Access and reimbursement:
A guide for patient groups

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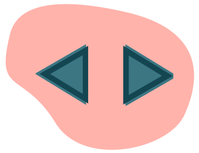
Introduction to the guide

How to use the guide

- This guide has been developed to provide an overview of the health technology assessment (HTA) process for patient groups (see next page for more details)
- The guide is separated out into different sections which can be accessed via the clickable **contents panel** to the right hand side of each page
- Please click on the **contents panel** to navigate to the first page of each section, for example, to return to a section if you navigate away



Click the **arrow buttons** in the bottom right hand corner of each page to move forwards and backwards between pages

Click the **help button** in the top right hand corner of each page to return to this page at any point

Click the **useful resources button** in the top right hand corner of each page to access additional useful resources and external links that may be helpful alongside the content of this guide

Click the **glossary button** in the top right hand corner of each page to access a glossary. This includes all terms highlighted in blue and yellow throughout the guide

Case studies can be found throughout the guide

Practical steps for patient groups can be found throughout the guide

Details on **patient group involvement** for each stage of the HTA process can be found throughout the guide

Introduction to the guide

Objectives

This guide has been developed for patient groups to:

- Understand how patient access to new treatments is obtained, including why access matters and when access considerations should start for patient groups
- Provide an overview of the health technology assessment (HTA) process for patients and patient groups
- Identify areas for patient groups to contribute to the different stages of the HTA process
- Set out practical steps for patient groups at each stage of HTA

Key definitions

Access: All patients who could benefit from a treatment are able to receive it when and where they need it, at a price that is affordable to the healthcare system

Health technology assessment (HTA): A process that looks at the short- and long-term consequences of using a new health technology (e.g. a treatment). It aims to summarise information about the medical, social, economic and ethical issues related to the use of this treatment. This is used to inform decisions about which treatments would be of most value in a healthcare system and which should be invested in

Reimbursement: When the company that makes a treatment is paid by the 'payer' of a healthcare system (for example, National Health Service [NHS England]) for the treatment, or when patients that use the treatment get repaid the cost of the treatment by the 'payer' (for example, private insurers)

Introduction to the guide

Contents

This guide will cover the different steps involved in the health technology assessment (HTA) process and is separated into the following sections:

- Understand how patient access to new treatments is obtained, including why access matters and when access considerations should start for patient groups
- Provide an overview of the health technology assessment (HTA) process for patients and patient groups
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Overview of access and reimbursement

What is access and reimbursement?

Healthcare systems, such as the NHS in the UK, have processes for deciding which new treatments will be available to patients. These processes determine which patients will have access to treatment. Patients and patient groups have an important role to play in these decisions.

In an ideal world, this access would mean all patients who could benefit from a treatment could receive it when and where they need it, at a price that is affordable to the healthcare system.

However, in reality, there is a limited budget to deliver healthcare services and difficult decisions must be made about how to prioritise. If a new treatment is introduced, something else may need to change or stop to make space for this new treatment; the cost impacts of new treatments are evaluated through health economic analyses.

This is where health technology assessments (HTAs) are used. This kind of assessment gives decision-makers (known as HTA bodies) important information to decide which treatments or services should be paid for (known as reimbursement). HTA can provide and inform them on the value of their decision.

However, before decisions can be made about whether a treatment will be reimbursed for a population, it first needs to be approved by regulatory authorities.

Overview of access and reimbursement

Regulatory approval

Regulatory approval is the first step towards access for a new treatment for patients.

Regulatory authorities decide if a treatment can be approved for use based on the following:

Safety of the treatment

Efficacy of a treatment

Quality of the product (from a manufacturing perspective)

Approval means that the treatment can be sold within that regulatory authority's domain, which is why regulatory approval is known as being given a marketing authorisation or license for a condition. For example:

- If the European Medicines Agency (EMA) grants a treatment a marketing authorisation, it can be sold to treat patients with that condition within the European Union
- Similarly, if the Food and Drug Administration (FDA) approves a treatment, it can be sold to patients with that condition in the United States of America

Regulatory authorities also monitor the use and long-term safety of any new treatments once approved. However, regulators will often accept greater uncertainty on the efficacy and safety of a treatment than a heath technology assessment (HTA).

Overview of access and reimbursement

Health technology assessment (HTA)

Healthcare systems differ between countries and sometimes between regions within the same country. Each system may be funded in a different way, and therefore have a different HTA body with different processes and priorities.

Examples of HTA bodies for different countries include:

- The National Institute for Health and Care Excellence (NICE) in England
- The Scottish Medicines Consortium (SMC) in Scotland
- Haute Autorité de Santé (HAS) in France
- Gemeinsamer Bundesausschuss (GBA) in Germany
- Agenzia Italiana Del Farmaco (AIFA) in Italy

HTA bodies look at the following things when deciding whether the treatment should be reimbursed in their patient population:

Clinical efficacy

- What is the impact of the treatment on the patients it is approved to treat?
- How does this compare to currently available treatments?

Cost or resources: HTA bodies generally assess this by:

- Cost effectiveness, do patients get enough benefit for the cost paid for the treatment?
- Budget impact, how does buying this treatment effect our ability to buy others?

The value of a treatment can go beyond its financial cost. Some HTA bodies consider the value that the treatment can offer to patients in terms of improving health-related quality of life (HRQoL) (a measure of someone's perceived physical and mental health over time) or being more convenient (for example, an oral treatment instead of an injection). They may also consider additional factors such as healthcare resources (for example, time needed from doctors or nurses to administer the treatment).

