



Health Equality
Europe

Understanding Health Technology Assessment (HTA)



This guide describes how patients and the public can get involved in decisions about what healthcare should be available. It can also be used to help raise awareness of patient needs.

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Note: Some HTA agencies may wish to adapt this guide for their own use. Agency or country specific information can supplement or be substituted for the original text in the following sections: The assessment process; When to become involved; How to become involved; How to provide patient evidence. The Appendices may be revised or deleted to suit local circumstances. Anyone adapting this toolkit is asked to acknowledge the original source

FOREWORD

The call to focus health care decisions on the needs of patients often seems overshadowed by the requirement to overcome financial pressures and obtain efficiency. The question is, can we achieve value for money in a health service system, whilst taking account of the needs and preferences of patients?

The answer is yes, but only through transparent, systematic processes to which all stakeholders can contribute and in which all evidence is judged fairly and robustly. Such a process already exists in the form of health technology assessment (HTA), but HTA is complex, varies across different health systems and does not consistently include all stakeholders.

In all cases, HTA is an 'evidence-based' process that seeks to examine the consequences of using a healthcare technology by considering the medical, social, economic and ethical issues. So what evidence can patients provide?

Patients have something to say about all aspects of an HTA, but the most important thing they can contribute is a description of the benefits or unwanted effects of a healthcare technology. No one else can explain the impact of a disease or a healthcare technology, better than a patient. However, it's not easy to understand how to put these important views, needs and preferences into a form of evidence that can be used by a HTA organisation. That is why this guide, Understanding HTA, is so valuable.

The guide is written for patient organisations who want to represent the views of patients, but who are struggling to understand what HTA is and how they can best contribute. It outlines typical HTA processes and provides detailed examples of how to contribute to questions about the burden of illness, the effectiveness of the healthcare technology and the impact on carers. It explains how to collect information about patients' experiences and how to present written information to ensure that messages are clear. The advice on how to present evidence in person, provides tips that would help us all!

It is only by working in partnership and ensuring effective involvement of patients, carers and the public that we will be able to estimate the long-term consequences of using a health technology and judge its true value. So I am pleased to commend this clear, practical guide about HTA to you all. Not just patients, carers and the public, but importantly to all those who undertake HTA and are seeking ways of improving their involvement.

Karen Facey

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Health Equality Europe (HEE) is an informal coalition of people who wish to see the patient voice placed firmly at the heart of healthcare decisions within Europe. HEE brings together people from a number of countries with a range of expertise united by a commitment to making the patient voice heard. This guide is intended to contribute to raising the patient voice in Europe. HEE was established with sponsorship from Novartis International AG, Switzerland.

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1 Purpose of the guide

This practical guide describes the contribution that patients, caregivers and the public can make to decisions about improving healthcare, based on gathering information about the experience of living with an illness and about the impact technologies have on Peoples' lives. The aim of the guide is to help patients, caregivers and the public to make their case effectively during health technology assessment (HTA) processes. **Its primary audience is likely to be patient groups who see their role as patient advocates.**

Following the advice in this guide does not ensure that the technology will be approved but it may help ensure your voice is heard. The HTA process can lead to technologies being turned down or not recommended.

Specifically, the guide aims to:

- Improve understanding of the HTA process, including the terminology used
- Increase the skills of non-professionals contributing to the HTA process
- Provide guidance for patient group and public contributions to the HTA process

In some of the countries where HTA is used to support healthcare decisions, patients and the public are invited to be part of the assessment process. For example, the Health Technology Assessment Agency for Andalusia (Spain) has a section on its website inviting citizens to give their opinion about health technologies and to suggest topics of interest.

As the HTA process varies from country to country – and even within countries – if you wish to be involved please ask the agency undertaking the HTA for advice about how you can contribute; the agency will know what they need as patient evidence.

Patient groups that have participated in an HTA have found it can be challenging and can involve considerable resources, especially time. This practical guide can help patients and the public participate in HTAs, but understanding what is involved is important. Talking to groups who have already participated can clarify how much work is involved. **Two case studies from patient groups who have experience**

of HTAs are included at Appendix 1. Probably the most experience of patient and public involvement has been gained with the National Institute for Health and Clinical Excellence (NICE) in England, and the NICE website (www.nice.org.uk) provides the names of groups that have contributed to each technology appraisal. [To find the names of groups that have contributed, on the NICE website (www.nice.org.uk) go to *NICE guidance by type* or *NICE guidance by topic* to find the list of stakeholders for individual appraisals.]

This guide may also be useful for patients and the public living in countries where HTA processes are not well developed. The sort of experience-based information that patients and the public contribute to HTA processes can be useful in raising public awareness about the impact of an illness and the need for effective prevention and treatment. It can help make the case for better treatment in a variety of settings such as media campaigns and lobbying, or in individual consultations with doctors.

2 Health Technology Assessment and its use

2.1 What is a Health Technology?

Health Technology is a term used to cover any aspect of healthcare. Examples include:

- Prevention programmes (example: childhood vaccination programmes)
- Diagnostic tests (example: the rapid diagnosis of Group-B streptococcus during labour)
- A device or piece of equipment (example: titanium implants in facial reconstruction)
- A drug (example: the use of rapid-acting insulin analogues in patients with diabetes mellitus type 1)
- A procedure (example: laparoscopy).

2.2 What is health technology assessment?

HTA is a form of policy research that examines the short and long-term consequences of using a healthcare technology. It is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Where there is a lack of data, HTA can be used to generate information. HTA is not only relevant to Europe: it has been used by healthcare providers across the world, and its use is increasing.

Although Health Technology Assessment is a relatively recent development as a formal process, decisions to introduce new approaches in healthcare were already based on relevant data. For example, the Swedish Council on Technology Assessment in Health Care (SBU) was established as recently as 1987 as a Government agency and later, in 1992, as an independent public authority. Another organisation, the Institute for Quality and Efficiency in Health Care (IQWiG) in Germany was founded in 2004; the process of assessment, deliberation and final policy decisions are split between IQWiG and the Federal Joint Committee (G-BA). Patient views are obtained by IQWiG through a process described in Appendix 2.

The goal of HTA is to inform the development of safe, effective, health policies that are patient focused and seek to achieve best value as defined by decision makers. HTA underpins decisions such as:

- Should treatment A be reimbursed in a national healthcare system?
- For which patients should it be provided?
- For how long should patients receive the treatment?

HTA is a broad concept: for example, in reviewing a drug it may include efficacy (how it works in the ideal setting of a clinical trial), safety, real world effectiveness, and the likely social, legal, ethical and political impact of using the drug. In some countries there is a formal requirement for economic evaluations (that is, cost effectiveness assessments) to be part of HTA in order to guide reimbursement and access decisions. The scope of HTA is illustrated by some recent examples from Finland:

- Lymphoedema therapy in breast cancer patients: its effectiveness, current practices and costs
- Blood Glucose Self-Monitoring in Diabetes Management
- Assessment of the effectiveness of helicopter emergency medical services

HTA may look at the impact of a technology on an individual patient, on a group of similar patients, on the healthcare system as a whole, or on all of these. HTA may assess evidence from a range of sources, for example:

- Systematic reviews of clinical trials;
- Economic evaluations;
- Assessments of implications for healthcare services;
- Evidence from users of the technology.

HTA may also use modelling, where specific assumptions are used to make an estimate or 'best guess' to predict, for example, the cost of using a technology in a certain setting.

2.3 Why is health technology assessment used?

People facing the challenge of illness need to receive effective treatment and care to give them the best possible chance of health. At the same time, there is a public health need to prevent disease wherever possible. However, available resources may be limited and must be allocated appropriately, based on affordability and effectiveness (many treatments are not effective in all patients with a particular illness).

