

Advocating for Reimbursement

The role of Advocates in
Health Technology Assessments

Advocating for Reimbursement

- What are Health Technology Assessments (HTAs)?
- What can Advocates do to improve the outcome of HTAs for patients?
- Overview of one HTA in the UK

What are HTAs?

- A **Health Technology Assessment** is a process for deciding whether a new drug, device or method of treatment represents value for money. In this context, it is separate from the process of deciding if the drug is safe
- Central to the process is the concept of the **Quality-Adjusted Life Year (QALY)**. There is much debate about the methods used to calculate QALYs, but the principle is unlikely to change
- **A new drug has to be proven to extend life, to improve the quality of life, or both.**
The QALY principle is a recognition of the difference between a year of perfect health and a year of partially improved life quality
- HTAs are necessary so that different proposals can be compared and **judgements made in a consistent and systematic way**

The Role of Advocates in HTAs

- The HTA process is similar in principle everywhere but different in implementation, especially in how input from patients is treated
- The most important thing that patient representatives do in HTAs is to explain **how the proposed drug improves quality of life (QoL)**:
 - **All the ways** that QoL is improved (symptom reduction, psychological benefit, economic impact ... not only to the individual patient but to the carer(s) and the wider community
 - The **magnitude** of these benefits – explaining how much the disease reduces quality of life and how much benefit the new drug delivers
- **Individual patient stories** have a lot of impact with assessors
- **Patient organisations** demonstrate the wider benefits of the proposal

Ruxolitinib in the UK

- Ruxolitinib has been assessed in the UK for treating Myelofibrosis Patients by NICE (NICE is responsible for HTAs for most of the UK)
- Patients were represented in this HTA by:
 - **Individual patients**
 - **Patient Organisations:** Leukaemia Care and MPN Voice
- Written submissions were provided in the form of responses to a standard set of questions from NICE plus a more general summary statement
- Individual patients and the patient organisations presented their submissions at HTA meetings

Ruxolitinib in the UK

The documents relating to this HTA are publicly available:

<https://www.nice.org.uk/guidance/TA386/documents/committee-papers-2>

1. Brief description of the organisation (aims, scope, funding, etc.)
2. What is it like to live with the condition?
3. Current practice in treating the condition
 - Which treatment outcomes are important to patients or carers?
 - What is your organisation's experience of currently available NHS care and of specific treatments for the condition? How acceptable are these treatments and which are preferred and why?
4. What do patients or carers consider to be the advantages of the treatment being appraised?
 - Please explain any advantages that patients or carers think this treatment has over other NHS treatments in England.
(see next page)
5. What do patients and/or carers consider to be the disadvantages of the treatment being appraised?
- ...
6. Key messages

- In no more than 5 bullet points, please summarise the key messages of your submission.

Ruxolitinib in the UK

- Specific aspects of the proposal's expected benefits:

Benefits of a treatment might include its effect on:

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- 1. the course and/or outcome of the condition
- 2. physical symptoms
- 3. pain
- 4. level of disability
- 5. mental health
- 6. quality of life (such as lifestyle and work)
- 7. other people (for example, family, friends and employers)
- 8. ease of use (for example, tablets rather than injection)
- 9. where the treatment has to be used (for example, at home rather than in hospital)
- 10. any other issues not listed above

In conclusion

- The HTA process is similar in principle in most countries
- Patient involvement varies widely
- Patient Advocates (both individually and as representatives of patient groups) can be provide a strong and well articulated patient voice in HTAs
- MPN Patient Advocates should pool their HTA experience and QoL evidence in order to maximise their effectiveness

Further reading

- **Guide to the single technology appraisal process – NICE**
<https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-technology-appraisals/Guide-to-the-single-technology-appraisal-process.pdf>
- **Health Technology Assessment – EUPATI**
<https://www.eupati.eu/category/health-technology-assessment>
- **Ruxolitinib for treating disease-related splenomegaly or symptoms in adults with myelofibrosis – NICE**
<https://www.nice.org.uk/guidance/ta386/evidence>