Overall, all HTA bodies are trying to answer the same questions: how effective is a treatment for patients and does the treatment represent good value?

Overview of access and reimbursement

Key steps in the health technology assessment (HTA) process

Broadly, the following steps are taken to complete a HTA:

- **Step 1: Getting started:** The group applying for reimbursement (usually a pharmaceutical company) start preparing for a HTA evidence submission by considering what questions need to be answered and gathering evidence (for example, what is the relevant patient population for this treatment?) This step is sometimes referred to as scoping
- **Step 2: Submission:** The group applying for reimbursement submits evidence to the HTA body for review, usually as a written evidence submission. The types of evidence may include background information about the condition and unmet need, clinical effectiveness and safety of the treatment, and cost effectiveness and/or budget impact analyses. These data are often derived from the key clinical trials of the treatment
- **Step 3: Assessment and consultation:** The HTA body will review the evidence provided and ask any clarifying questions. The HTA body will discuss the evidence and their initial decision with different stakeholders at committee meetings, which can include representatives from patient groups
- **Step 4: Decision:** The HTA body make their decision on whether the treatment will be reimbursed or not, based on the evidence. There may be some conditions of the reimbursement, such as the treatment having to be offered at a discounted price or limited to a smaller population within the broader population living with the particular condition
- **Step 5: Implementation:** Sometimes longer-term support is required, based on the HTA decision, which may involve patient groups of any HTA decision

Patient groups have a role to play at every stage of the HTA process, including before the HTA itself formally begins.

Overview of access and reimbursement

Patient group involvement

Involvement in HTA

Patient group involvement ensures that patient needs are heard during the health technology assessment (HTA) process and allows for a greater understanding of the real-life impact of new treatments.

- This helps make sure that treatments which are valuable to patients have a better chance of being reimbursed

Please refer to each section of this guide (accessible via the **contents panel** or **arrow buttons**) for further details on how patient groups can be involved at each step of the process.

Involvement before HTA

Patient groups can also be involved in the clinical development process, before regulatory approval of a new treatment.

- For more information on the clinical development process and patient group involvement, please refer to the Patient Group Research and Trial Design Hub Guide developed by Beacon. This is linked from the **useful resources** section

Before the HTA process starts, patient groups can start collecting relevant information for an upcoming HTA, which can be supported by pharmaceutical companies. These activities are covered in more detail later in this guide, but may include:

Organising patient surveys to understand more about the prevalence/incidence of a condition

Conducting questionnaires to learn about the burden of illness for patients and what patients ideally want from a treatment

Early patient group involvement helps pharmaceutical companies make sure that their clinical evidence is not only relevant for patients, but also helps them demonstrate the true value of a treatment when it comes to the HTA process.

Getting started

Key steps in the health technology assessment (HTA) process

Getting started	Submission	Assessment and consultation	Decision	Implementation
• Preparation • Scoping and setting the question • Gathering evidence				

Getting started

Preparation

The first step in any health technology assessment (HTA) process is deciding what to assess. Patient groups can contribute on deciding what is important to include in the evaluation for a given treatment.

The group wanting to apply for reimbursement of a treatment (usually the pharmaceutical company) will start preparing for the HTA process before the regulatory approval of a treatment is granted. HTA bodies sometimes progress with their evaluation of a treatment before it has been approved, so patient groups will want to have an awareness of what treatments are nearing the end of their clinical trials to start thinking about the HTA process.

- In this case, the company that will sell the treatment will be preparing for the HTA evidence submission well in advance of the regulatory approval

Scottish Medicines Consortium (SMC)

For the SMC, the evidence submission is usually made within the first month or soon after the regulatory approval of a treatment.

National Institute for Health and Care Excellence (NICE)

In the NICE process, HTA evidence submissions are made before treatments have regulatory approval.

NICE aim to make a decision as soon as possible after regulatory approval.

However, it is not compulsory to make an evidence submission to a HTA body and some pharmaceutical companies may decide not to do this for a range of reasons. In these cases, the treatment will not be reimbursed.