Decisions such as *‘Should every available treatment for rheumatoid arthritis be provided or should the resources be used to give home care to people with dementia?’* and *‘Should everyone over 50 have their cholesterol tested or should the funds be used to provide vaccines against HPV (human papilloma virus, associated with cervical cancer) for teenage girls?’* are the sorts of challenges faced by policy makers.

Decisions about what treatments should be made available should be based on medical need which is not being currently met. For example, the available treatments may be relevant to a small group of affected patients only or may have side effects which some patients find intolerable. **The need for new and/or better treatment is best understood by the people living with an illness and their caregivers. Their views should contribute to decisions about what healthcare should be provided.** This guide aims to help ensure those views are represented effectively.

2.4 How is health technology assessment used?

HTA is used differently across Europe. In some countries, such as England and Wales, it is used to guide decisions about whether treatments and other technologies should be available on the National Health Service, based on a judgement of whether they provide value for money. In other countries the focus may be less on value for money and more on evidence of effectiveness and cost effectiveness.

HTA can provide information to support decisions about priorities in healthcare or specific decisions about whether new treatments should be introduced, for example:

- Health authorities thinking of putting in place screening programmes;
- Health care payers deciding which technologies (e.g., operations, drugs) should be paid for;
- Health care organisations deciding whether to exclude or implement new technologies (e.g. modern types of radiotherapy);
- Health care companies producing new products (e.g. to demonstrate a level of benefit for the product that justifies the cost).

Some elements of HTA reports can also be used by individuals, including:

- Patients and carers deciding which of the available treatment options best meets their needs;
- Members of the public thinking of taking part in a screening programme.

2.5 How is health technology assessment carried out?

The assessment process and the way the assessment is used varies from organisation to organisation. It may simply be an effective way to assess the available evidence to better understand the clinical condition, or to clarify the state of the art of the technology, or to decide which treatments a healthcare system will pay for. Patients and the public who become involved need a clear understanding of the process and how the HTA will be used. Two examples of an assessment process are included at Appendix 3. A list of national HTA agencies in Europe is included at Appendix 4.

Although the process varies, it is generally acknowledged that four core components are considered in a health technology assessment:

- The existing medical need which is not already being met
- The clinical process which addresses that need
- The evidence on the technology being considered and the interpretation of the evidence
- Value for money of the technology (although this is not always considered)

The evidence may be used to assess the answers to questions such as:

- Does the technology work?
- What benefit does it provide and for whom?
- What does it cost (to the healthcare service, to the patient, etc.), including opportunity costs (what could be gained if the funds were spent on alternative healthcare strategies)?
- How does it compare in terms of efficiency with the available alternatives?
- Does it work in this healthcare setting? Should we do it here? If so, how should we do it? (It is the analysis of *context* that makes HTA different from other disciplines and which allows the assessors to make recommendations to policy makers in individual countries relevant to their own health care system).

DOES HTA REALLY MATTER FOR PATIENTS?



Health economics, in which the principles of economics are applied to health and healthcare, is used to provide evidence to support value for money considerations. **Health economics data may cover both direct costs (such as the number of drugs used by a patient or the number of hospital visits in a given period) and indirect costs (such as the cost of time lost from work).** The cost data combined with clinical effectiveness data leads to cost-effectiveness estimates.

Some HTA processes have a formal methodology for assessing cost effectiveness. This usually involves estimates based on assumptions drawn from clinical data and/or personal experience and opinion. The accuracy of the estimates depends on the assumptions on which it is based. If only very limited health economic data is available for new technologies, the model may not be entirely reliable.

Although the *scientific assessment* of the available data may be similar wherever the HTA is undertaken, the way in which a technology is *appraised* (that is, how the assessment is used to devise recommendations on its value in healthcare) may vary from country to country. As a result, different decisions are taken about which technologies should be provided, leading to variation in access to treatments.

2.6 What if your country does not have health technology assessment processes?

Some countries do not have formal HTA or other similar processes which take external views into account. This practical guide may be useful in such countries as an indication of how to gather information to demonstrate the importance of a technology. This information can be used:

- to persuade healthcare professionals of the value of a treatment to patients
- as the basis of a media campaign
- to lobby politicians
- to support individual patients who are trying to get access to the treatment in question.

Gathering information from a range of people can help to demonstrate that the technology is important to many patients, rather than just to one individual. Even in countries where HTA is used, patients may still use this information to raise awareness of their needs in settings outside the HTA process.

3 Patient, caregiver and the public contribution to health technology assessment

This section sets out why patient experience is important, it describes what patient evidence is and gives some guidance on contributing to the HTA process. It should not be seen as a definitive guide; rather, it is a framework that should be adapted to the particular process involved. The information which supports the patient evidence may be useful for other purposes, such as making the case to politicians for access to a treatment.

Following the advice in this guide does not ensure that the technology will be approved but it may help ensure your voice is heard. The HTA process can lead to technologies being turned down or not recommended.



3.1 Why patient experience is important

Although the outcomes of HTAs may be relevant to a range of stakeholders, the public, patients and their caregivers are often the group most directly affected by HTA decisions. It is important, therefore, that their perspective is considered in the HTA process.

HTA tends to review evidence that is collected in scientific studies (as described in section iii of the Swedish assessment process [see Appendix 3]). As a result there may only be limited evidence about the real impact of the technology on the daily lives of patients, their caregivers and the public. The best way to ensure that the real impact is understood is for the patients themselves (and their caregivers) to provide evidence.

The evidence that patients and carers provide is set in the real world; it is 'experiential' evidence (based on experience). **No one knows better what it is like to live with an illness day in, day out, than those who are doing this - the patients and their family and friends who care for them. It is this unique insight that patients and patient groups can most usefully contribute to the HTA process.** Patients and their caregivers understand and can describe the true benefits delivered by a treatment and the real extent of the unwanted effects. For example:

- Saying you were sick five times each day is less meaningful than explaining that this means you cannot manage to go to work, or that it happens so quickly that you cannot make it to the toilet and have to clean up after yourself.
- Recording that the fatigue caused by existing treatments is so severe that it means you have to lie down all day and so cannot look after your children.
- Explaining that a pill is more acceptable than an intravenous treatment not just because it means less trips to hospital but because it allows you to continue living a more normal life.
- Describing the effect a treatment has on your daily life – such as, 'it makes it impossible to stand on my feet all day, which means I cannot work'.

Other effects might include the time taken in attending clinics or in-patient stays to receive treatments; discomfort during treatment; the problems of not being fit enough to work; the boredom of being attached to a drip for days on end; or the lack of energy to eat properly. **It is the effect of illness and treatment on all aspects of life that is one of the reasons that evidence from patients and the public is so important.**

It may be more beneficial to focus your patient evidence on communicating patient needs rather than advocating a particular treatment as this may enable an HTA organisation to respond to these needs even if the treatment is not approved. Getting involved in an HTA provides an opportunity to increase health professionals' and policy makers' understanding of the needs and preferences of the patients you represent (whether you are an individual or an organisation).

Members of the public (i.e. citizens) have a role in some HTAs. For example, members of the public can clarify the ways in which a screening programme may be unacceptable, which would result in low uptake.

3.2 What is patient evidence?

Patient evidence can be developed through qualitative research, which describes views and opinions rather than the statistical analysis which is typical of quantitative research. Patients and carers may be invited to participate in qualitative research through focus groups or interviews. This is an effective way to contribute to HTA if the opportunity is available.

