Patient Involvement in HTA: An example of How and Why

26 September 2017
Background

• A pharma company launching a new drug commissioned an HTA evaluation to an Academic Institution in Italy

• Patient Experts were asked to give their evaluation of the drug and contribute their views

• The Pharma company decided to implement the “NICE Model”: have Patient Experts from the specific disease area and a Patient Expert from a different disease area
What were the different steps?

1 - We, as patients, were given the full details of the clinical trial and were asked to provide our comments/input.

2 - We were also asked to review a patient survey that the pharma company had prepared to argue their case in favour of their new drug. It was particularly interesting to see which data was used and how the pharma company argued its case.

3 - Our comments/input were included in the HTA evaluation as a relevant part of the dossier.
How were patients involved?

• Pharma decided to follow the NICE Model and selected 3 patient representatives from the specific disease area + a Patient Expert from a different disease area

• Representatives of the relevant Patient Associations were asked to participate because of their experience of the disease, the additional Patient Expert was selected because of proven expertise and because of the relation of trust established over time
Why is it important to be involved?

- To get pharma/relevant stakeholders to listen to patients and involve them in a consistent way

- To show that patients are well trained and knowledgeable

- To establish trust with pharma and other relevant stakeholders

- You can help decision makers understand how important a new drug can be for you and your fellow patients
Lessons to be learned?

• If pharma companies know that you have sound knowledge and add value to their proposition they will seek your expertise

• It doesn’t matter if you don’t have previous experience of HTA, if they ask you to get involved it’s because your input is valuable

• Always get involved if they offer you the chance, even if you don’t feel you know enough about the subject. There is plenty of information available to gain extra knowledge and free online courses to follow, just don’t miss out on opportunities!
Patient Involvement in HTA- When the maths don’t add up.

Joan Jordan EUPATI Graduate
EUPATI Webinar
26 Sept 2017
RRMS and PPMS

- In Relapsing Remitting Multiple Sclerosis (RRMS), people have distinct attacks of symptoms which then fade away either partially or completely.
- Around 85 per cent of people with MS are diagnosed with this type.
- There are 14 Disease Modifying Therapies (DMTs) currently available to treat RRMS.
  My DMT is paid for by the Irish Government and the ICER for it exceeds €45,000 per Quality Adjusted Life Year.
- For most people with MS, this is the way their MS begins, except for the small group of people who have Primary Progressive MS (PPMS). This is about 15 per cent of all people with MS.

There are currently NO DMTs for PPMS!
A new drug has been developed which can slow the progression of MS, according to the results of its phase 3 trials. It is the first treatment that can slow the advancement of primary progressive MS. It can also treat relapsing MS. The drug is an intravenous infusion treatment that has been developed by Roche. In March 2017 it was approved by the US Food and Drug Administration (FDA) as a treatment for both relapsing and primary progressive MS. It is being reviewed the European Medicines Agency (EMA), and a decision is expected in autumn 2017.

**Current phase of trial:** Under review by EMA

**Type of MS:** primary progressive and relapsing remitting MS
Adapted From MS Society UK contd.

When is the drug likely to become available?

In July 2016 Roche submitted a licensing application to the European Medicines Agency (EMA), the body responsible for licensing drugs in Europe. The EMA has accepted this application and is currently reviewing the data.

We expect a decision to be announced in late 2017. In March 2017 the drug was approved by the US Food and Drug Administration (FDA) as a treatment for both relapsing and primary progressive MS.
Using Projected HTA Calculations

- It is possible that the drug will be approved as cost effective for RRMS in Ireland but not for PPMS.
- This is because for PPMS, the comparison is made to Best Supportive Care.
What can MS Ireland do about this?
Other strategies

- MS Ireland does not have a Patient Registry, so surveys are being used to gather data, rather than relying on anecdotal evidence.
- Work with Patient Organisations, IPPOSI, Roche and NCPE to become familiar with the current process. Know my ICERs from my QALYs.
- Call for the development of a new sustainable national strategy on access to new and innovative drug therapies in Ireland involving all key stakeholders, including patient groups and the public.
- Collaborate with MS Societies from other countries to gather data.
- Apply my EUPATI experience of HTA theory to navigate the process in an Irish context.
- Refresh my HTA knowledge using free Futurelearn online course from University of Glasgow [https://www.futurelearn.com/courses/hta](https://www.futurelearn.com/courses/hta)
- Saving to buy Karen Facey’s book on Patient Involvement in Health Technology Assessment ISBN 9811040672, 9789811040672
WISH TO

Web: www.eupati.eu

Twitter: @eupatients

as well as:
Public Policy Manager

Colm Fahey
New Medicines – Assessment Process

Stage 1: Rapid Review Process
- Rapid Review Dossier Submitted
- Rapid Review undertaken by NCPE
- Price Application Submitted to HSE (once licence is granted)
  - HSE will consider outcome of Rapid Review & other criteria
    - Positive decision to reimburse at applied terms
    - Further engagement with company required
    - Formal HTA required

Stage 2: HTA Process
- HTA undertaken by NCPE
- HSE will consider outcome of HTA & other criteria
  - Positive decision to reimburse at applied terms
  - If decision to reimburse is made, HSE will implement reimbursement within 45 days
- Further engagement with company required
  - Drug considered by HSE Drugs Group
  - Decision considered by HSE following recommendation by Drugs Group
- Negative Decision taken by HSE

Decisions subject to overall HSE resources as allocated by the Dail

EUPATI
European Patients’ Academy
on Therapeutic Innovation
Increasing Timelines

- 2007
- 2009
- 2012
- 2015

3 Months 6 Months 10 Months >18 Months
Erivedge
Industry Wide Oncology Data

2014-2016

- UK: 22
- AUSTRIA: 22
- GERMANY: 22
- SWEDEN: 21
- DENMARK: 20
- NETHERLANDS: 18
- FRANCE: 17
- PORTUGAL: 17
- NORWAY: 17
- SWITZERLAND: 16
- FINLAND: 14
- SPAIN: 13
- ITALY: 12
- BELGIUM: 10
- IRELAND: 10
The **rate of availability**, measured by the number of medicines available to patients in European countries as of 2016: for most countries this is the point at which the product gains access to the reimbursement list.
Vismodegib (Erivedge®) is indicated for the treatment of adult patients with locally advanced basal cell carcinoma inappropriate for surgery or radiotherapy or metastatic basal cell carcinoma.