Getting started

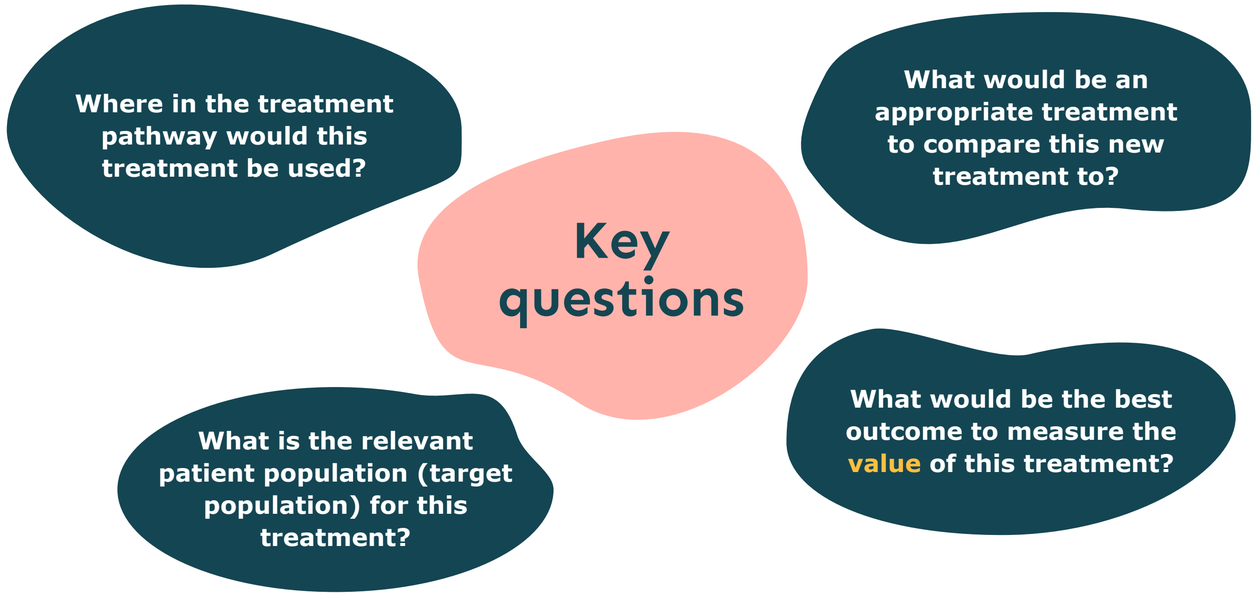
Practical steps for patient groups

- Make a list of what you as a patient group already have in terms of:
  - Expertise
  - Time
  - Resources
  - Patient experience and evidence (for the group of patients which the treatment is being appraised for)
- Identify a lead within your organisation
- Consider how you will keep your community informed throughout the process
- Contact the HTA body and ask to be registered as a stakeholder
- Ask for a contact name for consistency
- Enquire as to what support there is for patient groups during the process
- Find out who/which group of patients the treatment is being appraised for
- Contact the company and establish likely timelines for treatments being developed in your disease area
  - Ask for a contact name for consistency
- Contact other patient groups with an interest in the disease area
- Consider how/when to generate evidence for any gaps
- Consider what additional resources you may need and where you might get it from
- Identify patient representatives for attendance at the meeting i.e. patient experts (they may need support to upskill before the meeting)

Getting started

Scoping and setting the question

The first part of the health technology assessment (HTA) process itself sets out the key question(s) that need to be answered (also known as scoping). These questions provide a framework for the evidence and assessment, and may include:



At this stage, it will also be decided which type of HTA process should be used. For example:

- The National Institute for Health and Care Excellence (NICE) consider most treatments through a single technology appraisal (STA) under their normal process. However, some treatments for very rare conditions may be assessed in a highly specialised technology (HST) evaluation. This involves slightly different processes and criteria than standard NICE evaluations
- In Scotland, treatments for very rare conditions are assessed by the Scottish Medicines Consortium (SMC) in their ultra-orphan medicines process
  - For the criteria for these processes, please see the link in the **useful resources** section

Getting started

Gathering evidence

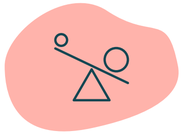
Evidence for a health technology assessment (HTA) will be gathered on the condition, any unmet need for patients, the treatment being assessed and the current costs of care, so that the right questions are addressed in the evidence submission. This information will be used to create an economic model, which looks at how cost effective a new treatment is compared to other available treatments and the current care of patients.

Types of evidence:

An understanding of the treatment pathway

Prevalence/incidence of the condition

Efficacy of the treatment (for example, from clinical trials)



A review of comparator treatments

Health-related quality of life (HRQoL) data

Resources currently used to treat patients

The burden of illness

- HTAs mainly focus on the impact to the health service or patients but do not typically look at the impact on society of a particular condition (for example, the impact on work, employment or education)

It is important that evidence used for a HTA submission is relevant to the patients that the treatment is being considered for

— For example, if a treatment is only being assessed for reimbursement in a particular subgroup of patients, the evidence should be relevant for this subgroup instead of the whole population with that disease

Getting started

Patient group involvement

The most valuable evidence patient groups can provide is that which directly involves patients. This could include talking to patients, understanding the treatment pathway, identifying patient expectations/concerns about new treatments and other relevant experiences.

Patient groups can contribute at this stage in the following ways:

- **Comment on the draft questions, aims and stakeholder list for a health technology assessment (HTA)**
- **Participate in scoping workshops to determine the key question(s)**
- **Provide guidance on current standard of care and appropriate comparators**
- **Advise on the areas of unmet need for patients**
- **Provide input on real-world prevalence or support with data collection**
- **Provide advice on the topic selection and type of HTA**

Patient groups will have a good understanding of their rare condition, an appreciation of the researchers and clinicians who understand it, and insights into the lived experience of patients. This makes them uniquely placed to generate both insight about the condition, and evidence to support it.

Patient group insight can help decision-makers interpret the evidence presented by different stakeholders. For example, these insights can help translate data from clinical trials into meaningful outcomes by explaining the real-world impact.

In this situation:

- "Insight" refers to the broad set of conclusions that can be drawn from the patient groups' knowledge of the rare disease field, and the testimony and experiences of those affected by the condition
- "Evidence" refers to information collected directly from patients, researchers or clinicians. This can include surveys, published research or interviews

Patient group involvement can help ensure that evidence gathered for the HTA process truly reflects the real-world needs of patients and their families, the realistic delivery of the treatment to those patients, and the opinions of a group of experts who are most active in the field.

Getting started

Case study: UK Masto

UK Masto, a volunteer patient group, was contacted by the National Institute for Health and Care Excellence (NICE) to ask if they would like to be involved in an evidence submission for a new treatment for mastocytosis. UK Masto reached out to Leukaemia Care who had experience in the health technology assessment (HTA) process. Leukaemia Care helped UK Masto challenge the NICE topic selection and advise on the type of assessment.

UK Masto advised NICE on incidence, appropriate comparators for the new treatment and the standard of care in the UK using their knowledge of the literature and by asking clinicians/patients.