Alternatively, or in addition, information provided directly by patients, caregivers and the public can make an important contribution to a HTA. **Before developing your patient evidence please check with the relevant agency what you should provide. Individual agencies may have their own guidance for you to follow.**

For example, NICE has a useful guide for patients groups: NICE (2004)

Contributing to a Technology Appraisal: A Guide for Patient/Carer Groups available on their website at

(http://www.nice.org.uk/getinvolved/patientandpublicinvolvement/patient_and_public_involvement.jsp).



Patient evidence might include:

i) The burden of the illness:

- The nature of the illness:
 - Short or long term
 - Some limited symptoms or many
 - Symptoms that are difficult to live with

- Fatal or not
- The limitations it imposes on:
 - Daily life
 - Ability to work
 - Social life
 - Enjoyment of family and friends
- The impact on a person's mental wellbeing
- Activities which people living with the illness find difficult
- Whether the illness prevents people from fulfilling their chosen role in life
- Whether there is pain due to the illness and/or treatment
 - How the pain interferes with daily activities
 - Whether pain medication needs to be taken regularly
- The aspect of the illness that patients find most difficult
- ii) *The technology being assessed:*
 - What patients and carers expect from a new technology
 - What benefits the technology brings
 - How the benefits compare with those of existing treatments
 - Whether it helps affected people fulfil their chosen role in life
 - How important the benefits are to the patient
 - The outcome from a treatment they would value the most
 - How the benefits impact on patients' daily life
 - What unwanted effects the technology causes
 - How the unwanted effects compare with those of other treatments
 - How the unwanted effects are balanced against the potential benefit
 - What would happen to patients if there was limited access to the technology
 - How patients and carers value the technology
 - How easily the technology fits into patients' daily life
 - What is the impact on a person's mental wellbeing?
 - The financial impact of the technology
 - Cost of travel
 - Loss of earnings
 - Cost of paying a carer

It is important to explain in the patient evidence that although a technology may have an unwanted effect which may seem intolerable to an assessment panel, to a person living with the illness it may be a short term inconvenience for a long term gain. For example, bone marrow transplant as a treatment for cancer may be very difficult to tolerate, and requires a stay in hospital in isolation, but if it results in cure, most patients would consider it worthwhile. **It is how the benefits and problems are balanced by the person living with the illness (and their carers) which needs to be explained to, and understood by, the assessment panel.**

Patient groups may want to emphasise that patients use a range of coping mechanisms such as dietary changes or complementary therapies, and to put the technology into context alongside these.

iii) The impact on caregivers:

It is important to include the caregivers' experience. This might include how they are affected by the person's illness. For example:

- Taking time off work to care for the person
- Paying for a carer for the patient
- Paying for childcare because the patient cannot look after the child/children
- Financial hardship because they reduce their working hours
- Watching the patient suffer
- Poor health because all their energy goes into caring for the patient

It might also include how the technology makes life different for the caregiver(s)

3.3 When to become involved

Patients and the public may have an opportunity to be involved at all stages of the HTA process, but this will vary between and within countries. There may be opportunities to suggest topics for assessment that have a particular impact on people living with the illness (see Phase 1 in the NICE assessment process, Appendix 3). This might be a treatment which has fewer or different side effects, it might be access to a process such as physiotherapy, or it might be something that is not available to all patients in a country.

Careful consideration should be given to whether or not a HTA would be useful or whether it could actually reduce access to a technology. This may depend on how HTAs are used: if they are used to determine whether a technology is value for money, a negative assessment could damage access. Where a treatment is very new, or where it is relevant for a small number of people only, there may not be enough evidence to demonstrate value for money which may lead to a negative recommendation.

3.4 How to become involved

Finding out what HTAs are taking place

If you have a national or regional HTA agency/unit (see Appendix 4 for a list of HTA agencies in Europe), you should look at their website to see whether they provide their work plan. There may be more than one agency performing HTAs in a country or the agencies may specialise in a particular aspect of HTA so you may need to check several websites. Information may also be available on government health websites. If this information is not available on the internet, write to or call the agency and ask for it.

Deciding whether to contribute to a HTA

If the HTA will impact on you or someone close to you, or the people who use the services of your organisation, and you have the resources to do it, you should consider contributing information to the assessment about the illness and/or the technology. It is important that the panel carrying out the assessment understand what living with the particular illness is like, and you or your organisation can provide that evidence.

What if we are not asked to contribute information?

If you are not asked to contribute information to the HTA, you should contact the agency carrying out the assessment and offer your help. People who might be affected by the assessment have an important perspective to provide. You should explain the experience you have (with the clinical situation and/or the technology) and the relevance of what you are able to contribute.

For example, an assessment of a speech therapy programme for stroke patients would require an understanding of the practicalities of delivering such a programme and people affected by stroke (the patients and their carers) are best placed to provide this. Or an assessment of the relative values of in-patient and out-patient management strategies for patients with rheumatoid arthritis would benefit from a clear understanding of how acceptable the two approaches are to patients (issues such as travel, childcare, time off work, etc. may all impact on how the two approaches are viewed).

3.5 How to provide patient evidence

The earlier section '*What is patient evidence*', outlines the information patients, caregivers and the public might provide to an assessment. Below is described *how* you might provide this patient evidence.

Putting together written patient evidence

The key to making an effective contribution is to ensure that the important messages you want to get across are clear. The precise content of your experience-based evidence will depend on the assessment agency requirement. For this reason it is a good idea to ask the agency for advice. This section gives a sample format that will need to be revised to suit your particular circumstances. **A checklist for the contents of patient evidence is included at Appendix 5.**

Patient groups are able to collect information on the experience of an illness from a range of sources. Take time to plan what you would like to include in your patient evidence and how you will gather any evidence you wish to present:

- Do you have some of the data already (for example, enquiries to a help line may have uncovered some relevant information)?
- Do you want to ask patients directly about their experience through a survey?
- Can you use focus groups or questionnaires? One-to-one discussions?
- Are there support groups you can talk to about the problems patients and carers face?
- Have the issues been discussed on websites or at official meetings?
- Consider asking patients and their carers to choose between the technology being appraised and the standard it is being compared against: what would they choose, and why?

The amount of evidence an assessment panel may have to digest is likely to be large, so don't produce a long contribution if it isn't needed. If there are a lot of data supporting your evidence, such as the results of a survey, include these as an appendix so it doesn't dilute the messages you want to emphasise.

Some personal quotes or experiences can usefully illustrate the points you wish to make about the technology and will help the patient evidence 'come alive' to the assessment panel. But remember that the panel is expert at assessing scientific evidence and may not be influenced by anecdotes.

Don't assume that the panel will understand all the implications of the illness – it is more likely that they will not (although they may think they do). At the same time, don't get stuck on small details, make sure you put the emphasis on things that matter.

Learn from the experience of others. Ask groups which have participated how they have put their patient evidence together – ask them what worked and what didn't. Do ask for advice from the HTA agency undertaking the assessment; they will know what they need from patient evidence.

Patient groups should not try to provide the type of scientifically rigorous evidence that the professional agencies will provide. It is the unique experience of living with an illness that makes the evidence from patients and the public so important.

Providing written evidence

The format of the written patient evidence might use the following headings:

- a) Why your contribution is valid
- b) Commentary on existing data
- c) Experience of the illness
- d) The impact of the technology

a) Why your contribution is valid

In order to appreciate the value of evidence from an individual or a patient group, the panel undertaking the HTA need to understand the basis on which it is provided. It should include:

- A description of the individual's experience or the organisation submitting the evidence and its users
- An indication of the people the organisation reaches
- The type of services the organisation provides to its users
- How the patient evidence was collated and whether it was newly collected or already existed.