**Rapid Review**

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**Pharmacoeconomic Evaluation**

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**Summary**
Lumacaftor/ivacaftor (Orkambi®)

Orkambi® is indicated for the treatment of cystic fibrosis (CF) in patients aged 12 years and older who are homozygous for the F508del mutation in the CFTR gene.

Rapid Review

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Pharmacoeconomic Evaluation

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<td>01/06/2016</td>
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"The manufacturer Vertex Pharmaceuticals have confirmed that patients currently receiving Orkambi under the Managed Access Scheme will continue to receive the drug until reimbursement is finalised in Ireland"
Deal on 'miracle' cystic fibrosis drug expected in a matter of weeks

Health Minister Simon Harris said that he wants a good deal for the taxpayer and, most importantly, for patients.

A DEAL TO make the expensive cystic fibrosis drug Orkambi available in Ireland is expected within a few weeks, according to Health Minister Simon Harris.

“We are going to try to bring certainty to this issue within weeks,” Harris said today when speaking about the process of trying to get a good deal on the drug for taxpayers and patients.

On the same show, Harris ruled himself out of the Fine Gael leadership race.

With about 50 new cases of cystic fibrosis diagnosed in Ireland every year, campaigners are extremely eager for Orkambi – which has been heralded by them as a life-saving, ‘miracle’ drug – to be made available.

Cystic fibrosis greatly impacts the lifespan of those with the debilitating condition.

CF campaigner Jillian McNulty, who is on a trial of the drug, recently told TheJournal.ie that it has transformed her life.

Costly

But the huge cost of Orkambi – €158,000 per year per patient, at a cost to the State of €390 million over the course of five years – has led to protracted negotiations.

The National Centre for Pharmacoeconomics (NCPE), which is the country’s medicines watchdog, has said that Orkambi was not cost-effective, and advised against buying it.

As Health Minister, Simon Harris has been heavily involved in the negotiations with Vertex.
Irish patients denied vital medicines by slow drug approval

- Patient's cancer in Ireland is unable to access the treatment
- HSE had sought additional funding for drugs

Alice Merrigan, who was diagnosed with metastatic melanoma in 2014, did not get the treatment as the cancer had spread to her brain, heart, lungs, and liver. She was put on drugs called Nivolumab or 'Nivo'.

Her cancer disappeared. 'This treatment gave me back my life,' said Merrigan, who added that there was a period when she didn't think she would ever make it to her wedding day.
Patients fighting cancer denied top treatment as HSE out of cash

Noel O'Connor and Eileen O'Shea

Recordings from the National Cancer Registry of Ireland show that patients on the waiting list for top cancer treatments are facing delays and cancellations due to a lack of funding from the HSE.

Nuala O'Connor, whose husband has been diagnosed with a rare form of cancer, told RTÉ Radio 1 that she is worried about delays in treatment.

"It's a very stressful time for everyone, and it's not fair that some patients are getting the treatment they need, while others are waiting," she said.

The HSE has acknowledged that funding for cancer treatments is a priority, but has struggled to meet its targets due to a shortfall in funds.

A spokesperson for the HSE said that the organization is working closely with the Department of Health to find solutions to the problem.

"We are doing everything we can to ensure that patients have access to the care they need," the spokesperson said.

HSE seeks €120m for new drugs

The government is expected to allocate €120m to the Health Service Executive (HSE) for the purchase of new cancer drugs.

The HSE has been under pressure to increase the amount of funding allocated to cancer research, with some patient groups calling for a doubling of current spending.

The government has previously committed to increasing spending on cancer research, but has struggled to deliver on its promises due to budget constraints.

Minister for Health Simon Harris has said that the €120m will be used to fund new cancer drugs and research initiatives.

"This investment will not only benefit patients, but will also help to drive forward research in the fight against cancer," he said.

The move is expected to be welcomed by patient groups, who have long called for more funding to be allocated to cancer research.

Dept of Health drags heels on medicine funding requests

The Department of Health has been slow to respond to requests for funding for new medicines.

A recent report from the Health Research Board found that some patients were waiting up to two years for new treatments to be approved.

The report highlighted the need for urgent action to ensure that patients have access to the latest medicines.

Minister Harris has said that the government is committed to increasing funding for new medicines, but has not yet announced any specific plans.

"We need to ensure that patients have access to the latest treatments, and this requires significant investment," he said.

The Department of Health has been criticized for its slow response to requests for funding.

A patient group has called on the government to take urgent action to address the issue.

"Patients are being denied access to life-saving treatments and we need to see action," said a spokesperson for the group.

The government has said that it is committed to ensuring that patients have access to the latest treatments, but has struggled to deliver on its promises due to budget constraints.

Minister Harris has said that the government will announce plans for increased spending on new medicines in the upcoming budget.

The move is expected to be welcomed by patient groups, who have long called for more funding to be allocated to cancer research.
Doing now what patients need next