To address the burden of illness, they asked patients they were aware of to complete a survey, as well as interviewing patients. These results were put into an evidence submission template for NICE.

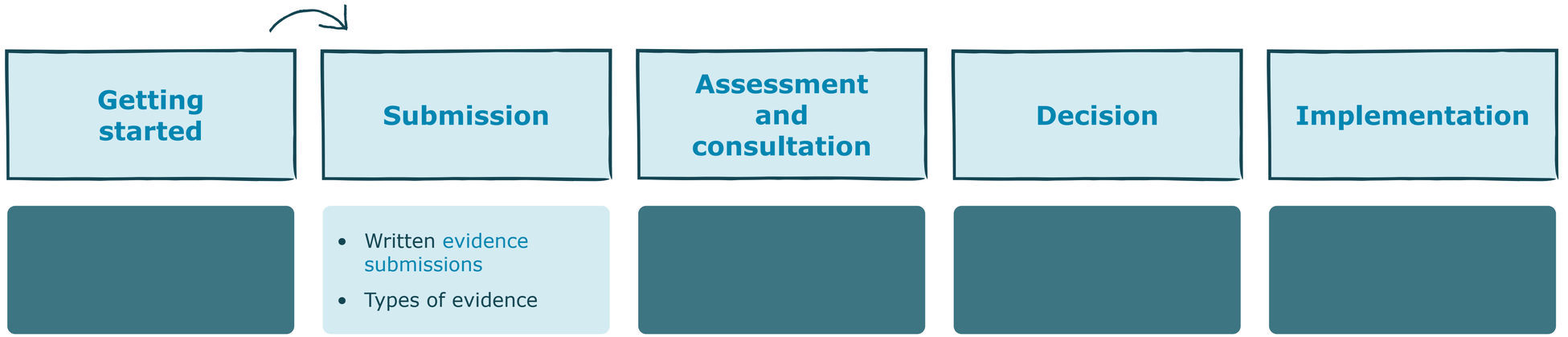
Ultimately, the treatment was recommended for reimbursement by NICE.

Key learnings:

- Surveys and interviews can be started way in advance of the HTA process (for example on the burden of illness or patient preferences for treatment)
- Patient group collaboration and knowledge sharing is invaluable

Submission

Key steps in the health technology assessment (HTA) process



Submission

Practical steps for patient groups

- Contact the health technology assessment (HTA) body to learn about the ways in which you can submit evidence for consideration
  - Is there a formal or informal process?
  - Should patient group evidence be written or verbal?
- Consider how long it will take for you to contribute and what resources/expertise you have to:
  - Identify any evidence gaps and produce a plan to address these
  - Ensure expertise and other resources are in place to implement this plan
  - Gather key information and evidence about the condition, treatments, unmet needs, patient experience and expectations for new treatments through surveys, focus groups or patient interviews. This may include the patient population, demographics and current treatment pathways
  - Meet with other interested patient groups, if relevant, agree whether or not to develop a joint evidence submission and assign a lead organisation
- Analyse your data and compile/write your evidence submission
- Contact the company and other stakeholders to share priorities for the evidence submission

Submission

Written evidence submissions

Stakeholders may be invited to submit evidence to a health technology assessment (HTA) body. This will include the manufacturer or sponsor and may also include clinicians, patients, payers and other interested parties. In the UK, patient groups are invited to submit evidence and this plays an important role in decision making. Patient groups can write their own submission, and patients can contribute directly to this by writing and submitting their own letters.

- In the National Institute for Health and Care Excellence (NICE) process, there are two ways for patients to submit evidence:
  - **Patient group statement:** this may come from one patient group or different groups may choose to produce a joint statement
  - **Patient experts' individual statements:** a nominated patient expert may either agree with the patient group's statement (if one has been submitted), or submit their own statement. Patient experts are people with a broad knowledge of the condition, its current treatments, the new treatment and what is important to patients. They could also be someone with personal experience of living with or caring for someone with the condition. They are nominated by the patient group
- Other stakeholders can write an evidence submission for a HTA (for example, the pharmaceutical company developing the treatment). Patients and patient groups can also get involved in these submissions
- Where no formal opportunities exist to get involved in a HTA, there may be informal routes to contribute to evidence submissions from other stakeholders

The most valuable evidence from patients is the evidence that links the patient experience to the questions the HTA body is trying to answer. For example, translating clinical trial endpoints into what this means for patients and the impact it will have on their daily lives.

Submission

Types of evidence

The evidence to support the reimbursement of a treatment will be brought together into a written evidence submission. These submissions generally have four key sections:

- Disease background and unmet need, including:
  - Description of what the disease is and the impact it has on patients
  - Description of current treatment options for patients and the need for new treatments

- Cost effectiveness:
  - This describes the value for money of the treatment and is shown in an economic model
  - Inputs into the economic model will come from a range of sources, such as patient groups, existing literature, and clinical experts
  - Cost effectiveness may also consider what healthcare resources (such as doctors'/nurses' time or hospital appointments) are needed to give patients this treatment
  - There will be a description of how the economic model was developed, and the associated results. For example, all health technology assessment (HTA) bodies in the UK measure cost-effectiveness using incremental cost-effectiveness ratios (ICERs) and quality-adjusted life years (QALYs)

- Clinical effectiveness and safety, including:
  - Description of the relevant clinical trials, as well as efficacy and safety results
  - Evidence from other sources for indirect treatment comparisons

- Budget impact on the healthcare system:
  - This describes the cost to the healthcare system based on how many patients are predicted to use the treatment over the next few years. This involves comparing the total costs if the new treatment is not introduced, with the total costs if the new treatment is introduced
  - This section will include an overview of how the budget impact was developed, and associated results

Submission

Patient group involvement

Patient groups have an essential role in helping decision-makers understand a condition. This is particularly important for rare diseases as there may be limited published evidence available and little experience within health technology assessment (HTA) bodies. During the HTA process, patient stories and how this experience is conveyed is very important.