This will help the panel understand the validity of the evidence.

A particular challenge for patient groups is that the people who use their services and whose views they represent may not reflect the interests of all patients. This does not make their views less important, but the patient evidence should acknowledge that the views expressed may not be those of all patients.

The patient evidence must not lose sight of the technology that is being appraised. A patient's life story is relevant only as a way of explaining the experience of living with an illness. The emphasis should be on information that is only available from the patients and carers.

b) Commentary on existing data

A commentary on existing data explaining how it is relevant to people affected by the illness may illustrate the patient perspective. The data might be clinical trial reports which can be accessed through science libraries or from patient groups' medical or scientific advisors. Patient evidence can explain the value of a technology in relation to the overall illness management strategy and can set it in the context of self-management, which many patients see as a priority.

Quality of life – an expression reflecting how content a person is with their current life status and well being - is the area where patient groups can potentially have the most to contribute to HTAs. The importance patients and caregivers assign to quality of life this needs to be reflected in the patient experience. Quality of life assessments from trials may benefit from interpretation by people affected by the illness being studied, to help HTA panel understand the impact on daily living of the technologies being studied. Patients and their caregivers know precisely how an illness impacts on daily life and how specific treatments or management strategies can influence its quality. Although clinicians understand much about diseases and may be sympathetic to the patients' situation, it is unlikely that they have experience of living with the illness on a day to day basis. Only the patients and caregivers can describe what this is like.

Many tools that measure quality of life are specific to a particular disease or are related to an aspect of illness (such as pain, breathlessness, etc). A list of these is available at <http://phi.uhce.ox.ac.uk/links.php>. It may take some effort to interpret the scientific evidence in a way that matters to patients and carers, but doing so might help the assessment panel to get a better understanding of its relevance.

Carers' quality of life is beginning to be recognised as an important aspect of illness but as yet is often poorly assessed or recorded.

c) *Experience of the illness.* This is covered in detail in *Section 3.2, What is patient evidence.* In brief, it should address the experience of living with the illness:

- Symptoms
- How it affects daily living
- Short and long term impact on length and quality of life
- The psychological and sociological effects of the illness

d) *The impact of the technology.* This is covered in detail in *Section 3.2, What is patient evidence.* In brief, it should address the effects the technology has on the patient and their caregivers. It might include:

- Benefits and unwanted effects of the treatment
- Impact on everyday life
- The psychological and sociological effects of the treatment and of not having the treatment
- The financial impact

Presenting evidence in person

In some HTA processes (such as NICE, in England & Wales), people living with illness are given the opportunity to attend meetings and present their evidence in person. Make sure you choose individuals who are able to make their message understood by academic and other professional experts. NICE acknowledges that it can be difficult for patient experts and provides them with dedicated support before and after attending meetings.

Make sure you deliver the two or three key messages you want to deliver at the beginning of any presentation while you have the panel's attention. You can then illustrate the points and return to reinforce them at the end of your allotted time.

Below are some general assertiveness methods that you can use to make you more comfortable in the meeting:

- Try to sit in the line of the Chairman's eye
- Have a notepad so you can write down what you want to say in response to anything said in the discussion
- If you are anxious, rehearse in your head how you will say something
- Acknowledge that you have understood the points being made but, if you do not agree, explain that you do not
- Be polite but persistent if you want to make a point
- Use brief statements that are to the point
- Avoid using expressions such as 'you must' but introduce your comments in a non-threatening way, e.g. 'people with this illness would prefer'
- If you feel that your comments have been misunderstood, make the same point but in a different way

You may have limited time to get key points across and you might not be asked the questions you want to answer, so a simple 'trick' can be useful. It is known as ABC - Answer, Bridge, Control; alternatively it is known as a three-part sentence. When asked a question you:

- **A**nswer it with your first phrase
- provide a link or **B**ridge to the message you want to give
- then provide the information you want to get across, that is: take **C**ontrol.

For example, say you want to make the case that physiotherapy allows you to walk unaided to the bathroom and this saves the embarrassment of asking someone to help you.

The panel ask you 'how many steps you can take without help?'

You can answer 'I used to manage five steps but after physiotherapy I can walk 20 steps, which allows me to go to the bathroom without help'.

It can also be helpful to point out a *consequence* for patients of an illness or treatment that the panel may not have considered in the discussion.

3.6 Working in partnership

There are many non-professional groups which have a legitimate view to express in HTA. Patients and the public may benefit from working with their colleagues in a range of settings, including universities, insurance companies, scientific societies, healthcare and industry. All these groups may share the interests of patients, carers and the public. Many of these stakeholder groups will be involved in the HTA process; for example, they may contribute evidence that is considered in the process, or they may clarify how the technology would be used.

Think about who might make good partners: are there other groups you can work with to make a submission? A joint contribution from several groups, who between them represent the views of a large number of patients, may be very powerful. By presenting a united voice, there is a greater chance that this voice will be heard. Patient groups can gather support from the people who use their services and this can strengthen the impact of their voice.

Professional groups [e.g. groups of doctors, nurses, or pharmacists] may share your organisation's views of a technology. If so, try to ensure that your submissions are coordinated so the assessment panel can see that there is a uniform view about the technology's value. Be aware if other stakeholders have a different view of the technology to yours: if they do, address this in your patient evidence.

It may be helpful to share your patient evidence with other stakeholders that you know are involved in an assessment. This may help to ensure that the panel does not receive conflicting information about the patient experience.

4.7 What will happen to our contribution?

The experience of people living with the illness, the patient evidence, will be considered with all the other available data by the expert group whose role is to assess the evidence.

Providing patient evidence does not ensure that the technology will be approved but it may help ensure your voice is heard. The HTA process can lead to technologies being turned down or not recommended.

How the patient experience is used will depend on the HTA process. It is important to clarify with the HTA agency how your contribution will be used, for example:

- Will it be considered with all the other available evidence?
- Will it carry as much weight as the other evidence?
- Is there a particular stage in the process when it is relevant?
- Will there be more than one chance to contribute to the process?

You should also find out if there is someone within the agency who can advise you about your contribution.

What happens after the assessment will vary. There is likely to be a report with a recommendation on how the technology should be used, in which group(s) of people and when. This may recommend that the technology is not used at all or it may limit the group of patients for whom it is recommended. If this is produced as a draft report, you may have the chance to provide comments on the draft. You may need to read the technical information in the report and this might be difficult to understand. The terminology can be confusing but a brief glossary is given in Appendix 6 (and a full glossary is available at http://www.inahta.org/upload/HTA_resources/Edu_INAHTA_glossary_July_2006_fi nal.pdf).

You may need to ask for help in interpreting the technical information. Help may be available from your organisation's scientific advisers, from an academic department that specialises in this type of work (try searching the local university or college website for 'HTA', or 'public health', or 'health administration' or 'health

economics'), from the producer of the technology or from the HTA agency. It is important that you understand the implications of the draft report so do ask for help if you need it.

Once the report is finalised decisions will be taken on whether or how to implement the recommendations.

In some assessment processes, if you do not agree with the recommendations there may be the chance to appeal. If this is the case, the appeal process will be explained to those groups or individuals that have provided evidence. There may be an opportunity to explain to the assessment panel that they have failed to take account of issues that are important to patients. The assessment panel may not have understood that patients need a choice of treatments because some patients can tolerate certain treatment-associated side effects that others can not. For example, some patients tolerate one type of statin while others cope better on another.

Not all patient groups or other contributors will choose to appeal. It can be frustrating if you feel that your views have not been heard but it may be that other factors were considered to be more significant. Other contributors may not have their views incorporated either – don't assume that your evidence was not influential because you represent patients and the public.



