Health Technology Assessment (HTA)

This Infosheet explains what health technology assessment (HTA) is and how it works, the role of HTA organisations such as NICE in deciding whether or not new myeloma drugs are funded by the NHS, and the impact HTA decisions can have on myeloma patients.

What is HTA?

Health technology assessment (HTA) plays a key role in whether or not new myeloma drugs are funded by the NHS.

Before a new myeloma drug can be widely used, it must first be licensed as a safe and effective treatment for myeloma. This is usually done by regulatory authorities at a European level and involves a review of evidence from large-scale clinical trials. As a licensed drug, it may be prescribed by doctors. However, at this stage it is not guaranteed NHS funding. Doctors can apply to their local funding body to fund the drug, though this is not guaranteed to be successful, or patients can purchase it

privately, which can often be very expensive.

The licensed drug must be approved by a UK drug assessment organisation through a process called HTA before it can be routinely prescribed by NHS doctors.

The HTA process differs from licensing – it compares the benefits of the newly-licensed drug to existing drugs already used in the treatment of myeloma on the NHS and decides whether it offers the NHS good value for money. Following the assessment, the HTA organisation will make a recommendation about whether or not the NHS should fund the new drug.

You may hear the terms health technology assessment and health technology appraisal used. These both mean the process by which myeloma drugs are assessed for use on the NHS.

Organisations responsible for HTA in the United Kingdom

England, Wales and Northern Ireland

The main HTA organisation in England, Wales and Northern Ireland is the National Institute for Health and Care Excellence (NICE).

NICE identifies new myeloma drugs requiring review through a system of 'horizon-scanning'. This means that they look at all the drugs in clinical trials that are likely to go through the European licensing process. By looking at drugs early on in their development, NICE can help ensure that new drugs are approved and made available to myeloma patients as quickly as possible.

Positive NICE recommendations are legally binding in England and Wales. This means that English and Welsh local funding bodies must provide funding within three months of a drug being approved.

Wales also has a separate HTA organisation called the All Wales Medicine Strategy Group (AWMSG). The AWMSG carries out a short assessment of new myeloma drugs that are not due to be reviewed by NICE within the next 12-18 months. From this assessment, it makes recommendations for the use and funding of these drugs by the NHS in Wales. As with NICE guidance, health boards in Wales must make the drug available within three months of the AWMSG approving a drug.

If NICE publishes guidance on a myeloma drug which has been evaluated by the AWMSG, NICE guidance usually overrides the AWMSG recommendation. However, if NICE guidance is at odds with an AWMSG recommendation, the AWMSG can make a case for why a particular drug is needed for myeloma patients in Wales.

In Northern Ireland, NICE guidance is reviewed by the Northern Irish Department of Health and is usually adopted within two months of publication by NICE.

Scotland

Scotland does not have to follow NICE guidance. New myeloma drugs are reviewed by the Scottish Medicines Consortium (SMC) within six months of receiving their licence. Unlike NICE guidance, recommendations given by the SMC are not legally binding. This means that health boards do not have to make new myeloma drugs available, even if approved by the SMC. If a health board decides not to make the new drug available to patients within three months of the SMC decision, it has to explain why on its website.

How do HTA organisations make a decision about a myeloma drug?

The clinical and cost-effectiveness of a new myeloma drug is evaluated by an evidence review committee. The committee considers the published evidence, such as data from clinical trials of the new drug, and speaks to clinical experts and patient groups such as Myeloma UK. The drug company which owns the drug is also asked to present evidence on the clinical and cost-effectiveness of the drug being assessed.

To decide if a myeloma drug is **clinically effective**, the committee looks at how well the drug has worked in clinical trials, what side-effects it can cause and whether there are any subgroups of patients where the drug works particularly well, for example relapsed myeloma patients who have previously been treated with thalidomide. The committee also considers any increase in clinical effectiveness the new drug may have compared to drugs currently available for the treatment of myeloma on the NHS.

To decide whether a myeloma drug is **cost-effective** the committee compares the cost and clinical benefit of the new drug against similar drugs currently available on the NHS using a health economic measure called the QALY (Quality-Adjusted Life Year).

What is the QALY?

QALYs measure the quality and quantity of life following treatment with a new myeloma drug. Put another way, a QALY reflects any increase in life expectancy alongside any change (positive or negative) to quality of life. One QALY is equal to one extra year of life in perfect health. Half a QALY is equal to one extra year of life but in poor health.

HTAs look at how many extra QALYs a new drug will give to a patient compared to current treatment for myeloma, and how much those extra QALYs will cost. In this way, using QALYs makes it easier to decide whether a new drug is clinically and cost-effective compared to drugs currently available on the NHS.

Using QALYs also allows HTA organisations to have a minimum QALY score that they want to see for all new drugs approved for use on the NHS. New drugs costing more than £20,000-£30,000 per QALY often require more evidence before they can be approved for use on the NHS.

For drugs that treat rare diseases such as myeloma or are used towards the end of life, both the SMC and NICE can use what they call 'decision-making modifiers'. These modifiers bring down the cost per QALY and help the SMC and NICE to approve these drugs for use on the NHS.

What are the possible outcomes of HTA?

For a new myeloma drug to be approved by HTA, the drug company must show that the benefits of the new drug are an improvement on current NHS treatment for myeloma. The company must also justify the cost of the new drug. If the HTA committee does not feel this has been shown the drug may not be recommended for use.

There are three possible outcomes of HTA for a myeloma drug being assessed:

- **Unrestricted approval:** it is approved for use on the NHS in the patient group it was intended for eg all relapsed myeloma patients

- **Restricted approval:** it is approved for use on the NHS but with some restrictions eg myeloma patients at first relapse only
- It is **not approved** for use on the NHS

Drugs that are approved with unrestricted or restricted approval have to then be funded by the NHS. As explained previously, this is a legal requirement in England and Wales. In Scotland, health boards are asked to make the drug available but this is not a legal requirement.