Patients and patient groups can get involved at this stage by:

Inputting into the evidence submission

Contributing evidence for economic models

Providing information on the burden of illness from patient surveys or registries

- The impact of the disease on patients and their families, as well as the impact a new treatment could have, is valuable information patients and patient groups can provide. The type of evidence patient groups provide may depend on the time and resources they have available:
  - A survey of patients could be conducted to ask patients about current treatments, their experiences of living with a condition, their treatment goals and potentially about their experiences of a treatment if they have been involved in a clinical trial. This evidence could also be generated through focus groups or patient interviews
  - If resources are available, a measure of health-related quality of life (HRQoL) could be developed

Patient groups play a key role in supporting with evidence, especially when limited information on a condition is available. The type of evidence a patient group can provide may depend on their time and resources.

Submission

Case study: Project HERCULES

Duchenne UK's Project HERCULES is a patient-led global collaboration for Duchenne Muscular Dystrophy (DMD) to develop evidence for health technology assessment (HTA) bodies.

- Ten pharmaceutical companies, several international patient groups, universities, HTA bodies and clinical experts came together, and aimed to build evidence and tools that could be used by multiple different companies working on DMD treatments
- The hope of this was to level to playing field for HTA in Duchenne, and ensure quality evidence and models were used for all treatments
- This approach helped them build a broad collaboration and obtain funding for the project

Project HERCULES developed a new natural history model based on the largest collection of clinical data in DMD.

- This model identified a disease stage which had not been previously defined, (a transfer stage which happens between patients being able to walk and being fully non-ambulant), which has had a significant impact on the quality of life of patients and their families, the care required and the associated costs (for example, the need for hoists and home adaptations)

Project HERCULES reviewed tools used to measure health-related quality of life (HRQoL) in DMD and found that there was no available tool that measured what was most important to patients and families. They developed a DMD specific measure of HRQoL, the DMD-QoL, which is now translated into over 25 languages and is starting to be used in research and clinical trials.

Project HERCULES also conducted a burden of illness study to collect information and understand the impact of DMD on healthcare resources, work and productivity (direct and indirect costs).

All this information was used to build a disease-level economic model, which incorporates patient perspectives and can be adapted by companies for individual treatments.

Key learnings:

- Surveys and interviews can be started way in advance of the HTA process (for example on the burden of illness or patient preferences for treatment)
- Patient group collaboration and knowledge sharing is invaluable

Assessment and consultation

Key steps in the health technology assessment (HTA) process

Assessment and consultation

Practical steps for patient groups

- Contact others with previous experience of committee meetings to better understand what to expect
- Identify a lead person and any additional representatives (patient experts) to attend the meetings. This can be discussed with other patient groups to agree the most appropriate advocates
- Find out about the purpose of the meeting and how it fits in to the overall health technology assessment (HTA) process
- Find out who else will be attending the meeting
- Find out how the meeting will be run and what opportunities you will have to speak
- Review the relevant documents and decide the key points you want to raise at the meeting
- Consider how to keep your community informed of progress

Assessment and consultation

Evidence review

The health techonlogy assessment (HTA) body will review the evidence submission from the manufacturer and other stakeholders, which may involve meetings to:

- Work through the evidence and any uncertainties associated with the treatment
- Ask any clarifying questions

The HTA body may involve the manufacturer and other stakeholders (such as patient groups and clinical experts) in discussions about the evidence so that different perspectives are considered ahead of a formal committee meeting. A draft decision is then made, which may be consulted on.

In the NICE process, an independent EAG assesses the evidence and writes a summary report.

National Institute for Health and Care Excellence (NICE)

External Assessment Group (EAG)

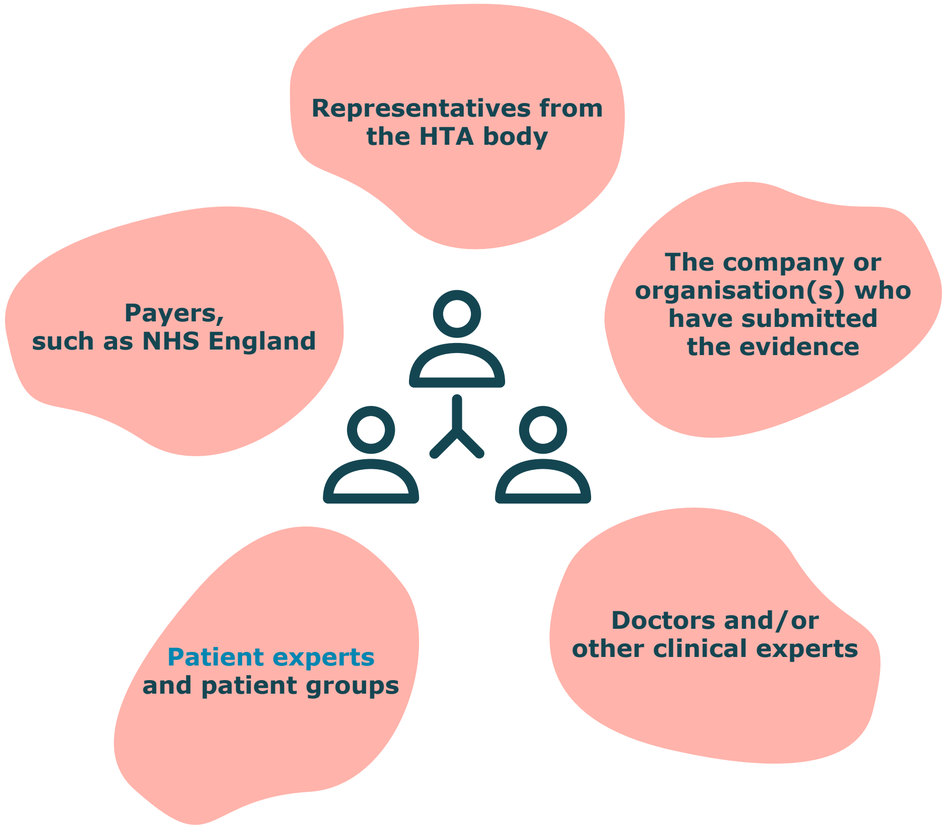
An EAG is an academic organisation that provides feedback on the evidence submission and guides NICE's decision making. They may invite people such as clinical experts to comment on their report.