4 Conclusion

HTA is likely to become an increasingly important influence in decisions about what is provided in healthcare, whether screening for diseases or treatments for serious illnesses. Already in Europe there is considerable discussion about greater collaboration between HTA agencies.

It is important that HTA takes account of the needs of the people who will be most affected by its recommendations: patients, their caregivers and the public. While participating in a HTA can be time consuming and challenging, it is a real opportunity to influence the delivery of healthcare. Patient and public input can help determine whether a new treatment is made available. This guide will, we hope, help patients and the public make the most of any opportunity to contribute to a HTA.

Appendix 1 - Case studies

Blodcancerförbundet



Patient opinion, blood cancer

For information about the Organisation, see following page

Description of the disease:

I have a Chronic Lymphatic Leukaemia (CLL) diagnosed nine years ago at the age of 61. I have gone through chemotherapies three times during these years. Each period of treatment has lasted for about half a year. I have been able to live a normal life and my disease has not really affected my relations to family and friends. During periods of treatment I have been sensitive to infections and I have had to be careful about taking part in social events and to travel by public transport. I have been able to work part time after my retirement. The chemotherapies have given minor long term problems with sensitive feet (like those of diabetes). However, I have no problems taking walks and doing other physical activities. On the other hand these treatments have had to be repeated at about two year intervals.

Present treatment:

This time the treatment is an antibody, MabCampath. The treatment is a subcutaneous shot in the thigh three days a week up to twelve weeks. The result of the treatment is followed by blood tests at least once a week. It is a relatively new treatment specific for CLL patients who have gone through several chemotherapy treatments without having received remission for longer periods.

Risks and benefits:

I have to take preventive medication against virus and bacteria and at start my thigh became purple and swollen. After six weeks I got fever and a virus attack (CMV) and the treatment had to be stopped for about a month. I have got blood transfusion three times and I am now on treatment with Erythropoietin (EPO) once a week. The same risks of infections as of chemotherapies do exist. This treatment is however for the moment the only option available that can give hope for a longer remission than I have received before.

Impact of the technology:

The treatment as such is easier to take than the previous ones as it does not involve intravenous injection. On the other hand it is like working part time to go to the hospital every other day. It takes me half the day when a blood test is taken and some weeks it is necessary to test all three days of the week. There is also not much to do while waiting, often I read.

The side effects, fever and virus, have had unwanted impact on my normal life during the time of treatment and this will most likely continue for at least another half year. Fever and virus made a break of the treatment of almost one month. During almost two weeks of this break I was really ill.

February 2008



The Swedish Blood Cancer Association

The task of the Blood Cancer Association is to support those who are affected by a blood cancer or another severe blood disease, their families and to haematological personnel. It was founded in 1982.

Our goal is that everyone having a blood cancer disease should be offered good and equivalent treatment and rehabilitation independent of where in the country they live.

To reach this goal we

- raise requirements on authorities and politicians in the interest of blood cancer patients as a body to which proposed measures are referred for consideration
- support research and development education within haematology
- spread knowledge about blood cancer diseases for example through medical booklets

The Blood Cancer Association has its central office in Sundbyberg (a suburb of Stockholm) and a number of local associations in different parts of the country. The local associations keep contact with local hospitals.

Some of the local associations also have economic foundations that make it possible for members and nursing staff to ask for support. Additional expenses for patients with illness as well as continual training for nursing staff are supported locally.

Our local associations offer support for those who recently have received a diagnosis and for their families. Experiences are thus taken care of and are valuable complementary information to the medical care given at the hospital. Nationally a network is formed to continually train these supporters.

The Blood Cancer Association is a non-profit organisation having support from the Swedish state. It is one of the principals for the Swedish Cancer Society. Internationally we are a member of the Lymphoma Coalition, Myeloma Euronet, The Leukaemia & Lymphoma Society, The European Cancer Patient Coalition (ECPC) and CML Advocates Network.

The Blood Cancer Foundation

The Blood Cancer Foundation is a non-profit foundation formed by the Blood Cancer Association. The entire return of the foundation goes to support research and development in haematology. The Blood Cancer Foundation is checked by Stiftelsen för insamlingskontroll, a member of IFCO, the International Committee on Fundraising Organisations.

Experience of the HTA setting by Myeloma UK

Myeloma UK has been involved with two NICE technology appraisals – erythropoietin for cancer treatment-induced anaemia and bortezomib (Velcade) for treatment of relapsed / refractory myeloma.

What went well during these appraisals?

Whilst Myeloma UK spearheaded the appraisal submissions, we recognised that submitting evidence jointly with other leading charities was a stronger approach to take. Working in partnership with other charities towards a common goal is a positive way of demonstrating unity to NICE, and is also a constructive means of brainstorming and sharing differing levels of knowledge about the NICE system.

We also worked very closely with the doctors submitting evidence to the appraisals. It is valuable to share thoughts, ideas and approaches but more importantly, it is crucial to have integrated tactics when submitting to NICE. The arguments used by all stakeholders are given much more weight by NICE if they are consistent.

Finally, we spent a considerable amount of energy mobilising the UK myeloma community, communicating the importance of the HTAs to them and the consequences of the subsequent decisions. The volume of support we got, and the action that the community took throughout the appraisals, was fundamental to sensitising the issues and keeping them alive in both the minds of the media and NICE.

What issues did we face?

An HTA presents a very steep learning curve for most patient groups / representatives, and it is easy to suppose that submitting emotional arguments from the patient perspective will be enough to win NICE over. Not the case. Rather it is meaningful, experiential evidence that can resolutely demonstrate the effects of approving and rejecting the technology on patients that is most effective.

It is also critical that patient groups / representatives quickly learn the practical requirements of an appraisal as the theory can only teach you so much about the process. For example, facing an panel of inquisitorial experts in an appeal setting can be quite a nerve-wracking experience, but one that becomes less daunting by speaking with others who have been through it in the past.

What would we have done differently?

From the get go we would have placed more importance on the power of supportive case studies and quantitative evidence to support our arguments.

Our top three tips

- Work in partnership with other groups / representatives to mount a unified approach
- Gather together evidence and meaningful case studies to support the emotional arguments
- Seek advice from others that have been through the process before you

Appendix 2: How IQWiG obtains the views of patients and the public

German Institute for Quality and Efficiency in Health Care (IQWiG)

Structural framework

IQWiG's evidence assessments are undertaken within a framework where the process of assessment, deliberation and final policy decisions are split between two agencies: IQWiG (the Institute) and the G-BA (Federal Joint Committee). The key steps are split as follows:

1. Commissioning of topic and detailing of the commission: G-BA
2. Evidence assessment and interpretation with recommendations/advice based on that evidence: IQWiG
3. Deliberation and policy decisions: G-BA
4. Implementation: G-BA (and, where applicable, the Federal Ministry of Health).

The input of patients at the level of the G-BA and the Federal Ministry occurs through a group of organisations of patients and people with disabilities that are officially designated as the representative organisations for Germany. This includes major coalitions of self-help groups and groups representing people with disabilities. The communication channel for community groups, then, is via this representative structure.

Representatives of these groups are involved in all the steps described above at G-BA, through the G-BA's own consultative mechanism with these organisations. The patient representatives do not, however, have voting status.

IQWiG's processes for understanding the views of patients and the public in its evidence assessments

Patients and the general public are represented within the Institute's structures in its Board of Trustees ("Kuratorium"). Here, the patients' organisations from the G-BA structure hold several seats on the Board. In addition, the Federal Commissioner for Patients' Issues is also represented. These representatives are involved in all consultation processes of the Institute's work. This includes the opportunity to comment on the drafts of IQWiG's evidence assessments. IQWiG is in a continuing dialogue with these groups on the processes for considering the views of patients and the public in its evidence assessments. It has run a training session for patients' representatives on participating in its hearings, and may develop further support activities in consultation with patient groups.

