is a relatively straightforward way for patient groups to capture patient and carer views and experiences. To submit you need to complete a Patient Group Submission Form

In your submission, it is very important to try to include quotes or statements from patients and/or carers - these can be very powerful and add valuable impact to the overall submission.

HTA Done Properly - The Scottish Experience



The invitation included links to the SMC website **https://www.scottishmedicines.org.uk/Public_Involvement** which included further links to videos and publications explaining the process and offering guidance for making an effective submission. These included a 20 page downloadable pdf guide and an example form made by another patient group.

The submission document itself is an online form of 11 pages and very clear to complete.

SMC staff were completely supportive throughout the submission process, although the process was so straightforward their help was not really needed.

HTA Done Properly - The Scottish Experience

With only 2 months to prepare our submission (and Christmas in the middle) we had to move quite quickly.

We agreed with SMC that for a small society with only c12 members in Scotland it was unrealistic to survey only Scottish members and so we surveyed all UK members.

We prepared a short online survey (which needed to be screen-readable so our VI members could respond unaided) and received a total of 34 responses, the majority of which were from members who had vision loss, with a few responses from family members and carers (and carriers of the condition who are as yet unaffected).

More surprisingly, a large percentage of those responding had prior experience of being treated with the drug which was the subject of the assessment.

Some very harrowing tales of hardship and marriage breakdowns etc which formed a core part of our submission to the SMC, using some powerful direct quotations.

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Upon submitting our presentation to SMC we were advised the following:

As the medicine **idebenone (Raxone)** is designated orphan / ultra orphan, it may go to a PACE (Patient and Clinician Engagement) meeting. The PACE meeting, which is attended by patient group representatives and, if they wish, a patient advocate, together with clinicians, and SMC staff, will discuss the added value of the medicine, prior to the medicine being discussed by the SMC Committee. You can read more about our PACE process here.

The PACE meeting for this medicine is provisionally scheduled to take place on **14 March 2017** in our offices in Glasgow. The meeting lasts for one hour and representatives can attend in person or can join by teleconference.

The output of the PACE meeting is a template document which then forms part of the papers for discussion at the SMC Committee meeting on **4 April 2017**.

As a submitting patient group, you will be invited to attend the PACE meeting

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The PACE meeting can be requested by the sponsor in circumstances where it appears that a negative outcome to the process might be anticipated.

It was impractical for me to attend the PACE meeting in person and so, along with some of the participating clinicians, I attended the meeting via teleconference from my home.

Approximately 12 participants attended the meeting, including SMC staff and clinicians from within Scotland and from elsewhere in the UK (with particular expertise in the condition). Clinicians included ophthalmologists and geneticists. The sponsor is not invited to this meeting.

I was the only patient representative at the meeting. I was given sufficient time to make all the points I wanted to make and, if anything, I received (or took) far more than my fair share of speaking time (probably nearly 50% of the meeting).

Our submissions to the meeting extended beyond what might normally be expected from a patient submission, including economic and efficacy issues that might otherwise not have been considered

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A report was compiled by SMC to reflect the content and recommendations of the meeting and this was circulated to attendees to facilitate any clarification or correction.

Although the PACE meeting discussions remain confidential, the report is made available for discussion at the SMC committee meeting to discuss the assessment and this meeting is held in public.

However the decision as to whether or not to accept the recommendations is taken in private and not communicated until some weeks later. The result is provided in confidence to the sponsor a week ahead of publication and to the patient group a few days ahead of publication in order to allow for any public statements to be prepared.

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Following participation of patients, a likely negative decision became positive

Advice

following a full submission assessed under the ultra-orphan medicine process:

idebenone (Raxone®) is accepted for restricted use within NHS Scotland.

Indication under review: Treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic Neuropathy (LHON).

SMC restriction: to patients with LHON who are not yet blind i.e. they do not meet the UK criteria to be registered as severely sight impaired.

In a 24-week double-masked randomised placebo-controlled study, patients who received idebenone had numerical improvements in visual acuity over placebo.

This SMC advice takes account of the benefits of a Patient Access Scheme (PAS) that improves the cost-effectiveness of idebenone. This advice is contingent upon the continuing availability of the PAS in NHS Scotland or a list price that is equivalent or lower.

This advice takes account of views from a Patient and Clinician Engagement (PACE) meeting.

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Key lessons to be learned:

1. The importance of a fair and transparent process cannot be over-stated. SMC have clearly worked hard to incorporate patients views in developing this process and the process itself is continuing to evolve, with further enhancements to the process under discussion and consultation.

2. Patients have far more to contribute than just the “Lived experience” of the condition under assessment, although this is also key to success.

3. A balanced and realistic approach is more likely to prove fruitful. Recognise what might be achieved and marshall your arguments toward those goals.

4. While the process itself is important, the human element is critical. SMC staff worked tirelessly to make this process work and proved flexible and understanding in dealing with queries and allowing us to make points that might not normally be expected from patient groups as well as those that would.