Assessment and consultation

Committee meetings

Formal committee meetings take place to discuss the health technology assessment (HTA) body's evaluation. This may involve a range of stakeholders to help address any remaining uncertainties.

Stakeholders at a committee meeting could include:



In some HTA processes, patient groups may be a part of the committee meetings. In other countries, there may be a separate step to hear the perspectives of patients and clinicians.

- For example, for the Scottish Medicines Consortium (SMC), there is a separate Patient and Clinician Engagement (PACE) meeting for end-of-life treatments or treatments for rare diseases. In this meeting, patient representatives and clinical experts are brought together to discuss the benefits of a treatment, including how it can impact a patient's quality of life. This describes the added benefits of a treatment that may not have been fully captured within the conventional clinical and economic assessments
- In the National Institute for Health and Care Excellence (NICE) process, patient representatives attend the committee meetings

Assessment and consultation

Patient group involvement

Patient groups may be consulted on the evidence presented during committee meetings.

Patients and patient groups can get involved at this stage by:

- **Attending committee meetings and nominating patient experts**
- **Answering questions or addressing uncertainties at committee meetings**
- **Providing comments on the health technology assessment (HTA) bodies draft decision and the supporting evidence**
- **Sharing the patient experience (such as their current experience of symptoms, their perceptions of treatment benefits and risks, treatment preferences), and the impact of the decision on patients**

Attending committee meetings

- Patient experts may be asked to share their perspective on the disease and current treatments during committee meetings and may also be asked questions about the evidence
- Preparation for committee meetings is very important. Patient experts should know what their key points to discuss are, what the priorities are for the other stakeholders, and what their role is within the meeting

It is important to ensure that patient experiences are shared in a way that demonstrates the value that a treatment would bring. For example, connecting an endpoint in a clinical trial to the impact on the daily lives and activities of patients and families.

Assessment and consultation

Case study: Soft Bones UK/Climb UK

A parent of a child with hypophosphatasia became a patient expert for Climb UK (now Metabolic Support UK) after their child was given a new treatment during a clinical trial. Their role in the health technology assessment (HTA) process was to describe their experience with the condition as a parent, how it had affected their child and their experience of the new treatment.

The initial draft guidance did not recommend use of the treatment. Following consultations and negotiations it was later approved in a managed access agreement. This agreement allows the treatment to be approved on an interim basis pending more data being collected. To support the managed access agreement, Soft Bones UK conducted patient surveys to collect more patient views.

- They also collected patient stories about the new treatment to show how the treatment had significantly improved their quality of life

Soft Bones UK was subsequently set up as the first patient advocacy group for hypophosphatasia.

Key learnings:

- Soft Bones UK recommended collecting information on the patient experience as early as possible

Decision

Key steps in the health technology assessment (HTA) process

Decision

Practical steps for patient groups

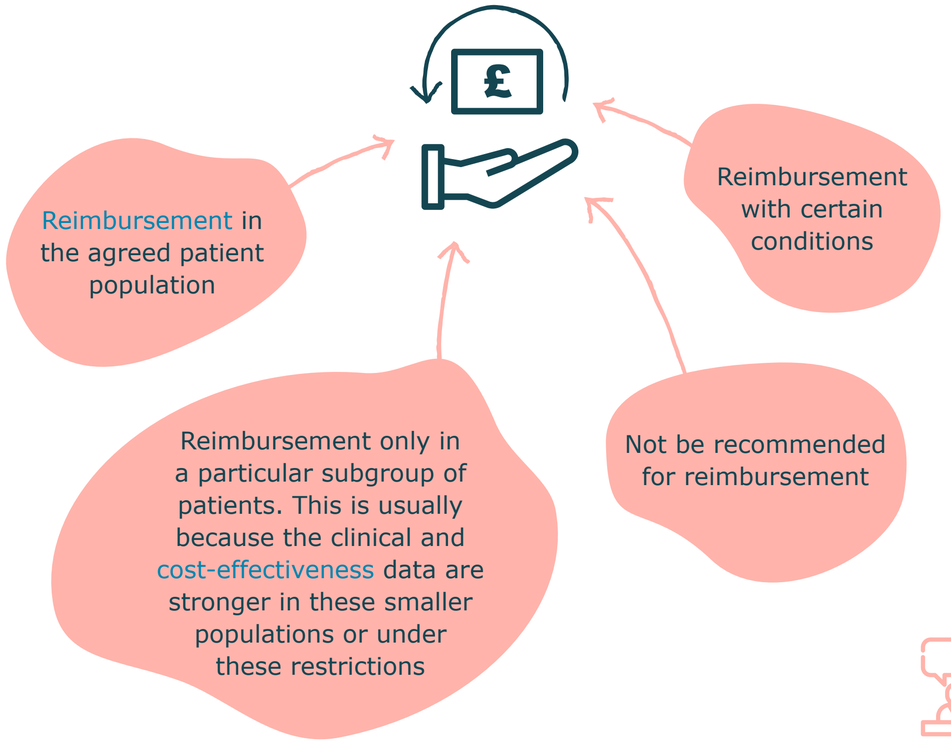
- Consider how to share the decision with the patient community
- Get guidance on the reasons for the decision, if clarity is needed
- Gather the evidence needed to respond to the decision
- Consider formal and informal routes for sharing comments and concerns
- Consider if you have grounds for appeal