Consultations are held on IQWiG's evidence assessments at two stages of their development:

1. Protocol: the planned methods for the evidence assessment
2. Draft report: the Institute's assessment of the evidence

It is also possible for the Institute to undertake rapid reports, where these steps do not apply. This is a small minority of the Institute's commissioned evidence assessments.

Preliminary consideration: patient-oriented outcomes

In addition, the Institute has a particular focus on the views of patients and the public in determining the patient-oriented outcomes for its evidence assessments. In this, it relies on two key inputs:

1. Published and other relevant literature on the views of patients and the public
- 2.

Consultation with patient representatives as nominated by the officially designated representative groups

Consultation with patient representatives often includes a personal meeting with one or more representatives. While these are usually from organisations nominated as above, additional groups are also sometimes involved, especially in areas where the G-BA representatives have not nominated patients.

In addition, the Institute has the possibility of running informal "poll-type" surveys of the public. This has been developed, in conjunction with patient groups, for the Institute's patient information.

Consultation on drafts:

All commissioned evidence assessments are listed on the Institute's websites. Any organisation or member of the public can subscribe to its "Infodienst" (Email alerting service) on evidence assessments. By this mechanism, IQWiG informs all interested parties of progress in the individual evidence assessments, including public announcements of opportunities to participate in consultations on drafts.

Public consultation periods on drafts are for one month. The drafts are published on the IQWiG website.

IQWiG has provided a plain language “FAQ” document to assist individuals and organisations in understanding its process for declaring potential conflicts of interest.

In most cases, the Institute will also hold hearings to further discuss the issues raised in comments on drafts. These can be held both for draft protocols and for draft evidence assessment reports. Representatives of patients who have submitted comments on the draft may also be invited to participate in these hearings.

The comments in written consultations as well as hearings are summarised and published along with final protocols and evidence assessment reports. Each version is published on the IQWiG website.

12 February 2008

Appendix 3: The assessment process

The assessment process differs from country to country, or even region to region, and you will need to identify the process used in your country to see where and how you can contribute. Two examples of the assessment process are described below.

The example of the National Institute for Health and Clinical Excellence (NICE) in England

Phase 1: Proposing topics for appraisal

Patients and the public are able to propose topics which NICE should consider through a form available on the NICE website.

Phase 2 Scope

The scope defines the technology to be considered, the patients and disease in which it is used, and – if appropriate – what it will be compared with. (This may be, for example, physiotherapy versus medication for back pain.) National organisations representing the interests of patients and the public that have been identified by NICE as having an interest are able to contribute to defining the scope.

Phase 3: Appraisal

- i Once it is agreed that NICE will undertake an appraisal, a range of stakeholders are consulted including patients or carers using the technology, the public (if appropriate), the technology manufacturer, healthcare professionals, and hospitals or other healthcare settings where the technology may be used.
- ii Once all the data are collected, an Assessment Report on the clinical efficacy and cost effectiveness of the technology is produced.
- iii The Assessment Report is reviewed by an independent Appraisal Committee. The Appraisal Committee hears evidence from nominated clinical experts, patients and carers.
- iv The Appraisal Committee makes **provisional** recommendations about the technology. Patient groups and other external stakeholders are given the opportunity to comment on the provisional recommendations.
- v The Appraisal Committee considers all the comments on the provisional recommendations, and issues **final** recommendations to NICE. The final recommendations may have been altered in the light of comments received, but this will not always be the case.
- vi NICE publishes the guidance for the National Health Service on whether and how the technology should be used, and for which group of people.
- vii If any of the interested parties do not agree with the recommendations there is an appeal process. This may result in the evidence being re-assessed and a new assessment produced, or it may not result in any change.

The example of the Swedish Council on Technology Assessment in Health Care (SBU)

i. Define the topic

First, the purpose of the assessment is defined. As the project begins, the group decides on the issues to be included and excluded.

ii. Set the standards for quality

Inclusion criteria are established for the quality and relevance of the studies. Only the results from research that is sufficiently rigorous will be used. SBU's list of common pitfalls provides examples of unacceptable results. In some cases, project groups may decide to include studies of lower quality or relevance if the material presents unique information.

iii. Collect relevant research findings

All available research findings addressing the important issues are systematically searched in computerized databases and by manually scanning reference lists in professional journals and scientific reports. Searches may identify several thousand articles if the assessment concerns a broad area, for instance, common methods to treat obesity or substance abuse.

iv. Select studies of acceptable quality

Since the quality of research may range from high to low, project groups must separate the 'wheat from the chaff'. Each research report is carefully reviewed and evaluated. The project groups evaluate every study for quality and relevance. At times, the groups use standardized checklists in this process. Research reports that do not meet the predetermined criteria for quality and relevance are eliminated in this step of the review process.

v. Weigh the results

Results from the selected studies are summarized in tables, scrutinized, and used to form the body of evidence. As with searching and selecting the literature, weighing the evidence must also be systematic and rigorous. All conclusions drawn must have scientific support. It is important to address not only the medical effects of different methods, but also the prevalence of the problem, current practices in Sweden, and the economic, social, and ethical aspects.

vi. Summarize the evidence and draw conclusions

Before SBU publishes its findings, the manuscript is evaluated by external experts and by experts from the SBU Scientific Advisory Committee. Manuscripts are always carefully edited, and the language is revised prior to publication. The SBU Board of Directors and the Scientific Advisory Committee approve the conclusions drawn from the evidence and consider the findings in a broader context. As a rule, the strength of the scientific evidence is noted for each conclusion by using the SBU evidence grading scale.

Appendix 4 – National and regional health technology assessment agencies

Austria	Ludwig Boltzman Institute of Health Technology Assessment, LBI@HTA (former ITA) Tel: +43(0)1-2368119-0; E-Mail: office@hta.lbg.ac.at
	Gesundheit Österreich GmbH, Austrian Health Institute Tel.: +43 1 515 61-0; E-mail: info@goeg.at
	Hauptverband der Österreichischen Sozialversicherungsträger Tel:+43 (1) 711 32; E-Mail:posteingang.allgemein@hvb.sozvers.at
Belgium	KCE - Belgian Health Care Knowledge Centre Tel: +32 2 287 33 88 or +32 2 287 33 97; E-mail: info@kce.fgov.be
Denmark	CAST - Center for Anvendt Sundhedstjenesteforskning og Teknologivurdering, University of Southern Denmark, Center for Applied Research and Technology Assessment Phone: +45 6550 1000; Email: sdu@sdu.dk
	DSI- Danish Institute for Health Services Research Tel.: 35 29 84 00; E-mail: dsi@dsi.dk
	DACEHTA – Danish Centre for Evaluation and HTA Tel: +45 72 22 74 00; E-mail: sst@sst.dk
	HTA and Health Service Research, Center of Public Health Tel: 8728 4750; E-mail: mtv-stf@rm.dk
England & Wales	NICE - National Institute for Health and Clinical Excellence Tel: +44 (0)845 003 7780; E-mail: nice@nice.org.uk
	National Institute for Health Research HTA Programme Tel: 023 8059 5586; E-mail: hta@hta.ac.uk
Estonia	University of Tartu, Department of Public Health Tel: +372 737 5100; E-mail: info@ut.ee
Finland	FinOHTA - Finnish Office for HTA Tel. +358 9 3967 2297; E-mail: finohta@stakes.fi
France	HAS - Haute Autorité de santé / French National Authority for Health Tél. : 01 55 93 70 00
	CEDIT - Committee for Evaluation and Diffusion of Innovative Technologies, Direction de la Politique Médicale Tel: (33) 1. 40. 27. 18. 90; E-mail: info.cedit@sap.aphp.fr
Germany	DAHTA@DIMDI- German Agency for HTA at the German Institute for Medical Documentation and Information Tel: +49 221 4724-1
	IQWIG - Institute for Quality and Efficiency in Health Care Tel: +49 - (0)221 - 35685 – 0
	German HTA Association Tel (0421) 218-3784
Hungary	HunHTA - Unit of Health Economics and Health Technology Assessment Tel: 0036-1-482-5147; E-mail: laszlo.gulacsi@uni-corvinus.hu
Ireland	HIQA - Health Information and Quality Authority Tel: +353 21 425 0610; E-mail: info@hiqa.ie
Italy	ASR - Agenzia Sanitaria Regionale, Emilia Romagna E-mail: fsarti@regione.emilia-romagna.it
	Università Cattolica del Sacro Cuore, Policlinico universitario “A. Gemelli”, Health Technology Assessment Unit and Laboratory of Health Economics (Institute of Hygiene) Tel. +39 06 3015 1; E-mail: rettorato@rm.unicatt.it
Latvia	VSMTA - Health Statistics and Medical Technology State Agency