Factors influencing the HTA process

Though the decision-making process can be strict, the HTA process is robust and makes sure that the myeloma drugs which are available on the NHS are effective and backed up by strong evidence.

However, there are a number of perceived limitations to the process. These include:

Timescales

There is often criticism that the HTA process can be slow – a NICE review, for example, can take nearly a year and if there is an appeal it can be an even longer wait for myeloma patients hoping to access a new drug.

One size fits all

The HTA system can be viewed as limiting doctors' flexibility in how to treat their patients, instead providing a 'one size fits all' approach. This is particularly felt in myeloma given its individual nature and the need for multiple treatment options at different stages of the disease.

There are also some external factors that can limit the effectiveness of the HTA process, including:

The price of drugs

There is a perception that HTA is dominated by financial rather than clinical considerations. There is broad agreement, however, that the price of new cancer drugs set by drug companies has become prohibitively high. Furthermore, HTA organisations are unable to negotiate with drug companies over the prices they set for new drugs. This can mean that, even when presented with robust clinical effectiveness evidence, HTA organisations may not approve a myeloma drug because of the high price that has been set for the drug.

Quality of evidence

The HTA process can be limited by the quality of evidence submitted by drug companies to support their new drug. For example, if clinical trials are not designed to compare the new myeloma drug to the standard drugs already available on the NHS, it is difficult to make the case for approving it. Trials may also not be designed to collect the right type of information about myeloma patients' quality of life while taking a new drug, which makes measuring the QALYs for the drug more difficult. These issues with evidence generation can lead to delays or negative guidance being published.

How do HTA decisions affect myeloma patients?

Myeloma is a complex relapsing and remitting cancer, which means that myeloma patients need new and effective treatment options readily available at different stages of their myeloma. Access to new drugs can therefore be a cause of worry and concern for patients.

HTA directly impacts what drugs myeloma patients can access and at which stage of their myeloma. Therefore, when HTA guidance is slow to be publicised or is negative it can limit the treatment options available to myeloma patients. This is particularly difficult for myeloma patients who have already had a number of the current treatments available on the NHS.

In addition, having different HTA organisations across the UK can result in a 'postcode lottery' of drug availability: sometimes drugs are approved for use in one part of the UK while being unavailable in others. For example, Imnovid® (pomalidomide) is currently available in Scotland and Wales for a subgroup of myeloma patients, but it is not available in England as NICE turned it down.

Some limited options do exist for myeloma patients to try and access drugs that have not been HTA-approved for use on the NHS, including:

Local Funding Requests

Across the UK, doctors can make funding requests to their local funding body on behalf of a patient to fund a myeloma drug which is not normally available. When applying, the doctor must usually present an exceptional reason why the patient needs the drug.

The Cancer Drugs Fund (England) and New Medicines Fund (Scotland)

Set up in 2011, the Cancer Drugs Fund (CDF) was put in place to improve access to drugs in England which had not been approved by NICE or were going through the NICE HTA process. Ongoing budget constraints have led to the removal of some myeloma drugs which were previously available through the CDF.

As of July 2016, the CDF has been incorporated into the NICE appraisal system. New drugs which appear promising but are missing some evidence to prove that they are clinically and cost-effective will be given 'conditional approval' by NICE and made available to patients through the CDF. This allows the drug company to collect more information about the benefit of the drug in a 'real life' clinical setting to submit as additional evidence to NICE.

The CDF will fund conditionally approved drugs for a maximum of two years, at the end of which time they will be further assessed by NICE.

In Scotland the New Medicines Fund helps health boards in Scotland to fund the cost of drugs for patients with rare diseases such as myeloma or who are at the end of life.

Patient Access Schemes

Sometimes drug companies can introduce a Patient Access Scheme which is a way to make a drug more cost-effective and help it get HTA approval. One example in myeloma is the Velcade Response Scheme, where the drug company offers the NHS money back if patients do not respond well to Velcade® (bortezomib).

Future directions

HTA continues to drive which drugs are available on the NHS for myeloma patients, but its effectiveness can be hampered by various limitations.

There have been widespread calls for reform to the HTA system to improve access to new drugs; however, reform of the entire process of bringing new drugs into the NHS would be more effective at overcoming the existing issues.

Patient access schemes and decision-making modifiers have been successful in the short-term at getting drugs through the

HTA process. However, longer-term improvements to the HTA submission process, such as better clinical trial design and fairer drug pricing, are needed to improve patient access to new drugs.

In 2016, Myeloma UK awarded a research grant of £120,000 to NICE to study the best way of capturing patient preferences about the benefits and risks of treatment. This project will ensure that accurate data on patient preferences are included in the assessment of new drugs.

About this Infosheet

The information in this Infosheet is not meant to replace the advice of your medical team. They are the people to ask if you have questions about your individual situation. All Myeloma UK publications are extensively reviewed by patients and healthcare professionals prior to publication.

Other information available from Myeloma UK

Myeloma UK has a range of Essential Guides, Infoguides and Infosheets available covering many areas of myeloma, its treatment and management.

To order your free copies or to talk to one of our Myeloma Information Specialists about any aspect of myeloma, call the **Myeloma Infoline: 0800 980 3332** or **1800 937 773** from Ireland.

The Myeloma Infoline is open from Monday to Friday, 9am to 5pm and is free to phone from anywhere in the UK and Ireland. From outside the UK and Ireland, call **0131 557 9988** (charged at normal rate).

Information and support about myeloma is also available around the clock at **www.myeloma.org.uk**

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