Decision

Potential outcomes

The health technology assessment (HTA) body will have a meeting where all the evidence and discussions are considered. During the evaluation, the HTA body will make a final decision on their recommendation.

Potential outcomes of the HTA process could include:



Reimbursement conditions for the National Institute for Health and Care Excellence (NICE) could include:

- A discounted price: The company that sells the treatment may offer the HTA body a discounted price for the treatment that would make it more cost effective (also known as a patient access scheme). These discounts are usually confidential
- A managed access agreement could be put in place to make a treatment available for a limited period of time. This will involve a discounted price and collecting evidence on its use to address any uncertainties. This will address any gaps in the evidence that were identified during the HTA process. At the end of this process, a new evidence submission will be submitted for review and a final decision on reimbursement will be made
- A commercial access arrangement may be offered if it would be particularly challenging to launch the new treatment. The arrangements may involve cost sharing or the introduction of start and stop criteria for the treatment

In some situations, treatments that are not initially recommended may be appealed for re-review. If new evidence becomes available, a decision can also be reviewed.

Patients and patient groups can get involved at this stage by:

- Commenting on the decision and reasoning
- Appealing the decision

Implementation

Key steps in the health technology assessment (HTA) process

Implementation

Practical steps for patient groups

- Communicate the decision with the community and consider any requirements or restrictions to access

Implementation

Patient group involvement

Sometimes patient groups will need to support the long-term implementation of a health technology assessment (HTA) decision to ensure patients are aware of the availability of the new treatment, and any restrictions or requirements that have been put in place.

The required patient group involvement and next steps will depend on the decision from the HTA body:

- If the treatment has received approval for a limited time, for example as part of a managed access agreement, the patient group can help:

Explain the decision to the patient community

Ensure patients understand the consent they need to give to receive treatment

Ensure patients understand any additional requirements, such as stopping rules or the need for regular assessments

Collect data to address data gaps, for example through patient surveys

- Patient groups may be involved in the negotiations for managed access agreements and part of any oversight of these agreements

Glossary

Access: Means all patients who could benefit from a treatment are able to receive it when and where they need it, at a price that is affordable to the healthcare system

Budget impact: Describes the additional cost to the healthcare system of a new treatment based on how many patients are predicted to use the treatment. This involves comparing the total costs if the new treatment is not introduced, with the total costs if the new treatment is introduced

Burden of illness: The impact that a condition has on patients and the wider healthcare system. This may include the problems that the patient faces as a result of their symptoms or treatment, the financial cost associated with treating and managing a condition, or the reduction in quality of life patients face

Clinical trial: A clinical experiment in which doctors give patients a new treatment to develop high quality evidence as to whether it is safe and effective at treating their disease

Commercial access arrangement: An agreement that may be offered if it would be particularly challenging to launch the new treatment. The arrangements may involve cost sharing or the introduction of start and stop criteria for the treatment

Comparator: A treatment that is currently used to treat a particular condition, which a treatment under investigation can be compared to in order to understand the effect or value of a new treatment

Cost effectiveness: Value for money of a new treatment, assessed using an economic analysis of costs and clinical benefits

Economic model: Aims to follow the patient journey through treatment and disease progression using available data. This can either be based on an 'average' group of patients, or can separate out different types of patients (for example, based on prior treatments)

Effectiveness: How well a treatment works for patients in a real-world environment

Efficacy: How well a treatment works in a patient, measured using the endpoints from clinical trials

Endpoint: A measure of the outcome of a clinical trial used by scientists to study the effect of a treatment on a patient's feeling, function and/or survival. Scientists test whether a treatment has achieved its endpoints using methods including interviews, questionnaires, examinations, laboratory tests and scans

Evidence submission: All the evidence to support the reimbursement of a treatment is brought together in one document for a HTA. These submissions generally cover disease background and unmet need, clinical effectiveness and safety, and cost effectiveness and/or budget impact

Health economics: The study of how best to allocate resources (such as money, nurses and doctors' time, appointments) in a healthcare system

Health-related quality of life (HRQoL): A measure of someone's perceived physical and mental health over time

Health technology: Any intervention that can be used to increase health of patients, prevent disease or help diagnose/treat a condition. It may include a treatment, a programme to prevent an illness (such as a childhood vaccination programme) or a procedure (such as surgery)

Health technology assessment (HTA): A process that looks at the short- and long-term consequences of a using a new health technology (e.g. a treatment). It aims to summarise information about the medical, social, economic and ethical issues related to the use of this treatment. This is used to inform decisions about which treatments would be of most value in a healthcare system and which should be invested in