	Tel: +371 67501590; E-mail: agentura@vsmtva.gov.lv
Netherlands	CVZ - College voor zorgverzekeringen Tel: (020) 797 8555; E-mail:
Poland	Agency for HTA in Poland, AHTAPol Tel. +48 22 566 72 00; E-mail: szkolenia@aotm.gov.pl
	CEESTAHC - Central and Eastern European Society for Technology Assessment in Health Care Tel. +48 (0) 12 357 76 34; E-mail: sekretariat@ceestahc.org
Scotland	SMC – Scottish Medicines Consortium Tel: +44 141 225 5552; E-mail: smcsecretariat@nhshealthquality.org
	NHS Quality Improvement Scotland Tel: 0131 623 4300; E-mail:
Slovenia	Institute of Public Health of the Republic of Slovenia Tel. +386 (1) 2441 518; E-mail: knjiznica@ivz-rs.si
Spain	AETS - Agencia de Evaluación de Tecnologías Sanitarias Tel: + (34) 91 822 20 04; E-mail: aets@isciii.es
	AETSA - Andalusian Agency for Health Technology Assessment Tel: +34 955006638; E-mail: aetsa.csalud@juntadeandalucia.es
	CAHTA - Catalan Agency for Health Technology Assessment and Research Tel. +34 935 513 888; E-mail: direccio@aatrm.catsalut.net
	Galician Agency for Health Technology Assessment Tel (0034) 981 542 737
	OSTEBA - Basque Office for Health Technology Assessment (AP) Servicio Canario de la Salud Tel.: + 34 945 019250; E-mail: Osteba-san@ej-gv.es
	UETS - Unidad de Evaluación de Tecnologías Sanitarias, Agencia Laín Entralgo Tel: +34 913089480; E-mail: uets.ale@salud.madrid.org
Sweden	SBU - Swedish Council on Technology Assessment in Health Care Tel: 08-412 32 00; E-mail: info@sbu.se
Switzerland	SNHTA - Swiss Network for Health Technology Assessment Tel: ++41 31 322 15 86; E-mail: info@snhta.ch

Appendix 5 - Checklist for patient group expert opinion

- 1 Description of the organisation giving evidence
 - *Purpose/aims and objectives*
 - *What services it provides and who uses it (number, types of users)*
 - *Who are the medical and scientific advisers*
 - *How the evidence was collected for the submission, for example from existing knowledge or newly collected in surveys, focus groups, etc*
 - *Funding sources*

- 2 A description of the disease and its impact on the patient
 - *How does it affect daily life? What does it prevent patients from doing?*
 - *What are the main disease-related problems faced by patients? Which of these cause patients the most trouble?*
 - *How does the disease affect patients' ability to work, social life, mental health, etc?*
 - *How does it affect the patient's relations with family and friends?*

- 3 A brief description of the technology and how it fits with the existing treatment and care options of patients with the disease.

- 4 Details of the benefits and risks of the technology under consideration: what specific benefits does it provide and at what 'cost' to patients and carers.
 - *What benefits does it bring? How do these impact on patients' daily life?*
 - *How do the benefits compare with those of existing treatments?*
 - *What unwanted effects does the technology cause? How tolerable are they? How do they impact on the patient's daily life?*
 - *How do the unwanted effects compare with those of other treatments?*
 - *What would happen to patients if there was limited or no access to the technology?*
 - *How easily does the technology fit into patients' daily life?*
 - *Do they have to go to hospital to receive it?*
 - *Do they have to take extra time from work?*
 - *Does the technology prevent them from doing anything routine?*
 - *Is anyone else affected, such as a family member accompanying the patient?*
 - *Does the technology impact on the patient's ability to work? Their self management of the illness? Their home life? Their social life and relationships?*

- 5 How do the illness and the technology affect the patient's family and friends?
 - *Do they have to take time from work to care for the patient?*
 - *Is there financial impact, such as a paid carer?*
 - *Is childcare a problem?*
 - *Is the carer's health poor because of the caring?*
 - *Is there an emotional burden?*

- 7 Any other relevant information

Appendix 6: Terminology used in HTA

Here are described some of the most commonly used terms in health technology assessments. It is intended to help patients and the public make sense of documents which contribute to health technology assessments.

A full glossary of the terms used in health technology assessment is available on the International Network of Agencies for Health Technology Assessment website at

http://www.inahta.org/upload/HTA_resources/Edu_INAHTA_glossary_July_2006_fi nal.pdf

Appraisal: This is the process of using the assessment evidence to derive recommendations about the use of a given technology. It may involve judgements of the relative value of the technology which may not be shared by different countries.

Appraisal Committee or Assessment panel. This is the expert group that will consider the available evidence and assess what it means for the technology.

Assessment. This is the process by which the health technology agency reviews the existing evidence to reach a conclusion on what all the data, taken together, tells us about the technology being assessed.

Bias. This is where the results are not as expected because of the impact of a factor not controlled for in a study. Bias is a systematic deviation, rather than a random ('play of chance') effect.

Case control study. This is a study where a given population – for example, people who have experienced a particular illness – is compared with a population which shares many characteristics *except* the one being studied. This type of study can be used to identify the causes of disease, for example that smoking causes lung cancer.

Clinical efficacy. This measures the impact of a technology in a clinical trial setting. If it is a drug to treat patients with Parkinson's disease, how much relief of symptoms do patients get? How sustained is the benefit?

Clinical effectiveness. This refers to how well the technology works in everyday circumstances.

Confounding. This occurs when an external factor interferes with a scientific assessment such as a clinical trial by influencing the outcome being measured. It can make the results of a study less clear cut than the researchers had planned.

Cost effectiveness. This is, at its simplest, how much benefit a technology delivers for what cost. The costs are measured in monetary units and outcomes in non-monetary units, e.g. life years gained or reduction of blood pressure.

Cost utility analysis. This is a type of study in which the consequences are measured in terms of financial cost and benefits are measured in terms of their

value, or utility, to the patient. The best known cost utility analysis is the cost per QALY.

DALY, or disability adjusted life year. The DALY measures the 'healthy' life years lost to disability or to being in a poor state of health. It is intended to measure the 'health gap' between the life of a person who is entirely healthy and lives a long life and a person who is unwell or disabled and whose life span is shorter than the ideal.