Glossary

Healthcare resources: All materials, funds, facilities, people or anything else that can be used to provide healthcare services. For example, this may include doctors' or nurses' time

HTA bodies: Organisations that conduct HTAs and provide recommendations on the treatments that should be paid for or reimbursed. For example, NICE is the HTA body in England, and the SMC is the HTA body in Scotland

Highly specialised technology (HST): A treatment for a very rare condition. This may also be referred to as an 'ultra-orphan' treatment for a very small population of patients that may only be available at a few clinical centres. If a HST meets pre-set criteria, the HTA may be prioritised

Incidence: How many new patients with a particular condition there will be during a specific time period (for example, how many new cases there will be in a year)

Incremental cost-effectiveness ratio (ICER): The total cost difference between using the new treatment and the comparator treatment, divided by the total difference in QALYs

Indirect treatment comparison: This is a way of comparing two treatments (for example, treatment A and treatment B) which have not been directly compared in clinical trials, but have both been compared to another treatment (treatment C). The relative efficacy of treatment A and B compared to treatment C can be compared

Managed access agreement: Where a treatment is made available for a certain period of time at a discounted price so that more evidence can be gathered on its use. This may be done to address a gap in the evidence that was identified during the HTA process. At the end of this process, a new evidence submission may be submitted to try and get full reimbursement

National Institute for Health and Care Excellence (NICE): The HTA body responsible for decision-making when recommending whether new treatments should be reimbursed in England

Natural history: Information collected from a study that follows a group of people over time who have, or are at risk of developing, a particular disease

Patient access scheme: Where the company that sell the treatment offer the HTA body a discounted price for the treatment that would make it more cost effective. These discounts are usually confidential

Patient and clinician engagement (PACE) meeting: This is a stage in the SMC's HTA process for end-of-life treatments or treatments for rare diseases. In this meeting, patient representatives and clinical experts are brought together to discuss the benefits of a treatment, including how it can impact a patient's quality of life. This describes the added benefits of a treatment that may not have been fully captured within the conventional clinical and economic assessments

Patient expert: People with a broad knowledge of the condition, its current treatments, the new treatment and what is important to patients. They could also be someone with personal experience of living with or caring for someone with the condition

Prevalence: How many people in a particular population are living with a condition

Quality adjusted life year (QALY): A numerical measure often used to quantify benefits in a HTA. It is a combination of the number of years lived and a measure of quality of life of patients (commonly by a measure called a utility value). QALYs are calculated by multiplying the number of years of life by the utility value for quality of life. For example, one QALY is equal to one year in perfect health, and half a QALY is equal to half a year in perfect health or one year with half of perfect health. These values allow a comparison across different conditions

Glossary

Regulatory approval: When a treatment is approved for use in a particular region, based on the safety and efficacy of the treatment, and how well it is made (this does not consider economic factors). This is sometimes referred to as licensing or marketing authorisation

Reimbursement/reimbursed: When the company that makes a treatment is paid by the 'payer' of a healthcare system (for example, NHS England) for the treatment, or when patients that use the treatment get repaid the cost of the treatment by the 'payer' (for example, private insurers)

Scoping: The initial process at the start of a HTA when the key questions are addressed and the context of the HTA is decided. This can include deciding the target population and appropriate treatment comparators

Scottish Medicines Consortium (SMC): The HTA body response for deciding whether to accept a new treatment for reimbursement in Scotland

Single technology appraisal (STA): Where one health technology is compared with the current standard of care for the condition of interest during the HTA process for NICE

Value: The value of a treatment includes a number of factors and can go beyond its financial cost. Some HTA bodies consider the value that the treatment can offer to patients in terms of improving HRQoL or being more convenient (for example, an oral treatment instead of an injection). They may also consider the healthcare resources associated with a treatment (for example, time needed from doctors or nurses to administer the treatment) into the value

Useful resources

External resources:

- Beacon's Resource Hub: https://resourceshub.rarebeacon.org/
  - What is health economics?: https://resourceshub.rarebeacon.org/courses/what-is-health-economics/
  - Patient group involvement in research and trial design: https://resourceshub.rarebeacon.org/courses/patient-group-research-and-trial-design-hub-guide/
  - How to work with pharma: https://resourceshub.rarebeacon.org/courses/working-with-industry/
- EUPATI Training Portfolio Website: https://eupati.eu/training/
- Project HERCULES: www.projecthercules.org
- Realise Advocacy: Patient Advocacy Website: https://www.realiseadvocacy.com/

Webinars:

- Navigating Health Technology Assessments: https://www.youtube.com/watch?v=mCPOKYanprs
- Understanding Health Economics: Preparing Your Patient Group for HTA: https://www.youtube.com/watch?v=zkdjiO0k1Ew

HTA body websites:

- National Institute for Health and Care Excellence (NICE): https://www.nice.org.uk
- Scottish Medicines Consortium (SMC): https://www.scottishmedicines.org.uk/
- Haute Autorité de Santé (HAS) in France: https://www.has-sante.fr/
- Gemeinsamer Bundesausschuss (GBA) in Germany: https://www.g-ba.de/
- Agenzia Italiana Del Farmaco (AIFA) in Italy: https://www.aifa.gov.it/en/web/guest/home
- NICE highly specialised technology (HST): https://www.nice.org.uk/process/pmg37/chapter/highly-specialised-technologies
- SMC Ultra Orphan Medicines Process: https://www.scottishmedicines.org.uk/how-we-decide/ultra-orphan-medicines-for-extremely-rare-conditions/