Discounting. This is a term used to describe how future costs and benefits may be worth less than current costs and benefits. Put another way, money spent now, or health benefits gained now, may be worth more than in the future. Discounting is relevant to assessments where the benefit may not be immediate but may occur in the future (such as in screening programmes).

EUROQOL or EQ-5D. This is a measure of quality of life which was developed when researchers identified that the same value was attached to different health states in several European countries. It measures quality of life in five dimensions and three levels of severity within each dimension. It is not disease specific so allows comparisons across different diseases.

Evidence. This is the information that is considered in the assessment. It may come, for example, from clinical trials or reviews of several trials, it may come from models of costs involved, and it may come from those living with an illness or the family and friends caring for them.

HRQOL, Health related quality of life. This term is used to describe the quality a person assigns to their life in health terms only; it does not include aspects such as ability to work, or socio-economic status.

Health technology. Health Technology is a term that can be applied to any aspect of healthcare: prevention programmes, a diagnostic test, a device or piece of equipment, a drug, or a procedure that may be used in any aspect of healthcare.

ICER, Incremental cost-effectiveness ratio. This is the extra cost associated with each unit of health gain achieved by using one technology rather than another. It is used, for example, to compare a new technology with one in standard use (such as a new treatment for diabetes compared with a standard treatment).

ITT, Intention to treat. This is a term related to the analysis of clinical trials. In a comparison of, say, two treatments the outcomes are assessed on the basis of the treatment the person was *intended* to have, even if in practice they received something else.

Internal & External validity. Validity refers to how 'true' something is. External validity relates to how well the result would hold up in other circumstances and is an assessment of how generally the results might be applied. Internal validity relates to how certain it is that the effect that has been identified is indeed due to the intervention being studied.

Life years gained. This identifies the additional number of years of life which a person lives as a result of the technology being studied. The comparator is life without the technology. For example, a drug may add weeks, months or years of life to a person. This measure is not appropriate for treatments which do not prolong life, in which circumstances the QALY may be more appropriate.

Outcome. This is the end point that is being examined in a clinical trial. It may be survival, quality of life, time to disease progression, disease-specific mortality, etc. There may be primary outcomes – the main effects - being studied and there may be secondary outcomes which are important but not the main purpose of the trial.

Opportunity cost. This is a reflection of what *cannot* be done as a result of using the money on a particular technology – i.e., what opportunity will be lost as a result of funding or using this technology. If the money is spent providing this technology it is not available to use on some other aspect of healthcare.

QALY, or quality adjusted life year. The QALY attempts to put a joint numerical figure on the extent and quality of survival for an individual. It allows comparisons between different diseases by using a value which takes account of both the length of a person's life and their quality of life. Because it takes account of quality it can be a negative number (if the quality of life is very poor).

Often in HTA the measure used is the 'cost per QALY', which is used to assess how expensive (or cheap) the treatment is in delivering the period and quality of survival.

QOL or Quality of life. This is a measure of how well a person is feeling in physical and mental health and social wellbeing. It is ideally measured by the patient and there are several scales available to do this.

Time trade off. This is a way to assess the value of a technology by measuring the point at which someone would choose between two scenarios. It suggests how much extra time alive a person might be willing to give up to have better health now: how much time will they trade for better health. A person might be given different choices, in a pretend situation, of how many months of life they would be willing to lose for how many months without symptoms. It is one method to generate utility measures.

Utility. This measures a person's preference for a particular health outcome – usually expressed as being between zero and one (e.g. 0= death; 1=perfect health). A numerical value is assigned to this by using different methods like time trade off or standard gamble.

Reference case. This is the standard against which a technology is being assessed.

Scope. This describes the parameters within which a health technology assessment is examining the technology in question. It is likely to cover the disease (or the stage of the disease), the patients (which may be all patients or a subset of patients), the technology to be assessed and what it will be compared against. It defines the assessment: problems with the scope can affect the

outcome of the assessment– for example, if the comparator is not appropriate in clinical practice the outcome may not be relevant to clinical practice.

Standard gamble. This is where individuals are asked to decide how much they would be prepared to lose (i.e. gamble) for what potential benefit. For example, if they have a chronic disease which can potentially be treated, how much life (weeks, months, years) would they be prepared to lose for the chance of being in better health?

Relative risk. This is a reflection of how likely an outcome is to occur in one group of patients compared with another. For example, the relative risk of death from treatment A compared with treatment B. It is calculated as the risk of the event in one group divided by the risk of the same event in another group.

Sensitivity & specificity. The sensitivity of a test describes how well it detects the effect it is set up to examine, i.e. is the effect really there. Another way to look at this is, of the subjects which are said to be positive, the number that really are. The specificity assesses how accurately it detects the effect it is set up to examine, i.e. does it detect the real effect or does it detect something else. This can be viewed as, if the effect is not detected how certain can we be that it does not exist?

Submission. This is another way of describing the patient evidence. It may be a written submission or it may be given in person.

Willingness to pay. The maximum amount that a person is willing to pay: (i) to achieve a particular good health state or outcome, or to increase its probability of occurrence; or (ii) to avoid particular bad health state or outcome, or to decrease its probability.

Appendix 7 – Useful resources

Critical Appraisal Skills Programme (CASP)

<http://www.phru.nhs.uk/Pages/PHD/CASP.htm>

European network for Health Technology Assessment, EUnetHTA

<http://www.eunetha.net/>

Health and Quality of Life Outcomes, online journal <http://www.hqlo.com/home/>

Health Technology Assessment International (HTAi) www.htai.org/

International Network of Agencies for Health Technology Assessment (INAHTA)

www.inahta.org

National Information Center on Health Services Research and Health Care

Technology (NICHSR) <http://www.nlm.nih.gov/nichsr/edu/healthecon/index.html>

National Institute for Health and Clinical Excellence <http://www.nice.org.uk> (home) and for a guide for patient and carer groups on how to get involved in a technology appraisal see

http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/contributing_to_a_technology_appraisal_a_guide_for_patient_carer_groups_reference_n0516.jsp

Patient reported health instruments <http://phi.uhce.ox.ac.uk/> (home)

<http://phi.uhce.ox.ac.uk/links.php> (list of instruments for measuring quality of life)

Scottish Medicines Consortium Guidance on Patient Group Submissions

<http://www.scottishmedicines.org/smc/files/PatientInterestGroupSubmissionTemplate.doc> (template) and

<http://www.scottishmedicines.org/smc/files/Patient%20Interest%20Group%20Guidance..doc> (guidance)

Kristensen FB & Sigmund H (ed.)

Health Technology Assessment Handbook

Copenhagen: Danish Centre for Health Technology Assessment, National Board of Health, 2007

http://www.sst.dk/publ/Publ2008/MTV/Metode/HTA_Handbook_net_final.pdf

National Board of Health, Danish Centre for Health Technology Assessment

A review of organisational and patient-related assessments in HTAs published by INAHTA members. Copenhagen: National Board of Health, Danish Centre for Health Technology Assessment 2007. Series Name: Danish Health Technology Assessment 2007; 9 (2)

http://www.sst.dk/publ/Publ2007/MTV/Litteraturstudie/A_review_of_HTAs.pdf

Appendix 8 - Feedback form

Please return to:

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Fax: +44 1505 871979

Did you find this guide useful? Yes No

If No; why not?

Was it easy to use? Yes No

If NO; why not?

Did it answer all your queries? Yes No

If NO; why not?

How could the content be improved?

How could the layout be improved?

What else would you like to see in the guide?

Please tell us if you have experience of HTA, what it was like and what the outcome was

Any other comment you wish to make